

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**  
Washington, D.C. 20549

**FORM S-1  
REGISTRATION STATEMENT  
UNDER  
THE SECURITIES ACT OF 1933**

**bluebird bio, Inc.**

(Exact name of registrant as specified in its charter)

**Delaware**  
(State or other jurisdiction of  
incorporation or organization)

**2836**  
(Primary Standard Industrial  
Classification Code Number)

**13-3680878**  
(I.R.S. Employer Identification Number)

**840 Memorial Drive, 4<sup>th</sup> Floor  
Cambridge, MA 02139  
(617) 491-5601**

(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

**Nick Leschly  
President and Chief Executive Officer  
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(617) 491-5601**

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**Approximate date of commencement of proposed sale to public:** As soon as practicable after this Registration Statement is declared effective.

If any of the securities being registered on this Form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box.

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

If this Form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

If this Form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer  Accelerated filer  Non-accelerated filer  Smaller reporting company

(Do not check if a  
smaller reporting company)

**CALCULATION OF REGISTRATION FEE**

Title of each class of securities to be registered	Proposed maximum aggregate offering price(1)	Amount of registration fee(2)
Common stock, \$0.01 par value	\$86,250,000	\$11,764.50

(1) Includes offering price of shares that the underwriters have the option to purchase to cover overallocments, if any. Estimated solely for the purpose of calculating the registration fee in accordance with Rule 457(o) of the Securities Act of 1933, as amended.

(2) Calculated pursuant to Rule 457(o) based on an estimate of the proposed maximum aggregate offering price.

**The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Securities and Exchange Commission, acting pursuant to said Section 8(a), may determine.**

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The information in this prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This prospectus is not an offer to sell these

securities, and it is not soliciting an offer to buy these securities in any jurisdiction where the offer or sale is not permitted.

Subject to completion, dated May 14, 2013

Prospectus

*shares*



## Common stock

This is an initial public offering of common stock by bluebird bio, Inc. We are selling \_\_\_\_\_ shares of common stock. The estimated initial public offering price is between \$ \_\_\_\_\_ and \$ \_\_\_\_\_ per share.

Prior to this offering, there has been no public market for our common stock. We have applied for listing of our common stock on The Nasdaq Global Market under the symbol "BLUE."

We are an "emerging growth company" under applicable Securities and Exchange Commission rules and will be subject to reduced public company reporting requirements.

	Per share	Total
Initial public offering price	\$ _____	\$ _____
Underwriting discounts and commissions	\$ _____	\$ _____
Proceeds to bluebird bio, before expenses	\$ _____	\$ _____

We have granted the underwriters an option for a period of 30 days to purchase up to \_\_\_\_\_ additional shares of common stock.

Investing in our common stock involves a high degree of risk. See "[Risk factors](#)" beginning on page 13.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed on the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

The underwriters expect to deliver the shares of common stock to investors on or about \_\_\_\_\_, 2013.

**J.P. Morgan**

**BofA Merrill Lynch**

**Cowen and Company**

**Canaccord Genuity**

**Wedbush PacGrow Life Sciences**

, 2013

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## **TRANSFORMING THE LIVES OF PATIENTS WITH SEVERE GENETIC AND ORPHAN DISEASES**

### **MAKE HOPE A REALITY**

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Our objective is to develop and commercialize a next generation of products based on the transformative potential of gene therapy to treat patients with severe genetic and orphan diseases.

Central to this effort is a collective determination within our company to provide these patients with hope for a better life in the face of limited or no long-term safe and effective treatment options.

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We have not authorized anyone to provide you with information other than that contained in this prospectus or in any free writing prospectus prepared by or on behalf of us or to which we have referred you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give to you. We are offering to sell, and seeking offers to buy, shares of our common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or any sale of our common stock. Our business, financial condition, results of operations and prospects may have changed since that date.

No action is being taken in any jurisdiction outside the United States to permit a public offering of our common stock or possession or distribution of this prospectus in that jurisdiction. Persons who come into possession of this prospectus in jurisdictions outside the United States are required to inform themselves about and to observe any restrictions as to this offering and the distribution of this prospectus applicable to that jurisdiction.

## Prospectus summary

### Overview

We are a clinical-stage biotechnology company focused on transforming the lives of patients with severe genetic and orphan diseases using gene therapy. Many diseases have a genetic aspect whereby a mutated gene linked to a disease is passed down from generation to generation. Genes produce proteins that perform a vast array of functions within all living organisms, through a process called gene expression. A mutation, or alteration, in the gene or in sequences that control the expression of that gene can cause proteins to be produced aberrantly in the cell, which can cause disease. Gene therapy seeks to introduce a functional copy of the defective gene into a patient's own cells, a process called gene transfer. Gene therapy thereby has the potential to change the way these patients are treated by correcting the underlying genetic defect that is the *cause* of their disease, rather than offering solutions that only address their *symptoms*. Accordingly, we believe gene therapy has the potential to provide transformative disease modifying effects with life-long clinical benefits based on a single therapeutic administration.

In the gene transfer process, a functional gene is delivered and incorporated into a patient's cells through a delivery system called a vector, which are most commonly based on naturally-occurring viruses that have been modified to take advantage of the virus' natural ability to introduce genes into cells. However, unlike naturally-occurring viruses, which replicate following infection of a target cell and have the capacity to infect new cells, viral vectors are modified to be non-replicating by deleting that portion of the viral genome responsible for replication. Gene transfer using a viral vector is called transduction and the resulting gene-modified cells are described as transduced cells.

A growing body of gene therapy-based clinical data, the establishment of regulatory guidelines to govern the development and approval of gene therapy products and increased investment from the biopharmaceutical industry suggest that the time is now for gene therapy to emerge as an important new therapeutic modality for patients with significant unmet medical need. We believe we are particularly well-positioned to drive the continued advancement of gene therapy technology for the treatment of severe genetic and orphan diseases. We have assembled extensive expertise in viral vector design, manufacturing and gene transfer, a broad intellectual property estate, an experienced management team and a world-class group of scientific advisors and key opinion leaders. We refer to our viral vector and gene transfer technology and know-how as our gene therapy platform.

We and our scientific collaborators have generated what we believe is human proof-of-concept data for our gene therapy platform in two underserved diseases, each of which has been granted orphan drug status by U.S. and European regulatory authorities. We expect to initiate in late 2013 a Phase II/III clinical study of our most advanced product candidate, Lenti-D, to evaluate its safety and efficacy in subjects with childhood cerebral adrenoleukodystrophy, or CCALD, a rare, hereditary neurological disorder affecting young boys that is often fatal. We also expect to initiate in mid-2013 Phase I/II clinical studies in the United States and Europe of our next most advanced product candidate, LentiGlobin, to evaluate its safety and efficacy in subjects with  $\beta$ -thalassemia major and, in the European clinical study, sickle cell disease, or SCD, which are rare, hereditary blood disorders that often lead to severe anemia and shortened lifespans. In addition,



in March 2013, we announced a global strategic collaboration with Celgene Corporation to discover, develop and commercialize novel, disease-altering gene therapies in oncology.

### **Our gene therapy platform and process**

Our gene therapy platform is based on viral vectors that utilize a modified, non-replicating version of the Human Immunodeficiency Virus Type 1, or HIV-1 virus, that has been stripped of all of the components required for it to self-replicate and infect additional cells. The HIV-1 virus is part of the lentivirus family of viruses, as a result of which we refer to our vectors as lentiviral vectors. Our lentiviral vectors are used to introduce a functional copy of a gene to the patient's own isolated blood stem cells, called hematopoietic stem cells, or HSCs, which reside in a patient's bone marrow and are capable of differentiating into a wide range of cell types. HSCs are dividing cells, thus our approach allows for sustained expression of the modified gene as we are able to take advantage of a lifetime of replication of the gene-modified HSCs. Additionally, we have developed a proprietary cell-based vector manufacturing process that is both reproducible and scalable. We believe our innovations in viral vector design and related manufacturing processes are important steps towards advancing the field of gene therapy and in realizing its full potential on a commercial scale, a concept we refer to as the industrialization of gene therapy.

We believe our lentiviral vectors have certain advantages over other viral vectors used for gene therapy, including the ability to achieve long-term, sustained expression of the modified gene and reduced risk of insertional oncogenesis, the process whereby the corrected gene inserted near a gene that is important in cell growth or division, and this insertion results in uncontrolled cell division also known as cancer. Although our initial focus is in CCALD,  $\beta$ -thalassemia and SCD, we believe our gene therapy platform has broad therapeutic potential in a variety of indications. We believe our vectors can be used to introduce virtually any gene and have the potential to be manufactured on a commercial scale reproducibly and reliably, as each new vector is produced using substantially the same process. We also take advantage of lentivirus' ability to transduce HSCs more efficiently than other vectors, such as those derived from another virus used in gene therapy approaches, called adeno-associated virus, or AAV, which gives us the potential to address diseases in a variety of cell lineages that are derived from HSCs, such as microglia (useful for CCALD), red blood cells (useful for  $\beta$ -thalassemia and SCD), T cells (useful for cancer and immunology) and others.

Based in part on these features, we believe our gene therapy platform has several potential advantages over current treatment options for CCALD,  $\beta$ -thalassemia and SCD, including the following:

- **Single administration with potential life-long benefit.** Our process allows us to potentially arrest, correct or treat a disease with a single therapeutic administration.
- **We know exactly what gene to insert.** We are initially pursuing diseases where the genetic abnormality is known and is found in a single gene, known as monogenic diseases, thus mitigating against the uncertainty of the disease biology.
- **Existing practice of transplanting cells from a donor provides proof-of-concept for our approach.** Clinical proof-of-concept already exists for the diseases we are targeting via

allogeneic hematopoietic stem cell transplant, or HSCT, an approach of treating a patient with HSCs contributed by a donor that contain a functioning copy of the gene underlying the disease.

- **We use the patient’s own cells.** By using the patient’s own isolated HSCs, we believe our approach will eliminate many of the challenges associated with allogeneic HSCT, such as the limited availability of optimally matched donors and risks of transplant rejection that often result in serious adverse events, such as graft-versus-host disease, or GVHD.
- **We modify our target cells outside the patient’s body.** By inserting the new functional deoxyribonucleic acid, or DNA, into the cells outside the patient’s body, or *ex vivo*, thereby creating a gene-modified cell, we reduce the risk of adverse events and remove one of the key biological complexities of any therapeutic—getting a drug directly to the target cells.
- **Administration of our drug product is consistent with existing stem cell transplant practices.** The final step of our process, in which patients are myeloablated and then transfused with the finished drug product, is consistent with widely-adopted stem cell transplant clinical practices and infrastructure already in use.
- **Value proposition to patients, families, providers and payors.** Given the potentially dramatic clinical and life-long benefits anticipated from such therapies delivered through a single administration, we believe the value proposition for patients, families, providers and payors would be significant.

## Our product candidate pipeline

Below is a summary of key information on our development programs:

Product/ Territories	Program Area	Preclinical	Phase I/II	Phase II/III	Status
Lenti-D Worldwide	CNS Diseases				
	Childhood Cerebral ALD - ALD-102 Study*	▶			• IND Active • Initiate Late 2013
	Adult Cerebral ALD	▶			
LentiGlobin® Worldwide	Hematologic Diseases				
	B-Thalassemia/SCD (France) - HGB-205 Study**	▶			• CTA Active • Initiate Mid-2013
	B-Thalassemia (U.S.) - HGB-204 Study**	▶			• IND Active • Initiate Mid-2013
CAR T Cells Global Collaboration	Oncology				
	Hematologic Malignancies	▶			
	Solid Tumors	▶			

\* The Phase II/III ALD-102 Study is our first clinical study of our current Lenti-D viral vector and product candidate. See “Business—Our Lenti-D product candidate.”

\*\* The Phase I/II HGB-205 and HGB-204 Studies are our first clinical studies of our current LentiGlobin viral vector and product candidate. See “Business—Our LentiGlobin product candidate.”

## **Our Lenti-D product candidate**

Our most advanced product candidate is called Lenti-D, which we are developing initially to treat patients with CCALD, a rare, hereditary neurological disorder affecting young boys that is often fatal. CCALD is caused by mutations in the ABCD1 gene, which encodes for a protein called the ALD protein, or ALDP, which in turn plays a critical role in the breakdown and metabolism of very long-chain fatty acids, or VLCFA. Without functional ALDP, VLCFA accumulate in cells, including neural cells, which causes damage to the myelin sheath, a protective and insulating membrane that surrounds nerve cells in the brain. CCALD is characterized by progressive destruction of myelin, leading to severe loss of neurological function and eventual death. The incidence rate for adrenoleukodystrophy, or ALD, the superset of CCALD, is approximately one in 20,000 newborn males, and the U.S. National Institute of Health, or NIH, estimates a prevalence of one in 20,000; CCALD accounts for about 30-40% of patients diagnosed with ALD.

Our approach involves the *ex vivo* insertion of a functional copy of the ABCD1 gene via an HIV-1 based lentiviral vector into the patient's own HSCs to correct the aberrant expression of ALDP in patients with CCALD. HSCs derived from the patient's own body are called autologous HSCs. We refer to autologous HSCs that have been modified to carry the functional copy of the ABCD1 gene as the final Lenti-D drug product, or our Lenti-D product candidate.

We performed a non-interventional retrospective data collection study, called the ALD-101 Study, from a total of 136 CCALD patients to assess the course of disease in patients who were left untreated and patients who received allogeneic HSCT. A non-interventional retrospective data collection study involves an examination of historical clinical records from patients with the pertinent condition in order to assess the typical course of the condition and the efficacy and safety of treatment options. We believe the ALD-101 Study is the most comprehensive natural history study ever conducted to characterize clinical outcomes in CCALD. Our analysis identified the Neurological Function Score, or NFS, Loes Score and gadolinium enhancement as the three most common cognitive, behavioral, functional and radiological modalities utilized to assess patients with CCALD. A comparison of data from treated and untreated patient cohorts in this data collection study provided a framework with which to correlate patterns in these modalities with the eventual stabilization or progression of disease in these patients. We believe the results of this study support our approach of using autologous, gene-modified HSCs to treat CCALD, especially in light of several significant safety concerns commonly associated with the current standard of care, allogeneic HSCT. Results from a Phase I/II study in four patients with CCALD conducted by our scientific collaborators in France with an earlier generation lentiviral vector supplied by a third party provide additional proof-of-concept support for our approach, and were helpful in the design of our own trials to evaluate the efficacy and safety of Lenti-D.

In April 2013, the U.S. Food and Drug Administration, or the FDA, informed us that the Investigational New Drug application, or IND, we filed in March 2013 for a Phase II/III clinical study to evaluate our Lenti-D product candidate in preserving neurological function and stabilizing cerebral demyelination in subjects with CCALD, which we refer to as the ALD-102 Study, is now active. Up to 15 patients will be enrolled to obtain at least 12 evaluable subjects that will be followed over a 24-month period for the onset of major functional disabilities, or MFDs, and other key assessments of disease progression. We expect to initiate the ALD-102 Study in the United States in late 2013. If successful, we believe the results of this study could support

submission of a Biologics License Application, or BLA, and a Marketing Authorization Application, or MAA, filing for our Lenti-D product candidate; however, there can be no assurance that regulatory agencies will not require one or more additional clinical studies prior to granting regulatory approval.

### **Our LentiGlobin product candidate**

Our next most advanced product candidate is called LentiGlobin, which we are developing to treat patients with  $\beta$ -thalassemia and SCD.  $\beta$ -thalassemia is a rare hereditary blood disorder caused by a genetic abnormality of the  $\beta$ -globin gene resulting in defective red blood cells. Symptoms of  $\beta$ -thalassemia can include severe anemia, splenomegaly, marrow expansion, bone deformities and iron overload in major organs. The total annual incidence of symptomatic individuals is estimated at one in 100,000 throughout the world and one in 10,000 in the European Union. SCD is a hereditary blood disorder resulting from a mutation in the  $\beta$ -globin gene that causes polymerization of hemoglobin proteins and abnormal red blood cell function. SCD is characterized by anemia, vaso-occlusive crisis (a common complication of SCD in which there is severe pain due to obstructed blood flow in the bones, joints, lungs, liver, spleen, kidney, eye, or central nervous system), infections, stroke, overall poor quality of life and early death in a large subset of patients. The global incidence of SCD is estimated to be 250,000-300,000 births annually, and the global prevalence of the disease is estimated to be about 20-25 million.

Our approach involves the insertion of a single codon variant of the normal  $\beta$ -globin gene, referred to as T87Q, into the patient's own HSCs via an HIV-1 based lentiviral vector to restore expression of the  $\beta$ -globin protein required for hemoglobin production. The codon variant is also used as a biomarker to quantify expression levels of  $\beta$ -globin protein derived from the vector ( $\beta^{A-T87Q}$ -globin), and provides strong anti-sickling properties in the context of SCD. We refer to the gene-modified HSCs as the final LentiGlobin drug product, or our LentiGlobin product candidate.

In a Phase I/II study of patients with  $\beta$ -thalassemia major being conducted by our scientific collaborators in France with an earlier generation of our LentiGlobin vector called HPV569, data have provided initial evidence of transfusion independence following treatment with gene modified HSCs. Going forward, we plan to use our new LentiGlobin vector for our studies based on higher transduction efficiency and expression of  $\beta$ -globin protein in target cells as compared to the HPV569 vector. We expect to initiate this study in France the first half of 2013 using a revised clinical protocol based on the use of LentiGlobin instead of HPV569. This Phase I/II continuation study, which we refer to as the HGB-205 Study, will enroll up to seven additional subjects with  $\beta$ -thalassemia major or SCD to evaluate transfusion requirements post-transplant, as well as the number of hospitalization days post-transplant discharge. In SCD patients only, efficacy will also be measured based on the number of vaso-occlusive crises or acute chest syndrome events.

We also expect to initiate in mid-2013 a Phase I/II clinical study in the United States to evaluate our LentiGlobin product candidate in increasing hemoglobin production and eliminating or reducing transfusion dependence in patients with  $\beta$ -thalassemia major, which we refer to as the HGB-204 Study. Up to 15 adults will be enrolled to evaluate production of hemoglobin containing  $\beta^{A-T87Q}$ -globin for the six-month period between 18 and 24 months post-transplant, followed by long-term monitoring to assess safety and efficacy beyond the initial 24 months.

## **Our strategic alliance with Celgene**

In March 2013, we announced a global strategic collaboration with Celgene Corporation to discover, develop and commercialize novel, disease-altering gene therapies in oncology. The collaboration will focus on applying gene therapy technology to genetically modify a patient's own T cells to target and destroy cancer cells. Such modified T cells, which are called chimeric antigen receptor, or CAR, cells, have been shown to have beneficial effects in human clinical trials for patients with B cell lymphomas. The multi-year research and development collaboration has the potential to lead to the development and commercialization of multiple CAR T cell products. See "Business—Our strategic alliance with Celgene."

## **Our strategy**

Our objective is to develop and commercialize a next generation of products based on the transformative potential of gene therapy to treat patients with severe genetic and orphan diseases. Central to this effort is a collective determination within our Company to provide these patients with hope for a better life in the face of limited or no long-term safe and effective treatment options. Specifically, our business strategy is based on the following principles:

- Relentlessly focus on serving our patients.
- Be the world's biggest gene therapy geeks, with world-class expertise in the field of gene therapy.
- Leverage our platform and technical expertise to build a gene therapy product engine for severe genetic and orphan diseases.
- Develop and commercialize drugs in our core disease areas and partner selectively to expand the scope of our pipeline.
- Pursue indications with high unmet medical need and greater probability of clinical, regulatory and commercial success.

## **Risks related to our business**

Our ability to implement our business strategy is subject to numerous risks that you should be aware of before making an investment decision. These risks are described more fully in the section entitled "Risk factors" immediately following this prospectus summary. These risks include, among others:

- We have incurred significant losses since our inception, which we anticipate will continue for the foreseeable future. We have never generated revenue from product sales and may never be profitable.
- Failure to obtain additional funding when needed may force us to delay, limit or terminate our product development efforts or other operations.
- Our gene therapy product candidates are based on a novel technology, which makes it difficult to predict the time and cost of product candidate development and subsequently for obtaining regulatory approval.

- We may find it difficult to enroll patients in our clinical studies, which could delay or prevent clinical studies of our product candidates.
- If our product candidates fail to demonstrate safety and efficacy to the satisfaction of regulatory authorities we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.
- No gene therapy products have been approved in the United States and only one product has been approved in Europe.
- Neither our current viral vectors nor our product candidates have ever been evaluated in human clinical studies, and we may experience unexpected results in the future.
- In previous clinical studies involving viral vectors for gene therapy, some subjects experienced serious adverse events, including the development of leukemia due to vector-related insertional oncogenesis.
- We expect to rely on third parties to conduct the majority of our current vector production, product manufacturing and clinical development. If they fail to meet deadlines or perform in an unsatisfactory manner our business could be harmed.
- The commercial success of any current or future product candidate will depend upon the degree of market acceptance by physicians, patients, third-party payors and others in the medical community.
- If we are unable to obtain or protect intellectual property rights related to our product candidates, we may not be able to compete effectively in our markets.
- Provisions in our collaboration agreement with Celgene Corporation may prevent or delay a change in control.

### **Corporate information**

We were incorporated in Delaware in April 1992 under the name Genetix Pharmaceuticals, Inc., and subsequently changed our name to bluebird bio, Inc. in September 2010. Our principal executive offices are located at 840 Memorial Drive, 4th Floor, Cambridge, MA 02139, and our telephone number is (617) 491-5601. Our website address is [www.bluebirdbio.com](http://www.bluebirdbio.com). The information contained in, or that can be accessed through, our website is not part of this prospectus.

We are an “emerging growth company,” as defined in the Jumpstart Our Business Startups Act of 2012. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of this offering, (b) in which we have total annual gross revenue of at least \$1.0 billion or (c) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds \$700 million as of the prior June 30th, and (2) the date on which we have issued more than \$1.0 billion in non-convertible debt during the prior three-year period.

We use “Lenti-D” and the bluebird bio logo as trademarks in the United States and other countries. We use and have registered “LentiGlobin” and “bluebird bio” in the United States.

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This prospectus contains references to our trademarks and to trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to in this prospectus, including logos, artwork, and other visual displays, may appear without the ® or ™ symbols, but such references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensor to these trademarks and trade names. We do not intend our use or display of other companies' trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other companies. Except where the context requires otherwise, in this prospectus "Company," "bluebird," "we," "us" and "our" refer to bluebird bio, Inc.

## The offering

**Common stock offered by us**                      shares

**Common stock to be outstanding after this offering**                      shares

**Option to purchase additional shares**                      The underwriters have an option for a period of 30 days to purchase up to                      additional shares of our common stock.

**Use of proceeds**                      We estimate that the net proceeds from this offering will be approximately \$                      million, or approximately \$                      million if the underwriters exercise their option to purchase additional shares in full, at an assumed initial public offering price of \$                      per share, the midpoint of the price range set forth on the cover of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds from this offering to fund direct research and development expenses for our Phase II/III clinical study for our Lenti-D product candidate and our Phase I/II clinical studies for our LentiGlobin product candidate. We intend to use remaining amounts for general and administrative expenses (including personnel-related costs), potential future development programs, early-stage research and development, capital expenditures and working capital and other general corporate purposes. We may also use a portion of the net proceeds to in-license, acquire or invest in complementary gene therapy businesses, technologies, products or assets. See "Use of proceeds."

**Risk factors**                      You should read the "Risk factors" section of this prospectus for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.

### **Proposed Nasdaq Global Market BLUE symbol**

The number of shares of common stock to be outstanding after this offering is based on 6,599,419 shares of common stock outstanding as of March 31, 2013, which excludes 2,506,114 shares of unvested restricted stock subject to repurchase by us and 310,841,204 additional shares of our common stock issuable upon conversion of all of our outstanding shares of preferred stock upon closing of this offering.

The number of shares of our common stock to be outstanding after this offering excludes the following:

- 69,284,748 shares of common stock issuable upon the exercise of outstanding stock options as of March 31, 2013 having a weighted-average exercise price of \$0.19 per share;



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- 8,352,387 shares of common stock issuable upon the exercise of outstanding warrants as of March 31, 2013 having a weighted-average exercise price of \$0.49 per share;
- 10,354,017 shares of common stock reserved for issuance pursuant to future equity awards under our 2010 Stock Option and Grant Plan, which will become available for issuance under our 2013 Stock Option and Incentive Plan immediately prior to this offering; and
- \_\_\_\_\_ shares of common stock reserved for issuance pursuant to future equity awards under our 2013 Stock Option and Incentive Plan, which will become effective immediately prior to this offering.

Except as otherwise indicated, all information contained in this prospectus:

- reflects the conversion of all of our outstanding shares of preferred stock into an aggregate of 310,841,204 shares of common stock prior to the completion of this offering;
- assumes the adoption of our amended and restated certificate of incorporation and amended and restated by-laws upon the completion of this offering;
- assumes that the underwriters do not exercise their option to purchase additional shares;
- assumes no exercise of outstanding options or warrants after March 31, 2013; and
- reflects a one-for-\_\_\_\_\_ reverse stock split of our common stock that will become effective prior to the effectiveness of the registration statement of which this prospectus forms a part.

## Summary consolidated financial data

The following summary consolidated financial data for the years ended December 31, 2012 and 2011 are derived from our audited consolidated financial statements appearing elsewhere in this prospectus. The summary consolidated financial data as of March 31, 2013 and for the three months ended March 31, 2012 and 2013 have been derived from our unaudited consolidated financial statements included elsewhere in this prospectus. In our opinion, these unaudited financial statements have been prepared on a basis consistent with our audited consolidated financial statements and contain all adjustments, consisting only of normal and recurring adjustments, necessary for a fair presentation of such consolidated financial data. You should read this data together with our audited consolidated financial statements and related notes appearing elsewhere in this prospectus and the information under the captions "Selected consolidated financial data" and "Management's discussion and analysis of financial condition and results of operations." Our historical results are not necessarily indicative of our future results, and our operating results for the three-month period ended March 31, 2013 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2013 or any other interim periods or any future year or period.

(in thousands, except per share data)	Year ended December 31,		Three months ended March 31,	
	2011	2012	2012	2013
			(unaudited)	
<b>Consolidated statements of operations data:</b>				
Revenue:				
Collaboration revenue	\$ —	\$ —	\$ —	\$ 1,042
Research and license fees	640	340	85	85
Grant revenue	242	—	—	—
	<u>882</u>	<u>340</u>	<u>85</u>	<u>1,127</u>
Expenses:				
Research and development	11,409	17,210	3,858	5,284
General and administrative	4,615	6,846	1,363	2,324
Total expenses	<u>16,024</u>	<u>24,056</u>	<u>5,221</u>	<u>7,608</u>
Loss from operations	(15,142)	(23,716)	(5,136)	(6,481)
Other income (expense), net	(456)	46	68	(63)
Net loss	<u>\$ (15,598)</u>	<u>\$ (23,670)</u>	<u>\$ (5,068)</u>	<u>\$ (6,544)</u>
Net loss per share applicable to common stockholders —basic and diluted(1)	<u>\$ (9.01)</u>	<u>\$ (0.73)</u>	<u>\$ (1.50)</u>	<u>\$ (1.05)</u>
Weighted-average number of common shares used in net loss per share applicable to common stockholders—basic and diluted	<u>2,285</u>	<u>4,972</u>	<u>4,236</u>	<u>6,226</u>
Pro forma net loss per share applicable to common stockholders—basic and diluted (unaudited)(1)		<u>\$ (0.10)</u>		<u>\$ (0.02)</u>
Pro forma weighted-average number of common shares used in net loss per share applicable to common stockholders—basic and diluted (unaudited)		<u>248,700</u>		<u>317,067</u>

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(in thousands)	As of March 31, 2013		
	Actual	Pro Forma(2)	Pro Forma Adjusted (3)(4)
	(unaudited)		
<b>Consolidated balance sheet data:</b>			
Cash and cash equivalents	\$131,836	\$ 131,836	
Working capital	105,390	105,390	
Total assets	137,459	137,459	
Preferred stock	122,177	—	
Common stock and additional paid-in capital	15,966	138,399	
Total stockholders' (deficit) equity	(61,595)	58,501	
<p>(1) See Notes 2 and 15 within the notes to our consolidated financial statements appearing elsewhere in this prospectus for a description of the method used to calculate basic and diluted net loss per share of common stock and pro forma basic and diluted net loss per share of common stock.</p> <p>(2) Pro forma to reflect the conversion of all outstanding shares of our preferred stock into shares of our common stock, and the reclassification of our outstanding warrants to purchase our Series B preferred stock to our common stock, upon the closing of this offering.</p> <p>(3) Pro forma as adjusted to further reflect the sale of shares of our common stock offered in this offering, assuming an initial public offering price of \$ per share, the midpoint of the price range set forth on the cover of this prospectus, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.</p> <p>(4) A \$1.00 increase (decrease) in the assumed initial public offering price of \$ per share, the midpoint of the price range set forth on the cover of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents and total stockholders' (deficit) equity by approximately \$ million, assuming that the number of shares offered by us, as set forth on the cover of this prospectus, remains the same and after deducting underwriting discounts and commissions and estimated offering expenses payable by us. A share increase in the number of shares offered by us together with a concomitant \$1.00 increase in the assumed initial public offering price of \$ per share, the midpoint of the price range set forth on the cover of this prospectus, would increase each of cash and cash equivalents and total stockholders' (deficit) equity by approximately \$ million after deducting underwriting discounts and commissions and any estimated offering expenses payable by us. Conversely, a share decrease in the number of shares offered by us together with a concomitant \$1.00 decrease in the assumed initial public offering price of \$ per share, the midpoint of the price range set forth on the cover of this prospectus, would decrease each of cash and cash equivalents and total stockholders' (deficit) equity by approximately \$ million after deducting underwriting discounts and commissions and any estimated offering expenses payable by us.</p>			

## Risk factors

*An investment in shares of our common stock involves a high degree of risk. You should carefully consider the following information about these risks, together with the other information appearing elsewhere in this prospectus, including our financial statements and related notes thereto, before deciding to invest in our common stock. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition, results of operations and future growth prospects. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment.*

### **Risks related to our financial condition and capital requirements**

***We have incurred significant losses since our inception and anticipate that we will continue to incur significant losses for the foreseeable future.***

We are a clinical-stage biotechnology company, and we have not yet generated significant revenues. We have incurred net losses in each year since our inception in 1992, including net losses of \$15.6 million and \$23.7 million for the years ended December 31, 2011 and 2012, respectively, and \$6.5 million for the three months ended March 31, 2013. As of March 31, 2013, we had an accumulated deficit of \$79.9 million.

We have devoted most of our financial resources to research and development, including our clinical and preclinical development activities. To date, we have financed our operations primarily through the sale of equity securities and convertible debt and, to a lesser extent, through grants from governmental agencies and charitable foundations. The amount of our future net losses will depend, in part, on the rate of our future expenditures and our ability to obtain funding through equity or debt financings, strategic collaborations or additional grants. We have not completed pivotal clinical studies for any product candidate and it will be several years, if ever, before we have a product candidate ready for commercialization. Even if we obtain regulatory approval to market a product candidate, our future revenues will depend upon the size of any markets in which our product candidates have received approval, and our ability to achieve sufficient market acceptance, reimbursement from third-party payors and adequate market share for our product candidates in those markets.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if and as we:

- continue our research and preclinical and clinical development of our product candidates;
- expand the scope of our current clinical studies for our product candidates;
- initiate additional preclinical, clinical or other studies for our product candidates, including under our collaboration agreement with Celgene Corporation;
- further develop the manufacturing process for our vectors or our product candidates;
- change or add additional manufacturers or suppliers;
- seek regulatory and marketing approvals for our product candidates that successfully complete clinical studies;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- seek to identify and validate additional product candidates;

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- acquire or in-license other product candidates and technologies;
- make milestone or other payments under any in-license agreements;
- maintain, protect and expand our intellectual property portfolio;
- attract and retain skilled personnel;
- create additional infrastructure to support our operations as a public company and our product development and planned future commercialization efforts; and
- experience any delays or encounter issues with any of the above.

The net losses we incur may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance. In any particular quarter or quarters, our operating results could be below the expectations of securities analysts or investors, which could cause our stock price to decline.

***We have never generated any revenue from product sales and may never be profitable.***

Our ability to generate revenue and achieve profitability depends on our ability, alone or with strategic collaboration partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize our product candidates. We do not anticipate generating revenues from product sales for the foreseeable future, if ever. Our ability to generate future revenues from product sales depends heavily on our success in:

- completing research and preclinical and clinical development of our product candidates;
- seeking and obtaining regulatory and marketing approvals for product candidates for which we complete clinical studies;
- developing a sustainable, scalable, reproducible, and transferable manufacturing process for our vectors and product candidates;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate (in amount and quality) products and services to support clinical development and the market demand for our product candidates, if approved;
- launching and commercializing product candidates for which we obtain regulatory and marketing approval, either by collaborating with a partner or, if launched independently, by establishing a sales force, marketing and distribution infrastructure;
- obtaining market acceptance of our product candidates and gene therapy as a viable treatment option;
- addressing any competing technological and market developments;
- implementing additional internal systems and infrastructure, as needed;
- identifying and validating new gene therapy product candidates;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter;

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- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how; and
- attracting, hiring and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the U.S. Food and Drug Administration, or the FDA, the European Medicines Agency, or the EMA, or other regulatory agencies, domestic or foreign, to perform clinical and other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations.

***Even if this offering is successful, we will need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.***

We are currently advancing our Lenti-D and LentiGlobin product candidates through clinical development and other product candidates through preclinical development. Developing gene therapy products is expensive, and we expect our research and development expenses to increase substantially in connection with our ongoing activities, particularly as we advance our product candidates in clinical studies.

As of March 31, 2013, our cash and cash equivalents were \$131.8 million. We estimate that the net proceeds from this offering will be approximately \$ million, assuming an initial public offering price of \$ per share, the midpoint of the price range set forth on the cover of this prospectus, after deducting estimated underwriting discounts and commissions and offering expenses payable by us. We expect that the net proceeds from this offering and our existing cash and cash equivalents will be sufficient to fund our current operations through at least the end of 2015. However, our operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches. In any event, we will require additional capital to obtain regulatory approval for, and to commercialize, our product candidates. Raising funds in the current economic environment may present additional challenges. Even if we believe we have sufficient funds for our current or future operating plans, we may seek additional capital if market conditions are favorable or if we have specific strategic considerations.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The sale of additional equity or convertible securities would dilute all of our stockholders. The incurrence of indebtedness would result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our

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ability to conduct our business. We could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects.

If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product candidates or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition and results of operations.

## **Risks related to the discovery and development of our product candidates**

***Our gene therapy product candidates are based on a novel technology, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval. At the moment, no gene therapy products have been approved in the United States and only one product has been approved in Europe.***

We have concentrated our therapeutic product research and development efforts on our gene therapy platform, and our future success depends on the successful development of this therapeutic approach. There can be no assurance that any development problems we experience in the future related to our gene therapy platform will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays in developing a sustainable, reproducible and scalable manufacturing process or transferring that process to commercial partners, which may prevent us from completing our clinical studies or commercializing our products on a timely or profitable basis, if at all.

In addition, the clinical study requirements of the FDA, the EMA and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied pharmaceutical or other product candidates. At the moment, only one gene therapy product, UniQure's Glybera, which received marketing authorization from the EMA in 2012, has been approved in the Western world, which makes it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in either Europe or the United States. Approvals by the EMA may not be indicative of what the FDA may require for approval.

Regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future. For example, the FDA has established the Office of Cellular, Tissue and Gene Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene therapy clinical studies conducted at institutions that receive funding for recombinant DNA research from the U.S. National Institutes of Health, or the NIH, are also subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC review process can impede the initiation of a clinical study, even if the FDA has reviewed the study and approved its

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initiation. Conversely, the FDA can put an investigational new drug application, or IND, on clinical hold even if the RAC has provided a favorable review. Also, before a clinical study can begin at an NIH-funded institution, that institution's institutional review board, or IRB, and its Institutional Biosafety Committee will have to review the proposed clinical study to assess the safety of the study. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other regulatory bodies to change the requirements for approval of any of our product candidates.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of our product candidates. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue to maintain our business.

***We may find it difficult to enroll patients in our clinical studies, which could delay or prevent clinical studies of our product candidates.***

Identifying and qualifying patients to participate in clinical studies of our product candidates is critical to our success. The timing of our clinical studies depends on the speed at which we can recruit patients to participate in testing our product candidates. We have experienced delays in some of our clinical studies, and we may experience similar delays in the future. If patients are unwilling to participate in our gene therapy studies because of negative publicity from adverse events in the biotechnology or gene therapy industries or for other reasons, including competitive clinical studies for similar patient populations, the timeline for recruiting patients, conducting studies and obtaining regulatory approval of potential products may be delayed. These delays could result in increased costs, delays in advancing our product development, delays in testing the effectiveness of our technology or termination of the clinical studies altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics to achieve diversity in a study, to complete our clinical studies in a timely manner. Patient enrollment is affected by factors including:

- severity of the disease under investigation;
- design of the study protocol;
- size of the patient population;
- eligibility criteria for the study in question;
- perceived risks and benefits of the product candidate under study;
- proximity and availability of clinical study sites for prospective patients;
- availability of competing therapies and clinical studies;
- efforts to facilitate timely enrollment in clinical studies;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.



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In particular, each of the conditions for which we plan to evaluate our current product candidates are rare genetic disorders with limited patient pools from which to draw for clinical studies. The total annual incidence of  $\beta$ -thalassemia is estimated at one in 100,000 throughout the world and one in 10,000 in the European Union, and the global incidence of SCD is estimated to be 250,000-300,000 births annually with a global prevalence estimated to be about 20-25 million. The incidence rate for adrenoleukodystrophy, or ALD, the superset of CCALD, is approximately one in 20,000 newborn males, and the U.S. National Institute of Health, or NIH, estimates a prevalence of one in 20,000. CCALD accounts for about 30-40% of patients diagnosed with ALD. Further, because newborn screening for CCALD is not widely adopted, and it can be difficult to diagnose CCALD in the absence of a genetic screen, we may have difficulty finding patients who are eligible to participate in our study. The eligibility criteria of our clinical studies will further limit the pool of available study participants. Additionally, the process of finding and diagnosing patients may prove costly. Finally, our treatment process requires that the patient be near one of our transduction facilities, as the hematopoietic stem cells, or HSCs, have limited viability following harvest and cannot be transported long distances.

Our current product candidates are being developed to treat rare conditions. We plan to seek initial marketing approval in the United States and Europe. We may not be able to initiate or continue clinical studies if we cannot enroll a sufficient number of eligible patients to participate in the clinical studies required by the FDA or the EMA or other regulatory agencies. Our ability to successfully initiate, enroll and complete a clinical study in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with contract research organizations, or CROs, and physicians;
- different standards for the conduct of clinical studies;
- our inability to locate qualified local consultants, physicians and partners; and
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical studies as planned, we may need to delay, limit or terminate ongoing or planned clinical studies, any of which would have an adverse effect on our business.

***We may encounter substantial delays in our clinical studies or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.***

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical studies to demonstrate the safety and efficacy of the product candidates in humans. Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical studies will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical studies can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory agencies on study design;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical study sites;

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- delays in obtaining required Institutional Review Board, or IRB, approval at each clinical study site;
- delays in recruiting suitable patients to participate in our clinical studies;
- imposition of a clinical hold by regulatory agencies, after an inspection of our clinical study operations or study sites;
- failure by our CROs, other third parties or us to adhere to clinical study requirements;
- failure to perform in accordance with the FDA's good clinical practices, or GCP, or applicable regulatory guidelines in other countries;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites;
- delays in having patients complete participation in a study or return for post-treatment follow-up;
- clinical study sites or patients dropping out of a study;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical study delays could also shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

If the results of our clinical studies are inconclusive or if there are safety concerns or adverse events associated with our product candidates, we may:

- be delayed in obtaining marketing approval for our product candidates, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes with the way the product is administered;
- be required to perform additional clinical studies to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation strategy;

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- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

Treatment with our product candidates involves chemotherapy and myeloablative treatments, which can cause side effects or adverse events that are unrelated to our product candidate, but may still impact the success of our clinical studies. Additionally, our product candidates could potentially cause other adverse events that have not yet been predicted. The inclusion of critically ill patients in our clinical studies may result in deaths or other adverse medical events due to other therapies or medications that such patients may be using. As described above, any of these events could prevent us from achieving or maintaining market acceptance of our product candidates and impair our ability to commercialize our products.

***We have not tested any of our current viral vectors or product candidates derived from these viral vectors in clinical studies. Success in early clinical studies may not be indicative of results obtained in later studies.***

Neither our current viral vectors nor our product candidates have ever been evaluated in human clinical studies, and we may experience unexpected results in the future. Earlier gene therapy clinical studies, which we believe serve as proof-of-concept for our product candidates, utilized lentiviral vectors similar to ours. However, these studies should not be relied upon as evidence that our future clinical studies will succeed. Study designs and results from previous studies are not necessarily predictive of our future clinical study designs or results, and initial results may not be confirmed upon full analysis of the complete study data. Our product candidates may also fail to show the desired safety and efficacy in later stages of clinical development despite having successfully advanced through initial clinical studies.

There is a high failure rate for drugs and biologics proceeding through clinical studies. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in later stage clinical studies even after achieving promising results in earlier stage clinical studies. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, regulatory delays or rejections may be encountered as a result of many factors, including changes in regulatory policy during the period of product development.

***The results from our ALD-102 Study may not be sufficiently robust to support the submission of marketing approval for our Lenti-D product candidate. Before we submit Lenti-D for marketing approval, the FDA and the EMA may require us to conduct additional clinical studies, or evaluate subjects for an additional follow-up period.***

The FDA has advised us that our ALD-102 Study, which is a single-arm, open-label study to evaluate the safety and efficacy of our Lenti-D product candidate to halt the progression of CCALD, may not be deemed to be a pivotal study or may not provide sufficient support for a Biologics License Application, or BLA, submission. The FDA normally requires two pivotal clinical studies to approve a drug or biologic product, and thus the FDA may require that we conduct additional clinical studies of Lenti-D prior to a BLA submission. The FDA typically does not consider a single clinical study to be adequate to serve as a pivotal study unless it is, among other things, well-controlled and demonstrates a clinically meaningful effect on mortality, irreversible morbidity, or prevention of a disease with potentially serious outcome, and a confirmatory study

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would be practically or ethically impossible. Due to the nature of CCALD and the limited number of patients with this condition, a placebo-controlled and blinded study is not practicable for ethical and other reasons. However, it is still possible that, even if we achieve favorable results in the ALD-102 Study, the FDA may require us to conduct a second clinical study, possibly involving a larger sample size or a different clinical study design, particularly if the FDA does not find the results from the ALD-102 Study to be sufficiently persuasive to support a BLA submission. The FDA may also require that we conduct a longer follow-up period of subjects treated with our Lenti-D product candidate prior to accepting our BLA submission.

In addition, the ALD-102 Study was not designed to achieve a statistically significant efficacy determination. Rather, we expect that safety and efficacy will be evaluated in light of the data collected in our retrospective data collection study, the ALD-101 Study. However, due to the nature of this retrospective data collection study, and the limited number of patients with this condition, the FDA has advised us that the ALD-101 Study is not sufficiently robust to serve as a conventional historical control group and as a basis of comparison against the results of the ALD-102 Study. Thus, we expect that the FDA will assess the totality of the safety and efficacy data from our ALD-102 Study in reviewing any future BLA submission for our Lenti-D product candidate. Based on this assessment, the FDA may require that we conduct additional preclinical or clinical studies prior to submitting or approving a BLA for this indication.

It is possible that the FDA or the EMA may not consider the results of this study to be sufficient for approval of Lenti-D for this indication. If the FDA or the EMA requires additional studies, we would incur increased costs and delays in the marketing approval process, which may require us to expend more resources than we have available. In addition, it is possible that the FDA and the EMA may have divergent opinions on the elements necessary for a successful BLA and Marketing Authorization Application, or MAA, respectively, which may cause us to alter our development, regulatory and/or commercialization strategies.

***In previous clinical studies involving viral vectors for gene therapy, some subjects experienced serious adverse events, including the development of leukemia due to vector-related insertional oncogenesis. If our vectors demonstrate a similar effect, we may be required to halt or delay further clinical development of our product candidates.***

A significant risk in any gene therapy product based on viral vectors is that the vector will insert near cancer-causing oncogenes leading to uncontrolled clonal proliferation of mature cancer cells in the patient. For example, in 2003, 20 subjects treated for X-linked severe combined immunodeficiency in two gene therapy studies using a murine gamma-retroviral vector showed correction of the disease, but the studies were terminated after five subjects developed leukemia (four of whom were subsequently cured). The cause of these adverse events was shown to be insertional oncogenesis, which is the process whereby the corrected gene inserts near a gene that is important in a critical cellular process like growth or division, and this insertion results in the development of a cancer (often leukemia). Using molecular diagnostic techniques, it was determined that clones from these subjects showed retrovirus insertion in proximity to the promoter of the LMO2 proto-oncogene. Earlier generation retroviruses like the one used in these two studies have been shown to preferentially integrate in regulatory regions of genes that control cell growth.

These well-publicized adverse events led to the development of new viral vectors, such as lentiviral vectors, with improved safety profiles. In published studies, lentiviral vectors have demonstrated an improved safety profile over gamma-retroviral vectors, with no known events

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of gene therapy-related adverse events, which we believe is due to a number of factors including the tendency of these vectors to integrate within genes rather than in areas that control gene expression, as well as their lack of strong viral enhancers. However, it should be noted that in our Phase I/II study (the LG001 Study) of autologous HSCs transduced *ex vivo* using an earlier generation of our LentiGlobin vector, called HPV569, we initially observed in one patient that a disproportionate number of the cells expressing our functional gene had the same insertion site. Tests showed that this partial clonal dominance contained an insertion of the functional gene in the HMGA2 gene that persisted for a period of two to three years. Although there was some initial concern that the observed clonal dominance might represent a pre-leukemic event, there have been no adverse clinical consequences of this event, or any signs of cancer, in over five years since the observation was made. The presence of the HMGA2 clone has steadily declined in this patient over time to the point that it is no longer the most common clone observed in this patient.

The risk of insertional oncogenesis remains a significant concern for gene therapy and we cannot assure that it will not occur in any of our planned or future clinical studies. There is also the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. The FDA has stated that lentiviral vectors possess characteristics that may pose high risks of delayed adverse events. If any such adverse events occur, further advancement of our clinical studies could be halted or delayed, which would have a material adverse effect on our business and operations.

***Even if we complete the necessary preclinical and clinical studies, we cannot predict when or if we will obtain regulatory approval to commercialize a product candidate or the approval may be for a more narrow indication than we expect.***

We cannot commercialize a product until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if our product candidates demonstrate safety and efficacy in clinical studies, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical studies and the review process. Regulatory agencies also may approve a treatment candidate for fewer or more limited indications than requested or may grant approval subject to the performance of post-marketing studies. In addition, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our treatment candidates. For example, the development of our product candidates for pediatric use is an important part of our current business strategy, and if we are unable to obtain regulatory approval for the desired age ranges, our business may suffer.

***Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory scrutiny.***

Even if we obtain regulatory approval in a jurisdiction, the regulatory authority may still impose significant restrictions on the indicated uses or marketing of our product candidates, or impose ongoing requirements for potentially costly post-approval studies or post-market surveillance. For example, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. The FDA

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typically advises that patients treated with gene therapy undergo follow-up observations for potential adverse events for a 15-year period. The holder of an approved BLA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with good manufacturing practices, or GMP, and adherence to commitments made in the BLA. If we or a regulatory agency discovers previously unknown problems with a product such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions relative to that product or the manufacturing facility, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of any of our product candidates, a regulatory agency may:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical studies;
- refuse to approve a pending BLA or supplements to a BLA submitted by us;
- seize product; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and generate revenues.

### **Risks related to our reliance on third parties**

***We expect to rely on third parties to conduct some or all aspects of our vector production, product manufacturing, protocol development, research and preclinical and clinical testing, and these third parties may not perform satisfactorily.***

We do not expect to independently conduct all aspects of our vector production, product manufacturing, protocol development, research and preclinical and clinical testing. We currently rely, and expect to continue to rely, on third parties with respect to these items.

Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it could delay our product development activities. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations and study protocols. For example, for product candidates that we develop and commercialize on our own, we will remain responsible for ensuring that each of our IND-enabling studies and clinical studies are conducted in accordance with the study plan and protocols.

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If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, we will not be able to complete, or may be delayed in completing, the preclinical and clinical studies required to support future IND submissions and approval of our product candidates.

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured the product candidates ourselves, including:

- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- reduced control as a result of using third-party manufacturers for all aspects of manufacturing activities;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers or suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier.

Any of these events could lead to clinical study delays or failure to obtain regulatory approval, or impact our ability to successfully commercialize future products. Some of these events could be the basis for FDA action, including injunction, recall, seizure or total or partial suspension of production.

***We and our contract manufacturers are subject to significant regulation with respect to manufacturing our products. The manufacturing facilities on which we rely may not continue to meet regulatory requirements and have limited capacity.***

We currently have relationships with a limited number of suppliers for the manufacturing of our viral vectors and product candidates. Each supplier may require licenses to manufacture such components if such processes are not owned by the supplier or in the public domain and we may be unable to transfer or sublicense the intellectual property rights we may have with respect to such activities.

All entities involved in the preparation of therapeutics for clinical studies or commercial sale, including our existing contract manufacturers for our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical studies must be manufactured in accordance with GMP. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants, or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of a BLA on a timely basis and must adhere to the FDA's good laboratory practices, or GLP, and GMP regulations enforced by the FDA through its facilities inspection program. Some of our contract manufacturers have not produced a commercially-approved product and therefore have not obtained the requisite FDA approvals to do so. Our facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the

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applicable regulations as a condition of regulatory approval of our product candidates or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will not be granted.

The regulatory authorities also may, at any time following approval of a product for sale, audit our manufacturing facilities or those of our third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical study or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business.

If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new drug product or biologic product, or revocation of a pre-existing approval. As a result, our business, financial condition and results of operations may be materially harmed.

Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in commercial supply. An alternative manufacturer would need to be qualified through a BLA supplement which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause the delay of clinical studies, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical studies may be delayed or we could lose potential revenue.

***We expect to rely on third parties to conduct, supervise and monitor our clinical studies, and if these third parties perform in an unsatisfactory manner, it may harm our business.***

We expect to rely on CROs and clinical study sites to ensure our clinical studies are conducted properly and on time. While we will have agreements governing their activities, we will have limited influence over their actual performance. We will control only certain aspects of our CROs' activities. Nevertheless, we will be responsible for ensuring that each of our clinical studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities.

We and our CROs are required to comply with the FDA's GCPs for conducting, recording and reporting the results of IND-enabling studies and clinical studies to assure that the data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical study participants are protected. The FDA enforces these GCPs through periodic



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inspections of study sponsors, principal investigators and clinical study sites. If we or our CROs fail to comply with applicable GCPs, the clinical data generated in our future clinical studies may be deemed unreliable and the FDA may require us to perform additional clinical studies before approving any marketing applications. Upon inspection, the FDA may determine that our clinical studies did not comply with GCPs. In addition, our future clinical studies will require a sufficient number of test subjects to evaluate the safety and effectiveness of our product candidates. Accordingly, if our CROs fail to comply with these regulations or fail to recruit a sufficient number of patients, we may be required to repeat such clinical studies, which would delay the regulatory approval process.

Our CROs are not our employees, and we are therefore unable to directly monitor whether or not they devote sufficient time and resources to our clinical and nonclinical programs. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical studies or other drug development activities that could harm our competitive position. If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements, or for any other reasons, our clinical studies may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

We also expect to rely on other third parties to store and distribute our vectors and products for any clinical studies that we may conduct. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, if approved, producing additional losses and depriving us of potential product revenue.

***Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.***

Because we rely on third parties to manufacture our vectors and our product candidates, and because we collaborate with various organizations and academic institutions on the advancement of our gene therapy platform, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, such as trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

In addition, these agreements typically restrict the ability of our collaborators, advisors, employees and consultants to publish data potentially relating to our trade secrets. Our academic

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collaborators typically have rights to publish data, provided that we are notified in advance and may delay publication for a specified time in order to secure our intellectual property rights arising from the collaboration. In other cases, publication rights are controlled exclusively by us, although in some cases we may share these rights with other parties. We also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets in cases where we do not have proprietary or otherwise protected rights at the time of publication. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

## **Risks related to commercialization of our product candidates**

***We intend to rely on third-party manufacturers to produce our vector, product candidates and other key materials, but we have not entered into binding agreements with any such manufacturers to support commercialization. Additionally, these manufacturers do not have experience producing our vectors and product candidates at commercial levels and may not achieve the necessary regulatory approvals or produce our vectors and products at the quality, quantities, locations and timing needed to support commercialization.***

We have not yet secured manufacturing capabilities for commercial quantities of our viral vectors or established transduction facilities in the desired commercialization regions to support commercialization of our products. Although we intend to rely on third-party manufacturers for commercialization, we have only entered into agreements with such manufacturers to support our clinical studies. We may be unable to negotiate binding agreements with the manufacturers to support our commercialization activities at commercially reasonable terms.

No manufacturer currently has the experience or ability to produce our vectors and product candidates at commercial levels. We are currently developing a scalable manufacturing process for LentiGlobin, which we plan to transfer to one or more contract manufacturers. We may run into technical or scientific issues related to manufacturing or development that we may be unable to resolve in a timely manner or with available funds. Although we have been able to produce our Lenti-D vector at commercial scale, we have not completed the characterization and validation activities necessary for commercial and regulatory approvals. If our manufacturing partners do not obtain such regulatory approvals, our commercialization efforts will be harmed.

Additionally, since the HSCs have a limited window of stability following extraction from the patient, we must set up transduction facilities in the regions where we wish to commercialize our product. Currently, we rely on academic institutions and one third-party contract manufacturer in the United States and Europe, respectively, to produce our product candidates for our clinical studies. Since a portion of our target patient populations will be outside the United States and Europe, we will need to set up additional transduction facilities that can replicate our transduction process. Establishment of such facilities may be impeded by technical, quality, or regulatory issues related to these new sites and we may also run into technical or scientific issues related to transfer of our transduction process or other developmental issues that we may be unable to resolve in a timely manner or with available funds.

Even if we timely develop a manufacturing process and successfully transfer it to the third-party vector and product manufacturers, if such third-party manufacturers are unable to produce

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the necessary quantities of viral vectors and our product candidates, or in compliance with GMP, or in compliance with pertinent regulatory requirements, and within our planned time frame and cost parameters, the development and sales of our products, if approved, may be materially harmed.

In addition, any significant disruption in our supplier relationships could harm our business. We source key materials from third parties, either directly through agreements with suppliers or indirectly through our manufacturers who have agreements with suppliers. There are a small number of suppliers for certain key materials that are used to manufacture our product candidates. Such suppliers may not sell these key materials to our manufacturers at the times we need them or on commercially reasonable terms. We do not have any control over the process or timing of the acquisition of these key materials by our manufacturers. Moreover, we currently do not have any agreements for the commercial production of these key materials.

***If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any revenues.***

We have no experience selling and marketing our product candidates. To successfully commercialize any products that may result from our development programs, we will need to develop these capabilities, either on our own or with others. We may enter into collaborations with other entities to utilize their mature marketing and distribution capabilities, but we may be unable to enter into marketing agreements on favorable terms, if at all. If our future collaborative partners do not commit sufficient resources to commercialize our future products, if any, and we are unable to develop the necessary marketing capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

***We face intense competition and rapid technological change and the possibility that our competitors may develop therapies that are more advanced or effective than ours, which may adversely affect our financial condition and our ability to successfully commercialize our product candidates.***

We are engaged in gene therapy, which is a rapidly changing field. We have competitors both in the United States and internationally, including major multinational pharmaceutical companies, biotechnology companies and universities and other research institutions. Some of the pharmaceutical and biotechnology companies we expect to compete with include GlaxoSmithKline plc, Sangamo BioSciences Inc., HemaQuest Pharmaceuticals, Inc., Merck & Co., Inc., Novartis AG and GlycoMimetics Inc. In addition, many universities and private and public research institutes are active in our target disease areas.

Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring or licensing on an exclusive basis, products that are more effective or less costly than any product candidate that we may develop, or achieve earlier patent protection, regulatory approval, product commercialization and market penetration than us. Additionally, technologies developed by our competitors may render our

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potential product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors.

Even if we are successful in achieving regulatory approval to commercialize a product candidate faster than our competitors, we may face competition from biosimilars due to the changing regulatory environment. In the United States, the Biologics Price Competition and Innovation Act of 2009 created an abbreviated approval pathway for biological products that are demonstrated to be “highly similar,” or biosimilar, to or “interchangeable” with an FDA-approved biological product. This new pathway could allow competitors to reference data from biological products already approved after 12 years from the time of approval. In his proposed budget for fiscal year 2014, President Obama proposed to cut this 12-year period of exclusivity down to seven years. He also proposed to prohibit additional periods of exclusivity due to minor changes in product formulations, a practice often referred to as “evergreening.” In Europe, the European Commission has granted marketing authorizations for several biosimilars pursuant to a set of general and product class-specific guidelines for biosimilar approvals issued over the past few years. In Europe, a competitor may reference data from biological products already approved, but will not be able to get on the market until ten years after the time of approval. This 10-year period will be extended to 11 years if, during the first eight of those 10 years, the marketing authorization holder obtains an approval for one or more new therapeutic indications that bring significant clinical benefits compared with existing therapies. In addition, companies may be developing biosimilars in other countries that could compete with our products. If competitors are able to obtain marketing approval for biosimilars referencing our products, our products may become subject to competition from such biosimilars, with the attendant competitive pressure and consequences. Expiration or successful challenge of our applicable patent rights could also trigger competition from other products, assuming any relevant exclusivity period has expired.

In addition, although our product candidates have been granted orphan drug status by the FDA and EMA, there are limitations to the exclusivity. In the United States, the exclusivity period for orphan drugs is seven years, while pediatric exclusivity adds six months to any existing patents or exclusivity periods. In Europe, orphan drugs may be able to obtain 10 years of marketing exclusivity and up to an additional two years on the basis of qualifying pediatric studies. However, orphan exclusivity may be reduced to six years if the drug no longer satisfies the original designation criteria. Additionally, a marketing authorization holder may lose its orphan exclusivity if it consents to a second orphan drug application or cannot supply enough drug. Orphan drug exclusivity also can be lost when a second applicant demonstrates its drug is “clinically superior” to the original orphan drug.

Finally, as a result of the expiration or successful challenge of our patent rights, we could face more litigation with respect to the validity and/or scope of patents relating to our competitors’ products. The availability of our competitors’ products could limit the demand, and the price we are able to charge, for any products that we may develop and commercialize.

***The commercial success of any current or future product candidate will depend upon the degree of market acceptance by physicians, patients, third-party payors and others in the medical community.***

Ethical, social and legal concerns about gene therapy and genetic research could result in additional regulations restricting or prohibiting the products and processes we may use. Even with the requisite approvals, the commercial success of our product candidates will depend in

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part on the medical community, patients, and third-party payors accepting gene therapy products in general, and our product candidates in particular, as medically useful, cost-effective, and safe. Any product that we bring to the market may not gain market acceptance by physicians, patients, third-party payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of these product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the potential efficacy and potential advantages over alternative treatments;
- the prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling;
- the prevalence and severity of any side effects resulting from the chemotherapy and myeloablative treatments associated with the procedure by which our product candidates are administered;
- relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party insurance coverage or reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical and clinical studies, market acceptance of the product will not be known until after it is launched. Our efforts to educate the medical community and third-party payors on the benefits of the product candidates may require significant resources and may never be successful. Such efforts to educate the marketplace may require more resources than are required by the conventional technologies marketed by our competitors.

***If we obtain approval to commercialize our product candidates outside of the United States, a variety of risks associated with international operations could materially adversely affect our business.***

If any of our product candidates are approved for commercialization, we may enter into agreements with third parties to market them on a worldwide basis or in more limited geographical regions. We expect that we will be subject to additional risks related to entering into international business relationships, including:

- different regulatory requirements for approval of drugs and biologics in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;

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- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including earthquakes, typhoons, floods and fires.

***The insurance coverage and reimbursement status of newly-approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for new or current products could limit our ability to market those products and decrease our ability to generate revenue.***

The availability and extent of reimbursement by governmental and private payors is essential for most patients to be able to afford expensive treatments, such as stem cell transplants. Sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or reimbursed by government health administration authorities, private health coverage insurers and other third-party payors. If reimbursement is not available, or is available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about reimbursement for new medicines are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, as CMS decides whether and to what extent a new medicine will be covered and reimbursed under Medicare. Private payors tend to follow CMS to a substantial degree. It is difficult to predict what CMS will decide with respect to reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these new products. Reimbursement agencies in Europe may be more conservative than CMS. For example, a number of cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European countries. In addition, costs or difficulties associated with the reimbursement of Glybera could create an adverse environment for reimbursement of other gene therapies.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada, and other countries has and will continue to put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medicines, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our

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products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Moreover, increasing efforts by governmental and third-party payors, in the United States and abroad, to cap or reduce healthcare costs may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of any of our product candidates, due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products.

***Due to the novel nature of our technology and the potential for our product candidates to offer therapeutic benefit in a single administration, we face uncertainty related to pricing and reimbursement for these product candidates.***

Our target patient populations are relatively small, as a result of which the pricing and reimbursement of our product candidates, if approved, must be adequate to support commercial infrastructure. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our product candidates will be adversely affected. The manner and level at which reimbursement is provided for services related to our product candidates (e.g., for administration of our product to patients) is also important. Inadequate reimbursement for such services may lead to physician resistance and adversely affect our ability to market or sell our products.

***If the market opportunities for our product candidates are smaller than we believe they are, our revenues may be adversely affected and our business may suffer. Because the target patient populations of our product candidates are small, we must be able to successfully identify patients and achieve a significant market share to maintain profitability and growth.***

We focus our research and product development on treatments for severe genetic and orphan diseases. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates. These estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of these diseases. The number of patients in the United States, Europe and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with our products, or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business.

## **Risks related to our business operations**

***Negative public opinion and increased regulatory scrutiny of gene therapy and genetic research may damage public perception of our product candidates or adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.***

Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians specializing in the treatment of those diseases that our product

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candidates target prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments they are already familiar with and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. For example, in 2003, 20 subjects treated for X-linked severe combined immunodeficiency in two gene therapy studies using a murine gamma-retroviral vector showed correction of the disease, but the studies were terminated after five subjects developed leukemia (four of whom were subsequently cured). Although none of our current product candidates utilize these gamma-retroviruses, our product candidates use a viral delivery system. Adverse events in our clinical studies, even if not ultimately attributable to our product candidates (such as the many adverse events that typically arise from the transplant process) and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our potential product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

***Our future success depends on our ability to retain key employees and to attract, retain and motivate qualified personnel.***

We are highly dependent on principal members of our executive team and key employees listed under "Management" located elsewhere in this prospectus, the loss of whose services may adversely impact the achievement of our objectives. While we have entered into employment agreements with each of our executive officers, any of them could leave our employment at any time, as all of our employees are "at will" employees. Recruiting and retaining other qualified employees for our business, including scientific and technical personnel, will also be critical to our success. There is currently a shortage of skilled executives in our industry, which is likely to continue. As a result, competition for skilled personnel is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for individuals with similar skill sets. In addition, failure to succeed in preclinical or clinical studies may make it more challenging to recruit and retain qualified personnel. The inability to recruit or loss of the services of any executive or key employee may impede the progress of our research, development and commercialization objectives.

***We will need to expand our organization and we may experience difficulties in managing this growth, which could disrupt our operations.***

As of March 31, 2013, we had 50 full-time employees. As we mature and undertake the activities required under our collaboration with Celgene, we expect to expand our full-time employee base and to hire more consultants and contractors. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. For example, in the past there have been errors in the preparation of our financial statements and there can be no assurance that other errors will not occur in the future as we grow. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively



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manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

***Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.***

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and commercial partners. Misconduct by these parties could include intentional failures to comply with the regulations of the FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of information obtained in the course of clinical studies, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

***We face potential product liability, and, if successful claims are brought against us, we may incur substantial liability and costs. If the use of our product candidates harms patients, or is perceived to harm patients even when such harm is unrelated to our product candidates, our regulatory approvals could be revoked or otherwise negatively impacted and we could be subject to costly and damaging product liability claims.***

The use of our product candidates in clinical studies and the sale of any products for which we obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. There is a risk that our product candidates may induce adverse events. If we cannot successfully defend against product liability claims, we could incur substantial liability and costs. In addition, regardless of merit or eventual outcome, product liability claims may result in:

- impairment of our business reputation;
- withdrawal of clinical study participants;
- costs due to related litigation;
- distraction of management's attention from our primary business;

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- substantial monetary awards to patients or other claimants;
- the inability to commercialize our product candidates; and
- decreased demand for our product candidates, if approved for commercial sale.

We carry product liability insurance of \$5,000,000 per occurrence and \$5,000,000 aggregate limit. We believe our product liability insurance coverage is sufficient in light of our current clinical programs; however, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. If and when we obtain marketing approval for product candidates, we intend to expand our insurance coverage to include the sale of commercial products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. On occasion, large judgments have been awarded in class action lawsuits based on drugs or medical treatments that had unanticipated adverse effects. A successful product liability claim or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business.

Patients with the diseases targeted by our product candidates are often already in severe and advanced stages of disease and have both known and unknown significant pre-existing and potentially life-threatening health risks. During the course of treatment, patients may suffer adverse events, including death, for reasons that may be related to our product candidates. Such events could subject us to costly litigation, require us to pay substantial amounts of money to injured patients, delay, negatively impact or end our opportunity to receive or maintain regulatory approval to market our products, or require us to suspend or abandon our commercialization efforts. Even in a circumstance in which we do not believe that an adverse event is related to our products, the investigation into the circumstance may be time-consuming or inconclusive. These investigations may interrupt our sales efforts, delay our regulatory approval process in other countries, or impact and limit the type of regulatory approvals our product candidates receive or maintain. As a result of these factors, a product liability claim, even if successfully defended, could have a material adverse effect on our business, financial condition or results of operations.

***If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.***

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential liabilities. In addition, we may incur substantial costs in order to comply with current or future

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environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

***We may not be successful in our efforts to identify or discover additional product candidates.***

The success of our business depends primarily upon our ability to identify, develop and commercialize products based on our gene therapy platform. Although our Lenti-D and LentiGlobin product candidates are currently in clinical development, our research programs, including those subject to our collaboration with Celgene, may fail to identify other potential product candidates for clinical development for a number of reasons. Our research methodology may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, which would have a material adverse effect on our business and could potentially cause us to cease operations. Research programs to identify new product candidates require substantial technical, financial and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful.

***We may use our financial and human resources to pursue a particular research program or product candidate and fail to capitalize on programs or product candidates that may be more profitable or for which there is a greater likelihood of success.***

Because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for product candidates may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

***We will incur significant increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.***

As a public company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act, as well as rules subsequently implemented by the Securities and Exchange Commission, or SEC, and The Nasdaq Global Market have imposed various requirements on public companies. In July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as “say on pay” and proxy access. Recent legislation permits smaller “emerging growth companies” to implement many of these requirements over a longer period and up to five years from the pricing of this offering. We intend to take advantage of this new

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legislation but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain our current levels of such coverage. We estimate that we will annually incur approximately \$1.0 million to \$3.0 million in additional expenses to comply with the requirements imposed on us as a public company.

***Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.***

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. The recent global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the recent global financial crisis, could result in a variety of risks to our business, including, weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. This is particularly true in Europe, which is undergoing a continued severe economic crisis. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our services. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

***We or the third parties upon whom we depend may be adversely affected by earthquakes or other natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.***

Earthquakes or other natural disasters could severely disrupt our operations, and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place currently are limited and are unlikely to prove adequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which, particularly when taken together with our lack of earthquake insurance, could have a material adverse effect on our business.

**Risks related to our intellectual property**

***If we are unable to obtain or protect intellectual property rights related to our product candidates, we may not be able to compete effectively in our markets.***

We rely upon a combination of patents, trade secret protection and confidentiality agreements to protect the intellectual property related to our product candidates. The strength

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of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates in the United States or in other foreign countries. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue and even if such patents cover our product candidates, third parties may challenge their validity, enforceability or scope, which may result in such patents being narrowed or invalidated. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property, provide exclusivity for our product candidates or prevent others from designing around our claims. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

If the patent applications we hold or have in-licensed with respect to our programs or product candidates fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our product candidates, it could dissuade companies from collaborating with us to develop product candidates, and threaten our ability to commercialize, future products. Several patent applications covering our product candidates have been filed recently. We cannot offer any assurances about which, if any, patents will issue, the breadth of any such patent or whether any issued patents will be found invalid and unenforceable or will be threatened by third parties. Any successful opposition to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any product candidates that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced. Since patent applications in the United States and most other countries are confidential for a period of time after filing, and some remain so until issued, we cannot be certain that we were the first to file any patent application related to a product candidate. Furthermore, if third parties have filed such patent applications, an interference proceeding in the United States can be initiated by a third party to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. In addition, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Various extensions may be available however the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product, we may be open to competition from generic medications.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

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Although we expect all of our employees and consultants to assign their inventions to us, and all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed or that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have a material adverse effect on our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all.

Further, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If we are unable to prevent material disclosure of the non-patented intellectual property related to our technologies to third parties, and there is no guarantee that we will have any such enforceable trade secret protection, we may not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, results of operations and financial condition.

***Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.***

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions and *inter partes* reexamination proceedings before the U.S. Patent and Trademark Office, or U.S. PTO, and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire.

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Similarly, if any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

***We may not be successful in obtaining or maintaining necessary rights to gene therapy product components and processes for our development pipeline through acquisitions and in-licenses.***

Presently we have rights to the intellectual property, through licenses from third parties and under patents that we own, to develop our gene therapy product candidates. Because our programs may involve additional product candidates that may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license or use these proprietary rights. In addition, our product candidates may require specific formulations to work effectively and efficiently and these rights may be held by others. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

For example, we sometimes collaborate with U.S. and foreign academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such right of first negotiation for intellectual property, we may be unable to negotiate a license within the specified time frame or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment. If we are unable to successfully obtain rights to required third-party intellectual property rights, our business, financial condition and prospects for growth could suffer.

***If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.***

We are a party to a number of intellectual property license agreements that are important to our business and expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty and other obligations on us. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license. See “Business—License agreements” for a description of our license agreements with Inserm-Transfert, Institut Pasteur, Stanford University, the Massachusetts Institute of Technology and Research Development Foundation, which includes a description of the termination provisions of these agreements.

We may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates, and we have done so from time to time. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we may be required to expend significant time and resources to develop or license replacement technology. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could harm our business significantly. We cannot provide any assurances that third-party patents do not exist which might be enforced against our current product candidates or future products, resulting in either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties.

In many cases, patent prosecution of our licensed technology is controlled solely by the licensor. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. In certain cases, we control the prosecution of patents resulting from licensed technology. In the event we breach any of our obligations related to such prosecution, we may incur significant liability to our licensing partners. Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues and is complicated by the rapid pace of scientific discovery in our industry. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.



If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

***We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming and unsuccessful.***

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid, is unenforceable and/or is not infringed, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing.

Interference proceedings provoked by third parties or brought by us may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our intellectual property rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

***Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.***

On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law, including provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. The U.S. PTO is currently developing regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, were enacted March 16, 2013. However, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

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***We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.***

We employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and independent contractors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of any of our employee's former employer or other third parties. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

***We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.***

We may also be subject to claims that former employees, collaborators or other third parties have an ownership interest in our patents or other intellectual property. We have had in the past, and we may also have to in the future, ownership disputes arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

***Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.***

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or applications will be due to be paid to the U.S. PTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and/or applications. We have systems in place to remind us to pay these fees, and we employ an outside firm and rely on our outside counsel to pay these fees due to non-U.S. patent agencies. The U.S. PTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply, and in many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, our competitors might be able to enter the market and this circumstance would have a material adverse effect on our business.

***Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court.***

If we or one of our licensing partners initiated legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the U.S. PTO, or made a misleading statement, during prosecution. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection would have a material adverse impact on our business.

***Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.***

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology industry involve both technological and legal complexity, and is therefore obtaining and enforcing biotechnology patents is costly, time-consuming and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the U.S. PTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

***We have not yet registered trademarks for a commercial trade name for Lenti-D and failure to secure such registrations could adversely affect our business.***

We have not yet registered trademarks for a commercial trade name for Lenti-D. During trademark registration proceedings, we may receive rejections. Although we would be given an opportunity to respond to those rejections, we may be unable to overcome such rejections. In addition, in the U.S. PTO and in comparable agencies in many foreign jurisdictions, third parties are given an opportunity to oppose pending trademark applications and to seek to cancel registered trademarks. Opposition or cancellation proceedings may be filed against our trademarks, and our trademarks may not survive such proceedings. Moreover, any name we

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propose to use with our product candidates in the United States must be approved by the FDA, regardless of whether we have registered it, or applied to register it, as a trademark. The FDA typically conducts a review of proposed product names, including an evaluation of potential for confusion with other product names. If the FDA objects to any of our proposed proprietary product names, we may be required to expend significant additional resources in an effort to identify a suitable substitute name that would qualify under applicable trademark laws, not infringe the existing rights of third parties and be acceptable to the FDA.

***We may not be able to protect our intellectual property rights throughout the world.***

Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

**Risks related to this offering and ownership of our common stock**

***The market price of our common stock may be highly volatile, and you may not be able to resell your shares at or above the initial public offering price.***

Prior to this offering, there has not been a public market for our common stock. An active trading market for our common stock may not develop following this offering. You may not be able to sell your shares quickly or at the market price if trading in our common stock is not active. The initial public offering price for the shares will be determined by negotiations between us and the representative of the underwriters and may not be indicative of prices that will prevail in the trading market.

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The market price of our common stock is likely to be volatile. Our stock price could be subject to wide fluctuations in response to a variety of factors, including the following:

- adverse results or delays in preclinical or clinical studies;
- reports of adverse events in other gene therapy products or clinical studies of such products;
- inability to obtain additional funding;
- any delay in filing an IND or BLA for any of our product candidates and any adverse development or perceived adverse development with respect to the FDA's review of that IND or BLA;
- failure to develop successfully and commercialize our product candidates;
- failure to maintain our existing strategic collaborations or enter into new collaborations;
- failure by us or our licensors and strategic collaboration partners to prosecute, maintain or enforce our intellectual property rights;
- changes in laws or regulations applicable to future products;
- inability to obtain adequate product supply for our product candidates or the inability to do so at acceptable prices;
- adverse regulatory decisions;
- introduction of new products, services or technologies by our competitors;
- failure to meet or exceed financial projections we may provide to the public;
- failure to meet or exceed the financial projections of the investment community;
- the perception of the pharmaceutical industry by the public, legislatures, regulators and the investment community;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us, our strategic collaboration partner or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- additions or departures of key scientific or management personnel;
- significant lawsuits, including patent or stockholder litigation;
- changes in the market valuations of similar companies;
- sales of our common stock by us or our stockholders in the future; and
- trading volume of our common stock.

In addition, companies trading in the stock market in general, and The Nasdaq Global Market in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance.

***Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.***

Our executive officers, directors, five percent stockholders and their affiliates beneficially own approximately % of our voting stock and, upon closing of this offering, that same group will beneficially own approximately % of our outstanding voting stock. Therefore, even after this offering, these stockholders will have the ability to influence us through their ownership positions. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders, acting together, may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may believe are in your best interest as one of our stockholders.

***We are an “emerging growth company,” and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.***

We are an “emerging growth company,” as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company for up to five years, although circumstances could cause us to lose that status earlier, including if the market value of our common stock held by non-affiliates exceeds \$700.0 million as of any June 30 before that time or if we have total annual gross revenue of \$1.0 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31 or, if we issue more than \$1.0 billion in non-convertible debt during any three-year period before that time, we would cease to be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may still qualify as a “smaller reporting company” which would allow us to take advantage of many of the same exemptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

***If you purchase our common stock in this offering, you will incur immediate and substantial dilution in the book value of your shares.***

Investors purchasing shares of common stock in this offering will pay a price per share that substantially exceeds the pro forma book value per share of our tangible assets after subtracting our liabilities. As a result, investors purchasing shares of common stock in this offering will incur immediate dilution of \$      per share, based on an assumed initial public offering price of \$      per share, the midpoint of the price range set forth on the cover of this prospectus, and our pro forma net tangible book value as of March 31, 2013. Further, based on these assumptions, investors purchasing shares of common stock in this offering will contribute approximately      % of the total amount invested by stockholders since our inception, but will own only approximately      % of the shares of common stock outstanding. For information on how the foregoing amounts were calculated, see "Dilution."

This dilution is due to the substantially lower price paid by our investors who purchased shares prior to this offering as compared to the price offered to the public in this offering, and the exercise of stock options granted to our employees. In addition, as of March 31, 2013, options to purchase 69,284,748 shares of our common stock at a weighted average exercise price of \$0.19 per share were outstanding. The exercise of any of these options would result in additional dilution. As a result of the dilution to investors purchasing shares in this offering, investors may receive significantly less than the purchase price paid in this offering, if anything, in the event of our liquidation.

***Sales of a substantial number of shares of our common stock in the public market could cause our stock price to fall.***

If our existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after the lock-up and other legal restrictions on resale discussed in this prospectus lapse, the market price of our common stock could decline. Based upon the number of shares of common stock, on an as-converted basis, outstanding as of      , 2013, upon the closing of this offering, we will have outstanding a total of      shares of common stock, assuming no exercise of the underwriters' option to purchase additional shares. Of these shares, approximately      shares of our common stock, plus any shares sold upon exercise of the underwriters' option to purchase additional shares, will be freely tradable, without restriction, in the public market immediately following this offering, assuming that current stockholders do not purchase shares in this offering. J.P. Morgan Securities LLC and Merrill Lynch, Pierce, Fenner & Smith Incorporated, however, may, in their sole discretion, permit our officers, directors and other stockholders who are subject to these lock-up agreements to sell shares prior to the expiration of the lock-up agreements.

The lock-up agreements pertaining to this offering will expire 180 days from the date of this prospectus (subject to extension upon the occurrence of specified events). After the lock-up agreements expire, as of      , 2013, up to an additional      shares of common stock will be eligible for sale in the public market,      of which shares are held by directors, executive officers and other affiliates and will be subject to Rule 144 under the Securities Act of 1933, as amended, or the Securities Act.

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In addition, as of \_\_\_\_\_, 2013, \_\_\_\_\_ shares of common stock that are either subject to outstanding options, reserved for future issuance under our equity incentive plans or subject to outstanding warrants will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements and Rule 144 and Rule 701 under the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline.

After this offering, the holders of approximately \_\_\_\_\_ million shares of our common stock, or approximately \_\_\_\_\_ % of our total outstanding common stock as of \_\_\_\_\_, 2013, will be entitled to rights with respect to the registration of their shares under the Securities Act, subject to the lock-up agreements described above. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares purchased by affiliates. Any sales of securities by these stockholders could have a material adverse effect on the market price of our common stock.

***Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.***

Additional capital will be needed in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

Pursuant to our 2013 Stock Option and Incentive Plan, or the 2013 Plan, our management is authorized to grant stock options and other equity-based awards to our employees, directors and consultants. The number of shares available for future grant under the 2013 Plan will automatically increase each year by up to \_\_\_\_\_ % of all shares of our capital stock outstanding as of December 31 of the prior calendar year, subject to the ability of our board of directors to take action to reduce the size of the increase in any given year. Currently, we plan to register the increased number of shares available for issuance under the 2013 Plan each year. If our board of directors elects to increase the number of shares available for future grant by the maximum amount each year, our stockholders may experience additional dilution, which could cause our stock price to fall.

***We could be subject to securities class action litigation.***

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because pharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

***We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.***

Our management will have broad discretion in the application of the net proceeds from this offering, including for any of the purposes described in the section entitled "Use of proceeds,"



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and you will not have the opportunity as part of your investment decision to assess whether the net proceeds are being used appropriately. Because of the number and variability of factors that will determine our use of the net proceeds from this offering, their ultimate use may vary substantially from their currently intended use. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds from this offering in short-term, investment-grade, interest-bearing securities. These investments may not yield a favorable return to our stockholders.

***Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.***

Under Section 382 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an “ownership change,” generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation’s ability to use its pre-change net operating loss carryforwards, or NOLs, and other pre-change tax attributes (such as research tax credits) to offset its post-change income may be limited. We believe we may have triggered an “ownership change” limitation. We may also experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

***We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.***

We have never declared or paid any cash dividends on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the appreciation of their stock.

***Provisions in our amended and restated certificate of incorporation and by-laws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders or remove our current management.***

Our amended and restated certificate of incorporation, amended and restated by-laws and Delaware law contain provisions that may have the effect of delaying or preventing a change in control of us or changes in our management. Our amended and restated certificate of incorporation and by-laws, which will become effective upon the closing of this offering, include provisions that:

- authorize “blank check” preferred stock, which could be issued by our board of directors without stockholder approval and may contain voting, liquidation, dividend and other rights superior to our common stock;
- create a classified board of directors whose members serve staggered three-year terms;
- specify that special meetings of our stockholders can be called only by our board of directors, the chairperson of our board of directors, our chief executive officer or our president;
- prohibit stockholder action by written consent;

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- establish an advance notice procedure for stockholder approvals to be brought before an annual meeting of our stockholders, including proposed nominations of persons for election to our board of directors;
- provide that our directors may be removed only for cause;
- provide that vacancies on our board of directors may be filled only by a majority of directors then in office, even though less than a quorum;
- specify that no stockholder is permitted to cumulate votes at any election of directors;
- expressly authorize our board of directors to modify, alter or repeal our amended and restated by-laws; and
- require supermajority votes of the holders of our common stock to amend specified provisions of our amended and restated certificate of incorporation and amended and restated by-laws.

These provisions, alone or together, could delay or prevent hostile takeovers and changes in control or changes in our management.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which limits the ability of stockholders owning in excess of 15% of our outstanding voting stock to merge or combine with us.

Any provision of our amended and restated certificate of incorporation or amended and restated by-laws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

***Provisions in our collaboration agreement with Celgene Corporation may prevent or delay a change in control.***

Our collaboration agreement with Celgene Corporation provides that, effective upon completion of this offering, during the initial three-year term of the collaboration and, if extended, during the first extension term of the collaboration which is two years, in the event that we engage in a change in control transaction, including for such purposes a merger or consolidation of bluebird bio or the sale of all or substantially all of our assets, or if another person or entity or group of persons or entities acquires at least 50% of our voting capital stock, then Celgene has the right, but not the obligation, to terminate the collaboration agreement and obtain perpetual, non-terminable, worldwide, exclusive, fully paid-up licenses to all, but not less than all, of the product candidates previously identified under the collaboration agreement. We refer to this right to acquire such licenses as the call option.

Under the call option, the product candidates to which Celgene would have the right to acquire fully paid-up licenses include any product candidate previously licensed out of the collaboration during the term of the collaboration, any product candidate for which we have exercised our right to co-develop and co-promote the product candidate within the United States, any product candidate for which Celgene previously declined its option to obtain a license and any product candidate for which at least *in vivo* efficacy studies have been initiated or authorized by the joint steering committee for the collaboration. The purchase price for such

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fully paid-up licenses would be determined pursuant to a binding arbitration process and would be paid on or about the consummation of the change of control transaction with our acquiror. The call option will lapse at the end of the three-year term of the collaboration, unless extended, in which case it will lapse at the end of the first extension term, which is two years, even if the collaboration is extended further.

In addition, during the initial three-year term of the collaboration, but not during any extension of the collaboration agreement, in the event that we engage in a change in control transaction described above and Celgene exercises the call option described above, then, in addition to the right to acquire the fully paid-up licenses described above, Celgene would also have the right to obtain a perpetual, non-terminable, worldwide, exclusive license to our intellectual property to develop one or more CAR T cell products targeting one or more oncology associated target antigens identified by Celgene following the third anniversary of the collaboration agreement. There is no limit to the number of oncology associated target antigens Celgene may select under this license. Upon commercialization of any such product candidate so licensed by Celgene, Celgene would be obligated to pay us a specified milestone payment upon regulatory approval and a percentage of net sales as a royalty. We refer to this license agreement to develop one or more CAR T cell products targeting one or more oncology associated target antigens as the target antigen license. The right to acquire a target antigen license will lapse after the initial three-year term of the collaboration, even if the collaboration is extended.

The call option and the right to acquire a target antigen license may have the effect of delaying or preventing a change in control transaction involving us, or may reduce the number of companies interested in acquiring us. If Celgene were to exercise the call option, it would gain exclusive development and marketing rights to the product candidates developed under the collaboration agreement, including any product for which we previously exercised our co-development and co-promotion rights. Were this to happen, our successor would not receive a royalty on net sales of any of the products out-licensed in connection with the call option, nor would it realize any value it may otherwise ascribe to our right to co-develop and co-promote within the United States any products developed during the collaboration. Moreover, if such event were to occur during the first three years of the collaboration, Celgene would also effectively have the exclusive right to develop and market an unlimited number of additional CAR T cell products using our gene therapy platform, whether or not these products were first identified or developed during the course of the collaboration, which product candidates would target a list of oncology associated target antigens that would not be known at the time we close our change in control transaction. This license could potentially give Celgene rights to our gene therapy platform for CAR T cell product candidates in the event we are acquired prior to the third anniversary of the collaboration.

These provisions could have the effect of delaying or preventing a change in control transaction involving bluebird bio, or could reduce the number of companies interested in acquiring us, in particular during the first three years of the collaboration. This risk may become particularly acute in the event either of our lead product candidates, Lenti-D or LentiGlobin, suffer material setbacks or delays in their clinical advancement, as a result of which the long-term strategic value potential acquirors may ascribe to us could increasingly be attributable to the potential long-term value of any CAR T cell products we develop under the collaboration.

## Cautionary note regarding forward-looking statements

This prospectus contains forward-looking statements that involve risks and uncertainties. All statements other than statements of historical facts contained in this prospectus are forward-looking statements. In some cases, you can identify forward-looking statements by words such as “anticipate,” “believe,” “contemplate,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “seek,” “should,” “target,” “will,” “would,” or the negative of these words or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- the initiation, timing, progress and results of our preclinical and clinical studies, and our research and development programs;
- our ability to advance product candidates into, and successfully complete, clinical studies;
- our ability to advance our viral vector manufacturing and transduction capabilities;
- the timing or likelihood of regulatory filings and approvals;
- the commercialization of our product candidates, if approved;
- the pricing and reimbursement of our product candidates, if approved;
- the implementation of our business model, strategic plans for our business, product candidates and technology;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and technology;
- estimates of our expenses, future revenues, capital requirements and our needs for additional financing;
- the potential benefits of strategic collaboration agreements and our ability to enter into strategic arrangements;
- our ability to maintain and establish collaborations or obtain additional grant funding;
- our financial performance;
- developments relating to our competitors and our industry; and
- other risks and uncertainties, including those listed under the caption “Risk factors.”

Any forward-looking statements in this prospectus reflect our current views with respect to future events or to our future financial performance and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under “Risk factors” and elsewhere in this prospectus. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

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This prospectus also contains estimates, projections and other information concerning our industry, our business, and the markets for certain diseases, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained this industry, business, market and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources.

## Use of proceeds

We estimate that the net proceeds from the sale of \_\_\_\_\_ shares of common stock in this offering will be approximately \$ \_\_\_\_\_ million at an assumed initial public offering price of \$ \_\_\_\_\_ per share, the midpoint of the price range set forth on the cover of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise their option to purchase additional shares in full, we estimate that the net proceeds will be approximately \$ \_\_\_\_\_ million after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. Each \$1.00 increase or decrease in the assumed initial public offering price of \$ \_\_\_\_\_ per share would increase or decrease our net proceeds by \$ \_\_\_\_\_ million, assuming the number of shares offered by us, as set forth on the cover of this prospectus, remains the same and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

We are undertaking this offering in order to access the public capital markets and to increase our liquidity. We intend to use the net proceeds of this offering as follows:

- Approximately \$ \_\_\_\_\_ million to fund direct research and development expenses for our ALD-102 Study, a Phase II/III clinical study of Lenti-D to evaluate its safety and efficacy in subjects with childhood cerebral adrenoleukodystrophy;
- Approximately \$ \_\_\_\_\_ million to fund direct research and development expenses for our HGB-204 Study, a Phase I/II clinical study in the United States of LentiGlobin to evaluate its safety and efficacy in subjects with  $\beta$ -thalassemia major;
- Approximately \$ \_\_\_\_\_ million to fund direct research and development expenses for our HGB-205 Study, a Phase I/II clinical study in Europe of LentiGlobin to evaluate its safety and efficacy in subjects with  $\beta$ -thalassemia major and sickle cell disease; and
- The remainder for general and administrative expenses (including personnel-related costs), potential future development programs, early-stage research and development, capital expenditures and working capital and other general corporate purposes.

Our expected use of net proceeds from this offering represents our current intentions based upon our present plans and business condition. As of the date of this prospectus, we cannot predict with certainty all of the particular uses for the net proceeds to be received upon the completion of this offering or the amounts that we will actually spend on the uses set forth above. We may also use a portion of the net proceeds to in-license, acquire or invest in complementary gene therapy businesses, technologies, products or assets. Due to the many variables inherent to the development of gene therapy products at this time, such as the timing of patient enrollment and evolving regulatory requirements, we cannot currently predict the stage of development we expect the net proceeds of this offering to achieve for our clinical studies and product candidates.

The amount and timing of our actual expenditures will depend upon numerous factors, including the results of our research and development efforts, the timing and success of preclinical studies, our ongoing clinical studies or clinical studies we may commence in the future and the timing of regulatory submissions. As a result, our management will have broad discretion over the use of the net proceeds from this offering.

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Pending the use of the proceeds from this offering, we intend to invest the net proceeds in short-term, interest-bearing, investment-grade securities, certificates of deposit or government securities.

## **Dividend policy**

We have never declared or paid cash dividends on our common stock. We currently intend to retain all available funds and any future earnings, if any, to fund the development and expansion of our business and we do not anticipate paying any cash dividends in the foreseeable future. Any future determination to pay dividends will be made at the discretion of our board of directors.



## Capitalization

The following table sets forth our cash, cash equivalents and capitalization as of March 31, 2013:

- on an actual basis;
- on a pro forma basis to reflect conversion of all outstanding shares of our preferred stock into an aggregate of 310,841,204 shares of common stock and the reclassification of our outstanding warrants to purchase shares of preferred stock to common stock, in each case prior to the completion of this offering; and
- on a pro forma as adjusted basis to additionally reflect the issuance and sale by us of shares of our common stock in this offering, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us, at an assumed initial public offering price of \$ \_\_\_\_\_ per share, the midpoint of the price range set forth on the cover of this prospectus.

You should read this information together with our audited financial statements and related notes appearing elsewhere in this prospectus and the information set forth under the heading "Selected consolidated financial data" and "Management's discussion and analysis of financial condition and results of operations."

(in thousands, except per share data)	As of March 31, 2013	
	Actual	Pro forma as adjusted (unaudited)
Cash and cash equivalents	\$ 131,836	\$ 131,836
Preferred stock warrant liability	256	—
Series A-2 convertible preferred stock, \$0.01 par value: 22,304 shares authorized; 22,304 shares issued and outstanding at March 31, 2013, and no shares issued and outstanding pro forma and pro forma as adjusted	7,137	—
Series B convertible preferred stock, \$0.01 par value: 115,779 shares authorized; 115,204 shares issued and outstanding at March 31, 2013, and no shares issued and outstanding pro forma and pro forma as adjusted	40,321	—
Series C convertible preferred stock, \$0.01 par value: 39,943 shares authorized; 39,943 shares issued and outstanding at March 31, 2013, and no shares issued and outstanding pro forma and pro forma as adjusted	12,382	—
Series D convertible preferred stock, \$0.01 par value: 120,409 shares authorized; 120,409 shares issued and outstanding at March 31, 2013, and no shares issued and outstanding pro forma and pro forma as adjusted	60,000	—
Stockholders' deficit:		
Series A-1 convertible preferred stock, \$0.01 par value: 18,817 shares authorized; 12,981 shares issued and outstanding at March 31, 2013, and no shares issued and outstanding pro forma and pro forma as adjusted	2,337	—
Common stock, \$0.01 par value: 408,000 shares authorized, actual and pro forma; 6,599 shares issued and outstanding at March 31, 2013, and 317,440 shares issued and outstanding pro forma(1); _____ shares authorized and _____ shares issued and outstanding, pro forma as adjusted	66	3,174
Additional paid-in capital	15,900	135,225
Accumulated deficit	(79,898)	(79,898)
Total stockholders' (deficit) equity	(61,595)	58,501
Total capitalization	\$ 58,501	\$ 58,501

(1) Excludes 2,506 shares of unvested restricted common stock.

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The actual, pro forma and pro forma as adjusted outstanding shares information in the table above excludes the following:

- 69,284,748 shares of common stock issuable upon the exercise of outstanding stock options having a weighted-average exercise price of \$0.19 per share;
- 8,352,387 shares of common stock issuable upon the exercise of outstanding warrants having a weighted-average exercise price of \$0.49 per share;
- 10,354,017 shares of common stock reserved for issuance pursuant to future equity awards under our 2010 Stock Option and Grant Plan; and
- \_\_\_\_\_ shares of common stock reserved for issuance pursuant to future equity awards under our 2013 Stock Option and Incentive Plan.

## Dilution

If you invest in our common stock in this offering, your interest will be diluted to the extent of the difference between the initial public offering price per share of our common stock in this offering and the pro forma as adjusted net tangible book value per share of our common stock after this offering.

As of March 31, 2013, we had pro forma net tangible book value of \$58.5 million, or \$0.18 per share of common stock, taking into account the expected conversion of our outstanding preferred stock into common stock and reclassification of our outstanding warrants to purchase our Series B preferred stock into common stock, prior to the completion of this offering. Without giving effect to the conversion of our outstanding preferred stock into common stock, we had a historical net tangible book value of \$(61.6) million, or \$(9.33) per share of common stock, as of March 31, 2013. Historical net tangible book value per share is equal to our total tangible assets, less total liabilities and preferred stock, divided by the number of outstanding shares of our common stock (excluding 2,506,114 shares of unvested restricted stock subject to repurchase by us). After giving effect to (1) the conversion of all of our preferred stock into 310,841,204 shares of common stock prior to the completion of this offering and (2) the sale of \_\_\_\_\_ shares of common stock in this offering, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us, at an assumed initial public offering price of \$ \_\_\_\_\_ per share, the midpoint of the price range set forth on the cover of this prospectus, our pro forma as adjusted net tangible book value as of March 31, 2013 would have been approximately \$ \_\_\_\_\_ million, or approximately \$ \_\_\_\_\_ per share of common stock. This represents an immediate increase in pro forma as adjusted net tangible book value of \$ \_\_\_\_\_ per share to our existing stockholders and an immediate dilution of \$ \_\_\_\_\_ per share to investors participating in this offering. The following table illustrates this per share dilution:

Assumed initial public offering price per share	\$
Historical net tangible book value per share as of March 31, 2013	\$(9.33)
Increase attributable to the conversion of outstanding preferred stock and reclassification of preferred stock warrants	_____
Pro forma net tangible book value per share as of March 31, 2013	_____
Increase in net tangible book value per share attributable to new investors	_____
Pro forma net tangible book value per share after this offering	_____
Dilution per share to new investors	\$

Each \$1.00 increase (decrease) in the assumed initial public offering price of \$ \_\_\_\_\_ per share would increase (decrease) our pro forma as adjusted net tangible book value by approximately \$ \_\_\_\_\_ million, the pro forma as adjusted net tangible book value per share by approximately \$ \_\_\_\_\_ per share and the dilution to investors purchasing shares in this offering by approximately \$ \_\_\_\_\_ per share, assuming the number of shares offered by us, as set forth on the cover of this prospectus, remains the same and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

The following table summarizes, on a pro forma as adjusted basis as of March 31, 2013, the differences between the number of shares of common stock purchased from us, the total consideration and the average price per share paid by existing stockholders (giving effect to the conversion of all of our preferred stock into 310,841,204 shares of common stock prior to the

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completion of this offering) and by investors participating in this offering, after deducting estimated underwriting discounts and commissions and estimated offering expenses, at an assumed initial public offering price of \$        per share, the midpoint of the price range set forth on the cover of this prospectus.

	Shares purchased		Total consideration		Average price per share
	Number	Percent	Amount	Percent	
Existing stockholders	319,946,737	%	\$	%	\$
New investors		%		%	\$
Total		100%	\$	%	

The number of shares of common stock to be outstanding after this offering is based on the number of shares outstanding as of March 31, 2013 and excludes the following:

- 69,284,748 shares of common stock issuable upon the exercise of outstanding stock options having a weighted-average exercise price of \$0.19 per share;
- 8,352,387 shares of common stock issuable upon the exercise of outstanding warrants having a weighted-average exercise price of \$0.49 per share;
- 10,345,017 shares of common stock reserved for issuance pursuant to future equity awards under our 2010 Stock Option and Grant Plan; and
- shares of common stock reserved for issuance pursuant to future equity awards under our 2013 Stock Option and Incentive Plan.

If the underwriters exercise their option to purchase additional shares in full, pro forma as adjusted net tangible book value as of       , 2013 will increase to \$        million, or \$        per share, representing an increase to existing stockholders of \$        per share, and there will be an immediate dilution of an additional \$        per share to new investors.

Furthermore, we may choose to raise additional capital through the sale of equity or convertible debt securities due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. New investors will experience further dilution if any of our outstanding options or warrants are exercised, new options are issued and exercised under our equity incentive plans or we issue additional shares of common stock, other equity securities or convertible debt securities in the future.

## Selected consolidated financial data

The selected consolidated statements of operations data and the consolidated balance sheet data are derived from our audited consolidated financial statements appearing elsewhere in this prospectus. The selected consolidated financial data as of March 31, 2013 and for the three months ended March 31, 2013 and 2012 have been derived from our unaudited consolidated financial statements included elsewhere in this prospectus. In our opinion, these unaudited financial statements have been prepared on a basis consistent with our audited consolidated financial statements and contain all adjustments, consisting only of normal and recurring adjustments, necessary for a fair presentation of such consolidated financial data. You should read this data together with our audited consolidated financial statements and related notes appearing elsewhere in this prospectus and the information under the caption "Management's discussion and analysis of financial condition and results of operations." Our historical results are not necessarily indicative of our future results, and our operating results for the three-month period ended March 31, 2013 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2013 or any other interim periods or any future year or period.

(in thousands, except per share data)	Year ended December 31,		Three months ended	
	2011	2012	2012	March 31, 2013
			(unaudited)	
<b>Consolidated statements of operations data:</b>				
Revenue:				
Collaboration revenue	\$ —	\$ —	\$ —	\$ 1,042
Research and license fees	640	340	85	85
Grant revenue	242	—	—	—
	<u>882</u>	<u>340</u>	<u>85</u>	<u>1,127</u>
Expenses:				
Research and development	11,409	17,210	3,858	5,284
General and administrative	4,615	6,846	1,363	2,324
Total expenses	<u>16,024</u>	<u>24,056</u>	<u>5,221</u>	<u>7,608</u>
Loss from operations	(15,142)	(23,716)	(5,136)	(6,481)
Other income (expense), net	(456)	46	68	(63)
Net loss	<u>\$ (15,598)</u>	<u>\$ (23,670)</u>	<u>\$ (5,068)</u>	<u>\$ (6,544)</u>
Net loss per share applicable to common stockholders—basic and diluted(1)	<u>\$ (9.01)</u>	<u>\$ (0.73)</u>	<u>\$ (1.50)</u>	<u>\$ (1.05)</u>
Weighted-average number of common shares used in net loss per share applicable to common stockholders—basic and diluted	<u>2,285</u>	<u>4,972</u>	<u>4,236</u>	<u>6,226</u>
Pro forma net loss per share applicable to common stockholders—basic and diluted (unaudited)(1)		<u>\$ (0.10)</u>		<u>\$ (0.02)</u>
Pro forma weighted-average number of common shares used in net loss per share applicable to common stockholders—basic and diluted (unaudited)		<u>248,700</u>		<u>317,067</u>

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(in thousands)	As of March 31, 2013		
	Actual	Pro Forma(2)	Pro Forma Adjusted (3)(4)
<b>Consolidated balance sheet data:</b>			
Cash and cash equivalents	\$131,836	\$ 131,836	
Working capital	105,390	105,390	
Total assets	137,459	137,459	
Preferred stock	122,177	—	
Common stock and additional paid-in capital	15,966	138,399	
Total stockholders' (deficit) equity	(61,595)	58,501	

(1) See Notes 2 and 15 within the notes to our consolidated financial statements appearing elsewhere in this prospectus for a description of the method used to calculate basic and diluted net loss per common share and pro forma basic and diluted net loss per common share.

(2) Pro forma to reflect the conversion of all outstanding shares of our preferred stock into shares of common stock, and the reclassification of our outstanding warrants to purchase our Series B preferred stock to our common stock, upon the closing of this offering.

(3) Pro forma as adjusted to further reflect the sale of shares of our common stock offered in this offering, assuming an initial public offering price of \$ per share, the midpoint of the price range set forth on the cover of this prospectus, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

(4) A \$1.00 increase (decrease) in the assumed initial public offering price of \$ per share, the midpoint of the price range set forth on the cover of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents and total stockholders' (deficit) equity by approximately \$ million, assuming that the number of shares offered by us, as set forth on the cover of this prospectus, remains the same and after deducting underwriting discounts and commissions and estimated offering expenses payable by us. A share increase in the number of shares offered by us together with a concomitant \$1.00 increase in the assumed initial public offering price of \$ per share, the midpoint of the price range set forth on the cover of this prospectus, would increase each of cash and cash equivalents and total stockholders' (deficit) equity by approximately \$ million after deducting underwriting discounts and commissions and any estimated offering expenses payable by us. Conversely, a share decrease in the number of shares offered by us together with a concomitant \$1.00 decrease in the assumed initial public offering price of \$ per share, the midpoint of the price range set forth on the cover of this prospectus, would decrease each of cash and cash equivalents and total stockholders' (deficit) equity by approximately \$ million after deducting underwriting discounts and commissions and any estimated offering expenses payable by us.

## Management's discussion and analysis of financial condition and results of operations

*You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and related notes appearing in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk factors" section of this prospectus, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.*

### Overview

We are a clinical-stage biotechnology company focused on transforming the lives of patients with severe genetic and orphan diseases using gene therapy. We believe that gene therapy has the potential to change the way these patients are treated by correcting the underlying genetic defect that is the *cause* of their disease, rather than offering solutions that only address their *symptoms*. We and our scientific collaborators have generated what we believe is human proof-of-concept data for our gene therapy platform in two underserved diseases, each of which has been granted orphan drug status by U.S. and European regulatory authorities. We expect to initiate in late 2013 a Phase II/III clinical study of our most advanced product candidate, Lenti-D, to evaluate its safety and efficacy in subjects with childhood cerebral adrenoleukodystrophy, or CCALD, a rare, hereditary neurological disorder affecting young boys that is often fatal. We also expect to initiate in mid-2013 Phase I/II clinical studies in the United States and Europe of our next most advanced product candidate, LentiGlobin, to evaluate its safety and efficacy in subjects with  $\beta$ -thalassemia major and, in the European clinical study, sickle cell disease, or SCD, which are rare, hereditary blood disorders that often lead to severe anemia and shortened lifespans. In addition, in March 2013, we announced a global strategic collaboration with Celgene Corporation to discover, develop and commercialize novel, disease-altering gene therapies in oncology.

Since our inception in 1992, we have devoted substantially all of our resources to our development efforts relating to our product candidates, including activities to manufacture product in compliance with good manufacturing practices, or GMP, preparing to conduct clinical studies of our product candidates, providing general and administrative support for these operations and protecting our intellectual property. We do not have any products approved for sale and have not generated any revenue from product sales. We have funded our operations primarily through the private placement of preferred stock, common stock, convertible notes and warrants to purchase common stock. In addition, in October 2012, we were awarded a \$9.3 million grant from the California Institute for Regenerative Medicine, or CIRM, to fund our U.S.  $\beta$ -thalassemia program. This grant will be issued in quarterly installments and is expected to be utilized over a four-year period starting in the second quarter of 2013.

In March 2013, we entered into a strategic collaboration with Celgene Corporation, or Celgene, to discover, develop and commercialize novel, disease-altering gene therapies in oncology. This collaboration has an initial term of three years, and Celgene has made a \$75 million up-front, non-refundable cash payment to us as consideration for entering into the

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collaboration. During the three months ended March 31, 2013, we recognized \$1.0 million of revenue associated with our collaboration with Celgene related to the research and development services performed. As of March 31, 2013, there is \$74.0 million of deferred revenue related to our collaboration with Celgene that is classified as current or long-term in the accompanying balance sheet based on the contractual term of the arrangement.

We have never been profitable and have incurred net losses in each year since inception. Our net losses were \$15.6 million and \$23.7 million for the years ended December 31, 2011 and 2012, and \$5.1 million and \$6.5 million for the three months ended March 31, 2012 and 2013, respectively. Substantially all our net losses resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. We expect our expenses will increase substantially in connection with our ongoing activities, as we:

- conduct clinical studies for our Lenti-D and LentiGlobin product candidates;
- continue our research and development efforts;
- increase research and development related activities for the discovery and development of oncology product candidates in connection with our recently-announced strategic collaboration with Celgene;
- manufacture clinical study materials and develop large-scale manufacturing capabilities;
- seek regulatory approval for our product candidates;
- add personnel to support our product development and commercialization efforts; and
- operate as a public company.

We do not expect to generate revenue from product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our product candidates, which we expect will take a number of years and is subject to significant uncertainty. We have no manufacturing facilities and all of our manufacturing activities are contracted out to third parties. Additionally, we currently utilize third-party contract research organizations, or CROs, to carry out our clinical development activities and we do not yet have a sales organization. If we obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing, and distribution. Accordingly, we will seek to fund our operations through public or private equity or debt financings or other sources. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements as and when needed would have a negative impact on our financial condition and our ability to develop our products.

## **Financial operations overview**

### ***Revenue***

To date, we have not generated any revenues from the sales of products. Our revenues have been derived from collaboration arrangements, research fees, license fees, and grant revenues.



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Collaboration revenue is generated exclusively from our collaboration arrangement with Celgene. The terms of this arrangement contain multiple deliverables, which include at inception: (i) discovery, research and development services, (ii) participation on the joint steering committee and (iii) participation on the patent committee. We recognize arrangement consideration allocated to each unit of accounting when all of the revenue recognition criteria in Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, Topic 605, *Revenue Recognition*, or ASC 605, are satisfied for that particular unit of accounting. Revenue from the Celgene arrangement associated with discovery, research and development services, joint steering committee services and patent committee services is recognized ratably over the associated period of performance.

Research and license fee revenue is primarily generated through license and research and development agreements with strategic partners and nonprofit organizations for the development and commercialization of our product candidates. There are no performance, cancellation, termination, or refund provisions in any of our arrangements that contain material financial consequences to us.

Nonrefundable license fees are recognized as revenue upon delivery provided there are no undelivered elements in the arrangement. Research fees are recognized as revenue over the period we perform the associated services or on a straight-line basis if the pattern of performance cannot be estimated.

Grant revenue is primarily generated through research and development grant programs offered by federal, state, and local governments. Revenue is recognized when there is reasonable assurance that the grant will be received and we have complied with the terms of the grant.

Our ability to generate product revenue and become profitable depends upon our ability to successfully commercialize products. We expect to incur losses for the foreseeable future, and we expect these losses to increase as we continue our development of, and seek regulatory approvals for, our product candidates and begin to commercialize any approved products. Because of the numerous risks and uncertainties associated with product development, we are unable to predict the timing or amount of increased expenses or when or if we will be able to achieve or maintain profitability. Even if we are able to generate revenues from the sale of our products, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

### **Research and development expenses**

Research and development expenses consist primarily of costs incurred for the development of our product candidates, which include:

- employee-related expenses, including salaries, benefits, travel and stock-based compensation expense;
- expenses incurred under agreements with CROs and investigative sites that will conduct our clinical studies;
- the cost of acquiring, developing, and manufacturing clinical study materials;
- facilities, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance, and other supplies; and
- costs associated with preclinical activities and regulatory operations.

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Research and development costs are expensed as incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and our clinical sites.

We cannot determine with certainty the duration and completion costs of the current or future clinical studies of our product candidates or if, when, or to what extent we will generate revenues from the commercialization and sale of any of our product candidates that obtain regulatory approval. We may never succeed in achieving regulatory approval for any of our product candidates. The duration, costs, and timing of clinical studies and development of our product candidates will depend on a variety of factors, including:

- the scope, rate of progress, and expense of our ongoing as well as any additional clinical studies and other research and development activities;
- future clinical study results;
- uncertainties in clinical study enrollment rate;
- significant and changing government regulation; and
- the timing and receipt of any regulatory approvals.

A change in the outcome of any of these variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. For example, if the FDA, or another regulatory authority were to require us to conduct clinical studies beyond those that we currently anticipate will be required for the completion of clinical development of a product candidate or if we experience significant delays in enrollment in any of our clinical studies, we could be required to expend significant additional financial resources and time on the completion of clinical development.

From inception through March 31, 2013, we have incurred \$69.8 million in research and development expenses. We plan to increase our research and development expenses for the foreseeable future as we continue the development of our Lenti-D and LentiGlobin product candidates and conduct research and development activities under our recently-announced strategic collaboration with Celgene. Our current planned research and development activities include the following:

- We plan to initiate during late 2013 a Phase II/III clinical study to examine the feasibility, safety and efficacy of our Lenti-D product candidate.
- We plan to initiate during mid-2013 a Phase I/II clinical study in France to study the feasibility, safety and efficacy of our LentiGlobin product candidate in subjects with  $\beta$ -thalassemia major and SCD.
- We plan to initiate during mid-2013 a Phase I/II clinical study in the United States to study the feasibility, safety and efficacy of our LentiGlobin product candidate in subjects with  $\beta$ -thalassemia major.
- We will continue to manufacture clinical study materials in support of our clinical studies.

Our direct research and development expenses consist principally of external costs, such as startup fees paid to investigators, consultants, central laboratories and CROs in connection with our clinical studies, and costs related to acquiring and manufacturing clinical study materials. We

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do not allocate personnel-related costs, costs associated with our general platform improvements, depreciation or other indirect costs to specific programs, as they are deployed across multiple projects under development and, as such, are separately classified as personnel and other expenses in the table below:

(in thousands)	Year ended December 31,		Three months ended March 31,	
	2011	2012	2012	2013
			(unaudited)	
Lenti-D	\$ 2,900	\$ 3,966	\$ 1,100	\$ 1,076
LentiGlobin	1,416	5,259	551	1,362
Total direct research and development expenses	4,316	9,225	1,651	2,438
Employee and contractor-related expenses	5,090	6,150	1,686	2,055
Platform-related lab expenses	717	727	265	348
Facility expenses	619	709	187	295
Other expenses	667	399	69	148
Personnel and other expenses	7,093	7,985	2,207	2,846
Total research and development expenses	\$ 11,409	\$ 17,210	\$ 3,858	\$ 5,284

**General and administrative expenses**

General and administrative expenses consist primarily of salaries and related costs for personnel, including stock-based compensation and travel expenses for our employees in executive, operational, finance and human resource functions. Other general and administrative expenses include facility-related costs and professional fees for directors, accounting and legal services and expenses associated with obtaining and maintaining patents.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research and development and potential commercialization of our product candidates. We also anticipate increased expenses related to audit, legal, regulatory, and tax-related services associated with maintaining compliance with exchange listing and Securities and Exchange Commission requirements, director and officer insurance premiums, and investor relations costs associated with being a public company. Additionally, if and when we believe a regulatory approval of the first product candidate appears likely, we anticipate an increase in payroll and related expenses as a result of our preparation for commercial operations, especially as it relates to the sales and marketing of our product candidates.

**Other income (expense), net**

Other income and expense consists primarily of interest income earned on cash and cash equivalents and the re-measurement gain or loss associated with the change in the fair value of the preferred stock warrant liability.

We use the Black-Scholes option pricing model to estimate the fair value of the warrants. We base the estimates in the Black-Scholes option pricing model, in part, on subjective assumptions, including stock price volatility, risk-free interest rate, dividend yield, and the fair value of the

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preferred stock underlying the warrants. The re-measurement gain or loss associated with the change in the fair value of the preferred stock warrant liability each reporting period is recognized as a component of other income (expense), net.

## **Critical accounting policies and significant judgments and estimates**

Our management's discussion and analysis of our financial condition and results of operations are based on our financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, and expenses and the disclosure of contingent assets and liabilities in our financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related to accrued expenses and stock-based compensation. We base our estimates on historical experience, known trends and events, and various other factors that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in the notes to our financial statements appearing elsewhere in this prospectus, we believe the following accounting policies to be most critical to the judgments and estimates used in the preparation of our financial statements.

### ***Revenue recognition***

We have primarily generated revenue through collaboration arrangements, research arrangements and license arrangements with strategic partners and nonprofit organizations for the development and commercialization of product candidates. Additionally, we have generated revenue from research and development grant programs.

We recognize revenue in accordance with ASC 605. Accordingly, revenue is recognized for each unit of accounting when all of the following criteria are met:

- Persuasive evidence of an arrangement exists
- Delivery has occurred or services have been rendered
- The seller's price to the buyer is fixed or determinable
- Collectability is reasonably assured

Amounts received prior to satisfying the revenue recognition criteria are recorded as deferred revenue in the our consolidated balance sheets. Amounts expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, current portion. Amounts not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, net of current portion.

### ***Collaboration revenue***

As of March 31, 2013, our collaboration revenue was generated exclusively from our collaboration arrangement with Celgene. The terms of this arrangement contains multiple deliverables, which include at inception: (i) discovery, research and development services, (ii) participation on the joint steering committee and (iii) participation on the patent committee. The

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collaboration arrangement also provides Celgene with the option to obtain a license to any product candidates resulting from the collaboration. Moreover, Celgene has the option to extend the term of the collaboration arrangement, first for a period of two years and then for an additional period of one year. Additionally, we have the sole right to manufacture or have manufactured supplies of vectors and associated payloads manufactured for incorporation into the associated product candidate in the event a product candidate is licensed. Non-refundable payments to us under this arrangement may include: (i) up-front research fees, (ii) product candidate license fees, (iii) extension term research fees, (iv) payments for the manufacture and supply of vectors and payloads, (v) payments based on the achievement of certain milestones and (vi) royalties on product sales. Additionally, we may elect to share in the costs incurred from the development, commercialization and manufacture of product candidates licensed by our collaborators and earn our share of the net profits or bear our share of the net losses generated from the sale of product candidates licensed by our collaborators.

We analyze multiple-element arrangements based on the guidance in FASB ASC Topic 605-25, *Revenue Recognition-Multiple-Element Arrangements*, or ASC 605-25. Pursuant to the guidance in ASC 605-25, we evaluate multiple-element arrangements to determine (1) the deliverables included in the arrangement and (2) whether the individual deliverables represent separate units of accounting or whether they must be accounted for as a combined unit of accounting. This evaluation involves subjective determinations and requires us to make judgments about the individual deliverables and whether such deliverables are separable from the other aspects of the contractual relationship. Deliverables are considered separate units of accounting provided that: (i) the delivered item(s) has value to the customer on a standalone basis and (ii) if the arrangement includes a general right of return relative to the delivered item(s), delivery or performance of the undelivered item(s) is considered probable and substantially in our control. In assessing whether an item has standalone value, we consider factors such as the research, manufacturing and commercialization capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. In addition, we consider whether the collaboration partner can use the other deliverable(s) for their intended purpose without the receipt of the remaining element(s), whether the value of the deliverable is dependent on the undelivered item(s) and whether there are other vendors that can provide the undelivered element(s). The collaboration arrangement does not contain a general right of return relative to the delivered item(s).

Arrangement consideration that is fixed or determinable is allocated among the separate units of accounting using the relative selling price method. Then, the applicable revenue recognition criteria in ASC 605 are applied to each of the separate units of accounting in determining the appropriate period and pattern of recognition. We determine the selling price of a unit of accounting following the hierarchy of evidence prescribed by ASC 605-25. Accordingly, we determine the estimated selling price for units of accounting within each arrangement using vendor-specific objective evidence, or VSOE, of selling price, if available, third-party evidence, or TPE, of selling price if VSOE is not available, or best estimate of selling price, or BEBP, if neither VSOE nor TPE is available. We typically use BEBP to estimate the selling price, since we generally do not have VSOE or TPE of selling price for our units of accounting. Determining the BEBP for a unit of accounting requires significant judgment. In developing the BEBP for a unit of accounting, we consider applicable market conditions and relevant entity-specific factors, including factors that were contemplated in negotiating the agreement with the customer and estimated costs. We validate the BEBP for units of accounting by evaluating

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whether changes in the key assumptions used to determine the BESP will have a significant effect on the allocation of arrangement consideration between multiple units of accounting.

Options are considered substantive if, at the inception of the arrangement, we are at risk as to whether the collaboration partner will choose to exercise the option. Factors that we consider in evaluating whether an option is substantive include the overall objective of the arrangement, the benefit the collaborator might obtain from the arrangement without exercising the option, the cost to exercise the option and the likelihood that the option will be exercised. For arrangements under which an option is considered substantive, we do not consider the item underlying the option to be a deliverable at the inception of the arrangement and the associated option fees are not included in allocable arrangement consideration, assuming the option is not priced at a significant and incremental discount. Conversely, for arrangements under which an option is not considered substantive or if an option is priced at a significant and incremental discount, we would consider the item underlying the option to be a deliverable at the inception of the arrangement and a corresponding amount would be included in allocable arrangement consideration. All of the options included in our collaboration arrangement have been determined to be substantive, and none of the options are priced at a significant and incremental discount.

We recognize arrangement consideration allocated to each unit of accounting when all of the revenue recognition criteria in ASC 605 are satisfied for that particular unit of accounting. We will recognize as revenue arrangement consideration attributed to licenses that have standalone value from the other deliverables to be provided in an arrangement upon delivery. We will recognize as revenue arrangement consideration attributed to licenses that do not have standalone value from the other deliverables to be provided in an arrangement over our estimated performance period as the arrangement would be accounted for as a single unit of accounting.

We recognize revenue from the Celgene arrangement associated with discovery, research and development services, joint steering committee services and patent committee services ratably over the associated period of performance. If there is no discernible pattern of performance and/or objectively measurable performance measures do not exist, then we recognize revenue under the arrangement on a straight-line basis over the period we expect to complete our performance obligations. Conversely, if the pattern of performance in which the service is provided to the customer can be determined and objectively measurable performance measures exist, then we recognize revenue under the arrangement using the proportional performance method. Revenue recognized is limited to the lesser of the cumulative amount of payments received or the cumulative amount of revenue earned, as determined using the straight-line method or proportional performance method, as applicable, as of the period ending date.

At the inception of an arrangement that includes milestone payments, we evaluate whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This evaluation includes an assessment of whether: (i) the consideration is commensurate with either our performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from our performance to achieve the milestone, (ii) the consideration relates solely to past performance and (iii) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. We evaluate factors such as the scientific, clinical, regulatory, commercial and other risks that must be overcome to achieve the respective milestone and the level of effort and

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investment required to achieve the respective milestone in making this assessment. There is considerable judgment involved in determining whether a milestone satisfies all of the criteria required to conclude that a milestone is substantive. We have concluded that all of the clinical and regulatory milestones pursuant to its collaboration arrangement are substantive. Accordingly, in accordance with FASB ASC Topic 605-28, *Revenue Recognition-Milestone Method*, revenue from clinical and regulatory milestone payments will be recognized in its entirety upon successful accomplishment of the milestone, assuming all other revenue recognition criteria are met. Milestones that are not considered substantive would be recognized as revenue over the remaining period of performance, assuming all other revenue recognition criteria are met. Revenue from commercial milestone payments will be accounted for as royalties and recorded as revenue upon achievement of the milestone, assuming all other revenue recognition criteria are met.

We will recognize royalty revenue in the period of sale of the related product(s), based on the underlying contract terms, provided that the reported sales are reliably measurable and we have no remaining performance obligations, assuming all other revenue recognition criteria are met.

***Accrued research and development expenses***

As part of the process of preparing our financial statements, we are required to estimate our accrued expenses. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. The majority of our service providers invoice us monthly in arrears for services performed or when contractual milestones are met. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary. Examples of estimated accrued research and development expenses include fees paid to:

- CROs in connection with clinical studies;
- investigative sites in connection with clinical studies;
- vendors in connection with preclinical development activities; and
- vendors related to product manufacturing, development and distribution of clinical supplies.

We base our expenses related to clinical studies on our estimates of the services received and efforts expended pursuant to contracts with multiple CROs that conduct and manage clinical studies on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the clinical expense. Payments under some of these contracts depend on factors such as the successful enrollment of subjects and the completion of clinical study milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepaid accordingly.

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Although we do not expect our estimates to be materially different from amounts actually incurred, if our estimates of the status and timing of services performed differs from the actual status and timing of services performed we may report amounts that are too high or too low in any particular period. To date, there has been no material differences from our estimates to the amount actually incurred.

### **Stock-based compensation**

#### *Stock-based awards*

We issue stock-based awards to employees and non-employees, generally in the form of stock options and restricted stock. We account for our stock-based awards in accordance with FASB ASC Topic 718, *Compensation—Stock Compensation*, or ASC 718. ASC 718 requires all stock-based payments to employees, including grants of employee stock options and modifications to existing stock options, to be recognized in the consolidated statements of operations and comprehensive loss based on their fair values. We account for stock-based awards to non-employees in accordance with FASB ASC Topic 505-50, *Equity-Based Payments to Non-Employees*, which requires the fair value of the award to be remeasured at fair value as the award vests. We recognize the compensation cost of stock-based awards to employees on a straight-line basis over the vesting period of the award and using an accelerated attribution model for awards to non-employees. Described below is the methodology we have utilized in measuring stock-based compensation expense. Following the consummation of this offering, stock option and restricted stock values will be determined based on the quoted market price of our common stock.

We estimate the fair value of our stock-based awards to employees and non-employees using the Black-Scholes option pricing model, which requires the input of highly subjective assumptions, including (a) the expected volatility of our stock, (b) the expected term of the award, (c) the risk-free interest rate, and (d) expected dividends. Due to the lack of a public market for the trading of our common stock and a lack of company specific historical and implied volatility data, we have based our estimate of expected volatility on the historical volatility of a group of similar companies that are publicly traded. For these analyses, we have selected companies with comparable characteristics to ours including enterprise value, risk profiles, position within the industry, and with historical share price information sufficient to meet the expected life of the stock-based awards. We compute the historical volatility data using the daily closing prices for the selected companies' shares during the equivalent period of the calculated expected term of our stock-based awards. We will continue to apply this process until a sufficient amount of historical information regarding the volatility of our own stock price becomes available. We have estimated the expected life of our employee stock options using the "simplified" method, whereby, the expected life equals the average of the vesting term and the original contractual term of the option. The risk-free interest rates for periods within the expected life of the option are based on the U.S. Treasury yield curve in effect during the period the options were granted.

We are also required to estimate forfeitures at the time of grant, and revise those estimates in subsequent periods if actual forfeitures differ from its estimates. We use historical data to estimate pre-vesting option forfeitures and record stock-based compensation expense only for those awards that are expected to vest. To the extent that actual forfeitures differ from our estimates, the difference is recorded as a cumulative adjustment in the period the estimates were revised. Stock-based compensation expense recognized in the financial statements is based on awards that are ultimately expected to vest.



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We have computed the fair value of employee stock options at date of grant using the following weighted-average assumptions:

	Year ended		Three months	
	December 31,	December 31,	ended March 31,	ended March 31,
	2011	2012	2012	2013
Expected volatility	83.0%	79.6%	78.8%	82.0%
Expected term (in years)	6.1	6.1	6.1	6.1
Risk-free interest rate	1.7%	1.0%	1.1%	1.0%
Expected dividend yield	0.0%	0.0%	0.0%	0.0%

The following table presents the grant dates, number of underlying shares and related exercise prices or purchase prices of stock options granted and restricted stock awards, or RSAs, issued between January 1, 2011 and March 31, 2013, along with the corresponding exercise price for each option grant and the fair value per share utilized to calculate stock-based compensation expense:

Date of grant	Type of award	Number of shares	Exercise price (options) or purchase price (restricted stock) per share	Common stock fair value per share on grant date
7/13/2011	Option	11,818,294	\$ 0.11	\$ 0.11
7/13/2011	Restricted stock award	272,941	0.11	0.11
10/25/2011	Option	5,920,069	0.11	0.11
1/8/2012	Option	3,591,408	0.11	0.11
2/10/2012	Option	1,236,182	0.11	0.11
4/13/2012	Option	2,982,369	0.11	0.11
6/4/2012	Option	4,045,000	0.11	0.11
10/9/2012	Option	3,044,000	0.14	0.14
1/16/2013	Option	26,554,400	0.29	0.29
2/4/2013	Option	1,290,000	0.29	0.29

Stock-based compensation totaled approximately \$0.8 million for the year ended December 31, 2012 and \$0.7 million for the three months ended March 31, 2013. As of March 31, 2013, we had \$6.5 million of total unrecognized compensation expense, net of related forfeiture estimates, which is expected to be recognized over a weighted-average remaining vesting period of approximately 3.6 years. We expect the impact of our stock-based compensation expense for stock options and restricted stock granted to employees and non-employees to grow in future periods due to the potential increases in the value of our common stock and headcount.

*Fair value of stock options*

We have historically granted stock options at exercise prices not less than the fair value of our common stock. As there has been no public market for our common stock to date, the estimated fair value of our common stock has been determined contemporaneously by our board of directors based on valuation estimates provided by management and prepared in accordance with the framework of the American Institute of Certified Public Accountants' Technical Practice Aid, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*, or AICPA Practice Aid, as well as independent third-party valuations. Our contemporaneous valuations of

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our common stock as of April 21, 2011, April 15, 2012, July 23, 2012, December 31, 2012 and March 31, 2013 were based on a number of objective and subjective factors, including external market conditions affecting the biotechnology industry sector and the prices at which we sold shares of preferred stock, the superior rights and preferences of securities senior to our common stock at the time of each grant and the likelihood of achieving a liquidity event such as an initial public offering.

*April 21, 2011 valuation*

For the contemporaneous valuation at April 21, 2011, we used the back-solve method of the option-pricing method, or OPM, which derives the implied equity value for one type of equity security from a contemporaneous transaction involving another type of equity security. We applied the OPM back-solve method to solve for the equity value and corresponding value of common stock based on the \$0.37554 per share price for the sale of Series C preferred stock in April 2011, which was led by an unrelated investor that had not previously invested in our Company. Given the proximity to the initial Series C preferred stock financing, we believe the per share issuance price of the Series C preferred stock provides an indication of the fair value of our equity as of April 21, 2011.

The OPM treats common stock and preferred stock as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under this method, the common stock has value only if the funds available for distribution to stockholders exceed the value of the liquidation preference at the time of a liquidity event, such as a strategic sale, merger or initial public offering, or IPO, assuming the enterprise has funds available to make a liquidation preference meaningful and collectible by the holders of preferred stock. The common stock is modeled as a call option on the underlying equity value at a predetermined exercise price. In the model, the exercise price is based on a comparison with the total equity value rather than, as in the case of a regular call option, a comparison with a per share stock price. Thus, common stock is considered to be a call option with a claim on the enterprise at an exercise price equal to the remaining value immediately after the preferred stock is liquidated. The option-pricing method uses the Black-Scholes option pricing model to price the call options. This model defines the securities' fair values as functions of the current fair value of a company and uses assumptions such as the anticipated timing of a potential liquidity event and the estimated volatility of the equity securities.

We estimated the time to liquidity as 3.3 years based on then-current plans and estimates of our board of directors and management regarding a liquidity event. The risk free rate was estimated as the interpolated 3.3 year yield on government bonds.

We applied a discount for lack of marketability to the value indicated for our common stock. A discount is appropriate because our common stock is unregistered, and the holder of a minority interest in the common stock may not influence the timing of a liquidity event for our Company. Our estimate of the appropriate discount for lack of marketability took into consideration put option methodologies consistent with the AICPA Practice Aid. A put option model indicated a discount of 47%. We selected a smaller discount after taking into account empirical studies of restricted stock issued by publicly-traded companies.

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The following table summarizes the significant assumptions used to determine the fair value of our common stock of \$0.11 as of April 21, 2011:

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**April 21, 2011 valuation**

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**Key assumptions**

Liquidity date	8/8/2014
Annual volatility	75%
Risk-free interest rate	1.3%
Discount for lack of marketability (DLOM)	35%
Estimated per share present value of marketable common stock (before DLOM)	\$ 0.17

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*April 15, 2012 valuation*

For the contemporaneous valuation at April 15, 2012, we used the guideline public company, or GPC, method under the market approach to value our equity. We identified two categories of GPCs. The first category consists of GPCs which are comparable to our Company in certain respects, such as a focus on gene therapy, dependence on a relatively limited number of compounds and exposure to risks associated with clinical studies. Similar to our Company, the majority of the GPCs have more than one product in various stages of development. The companies in this category are AVI BioPharma, CytRx Corporation, Oxford BioMedica, Sangamo Biosciences, and Synageva BioPharma. We considered the average enterprise values of these companies as one indication of the value of our equity. The second category consists of GPCs in the drug development industry which have completed IPOs within the year preceding the April 15, 2012 appraisal date. These companies differ in therapy focus but are similar to our Company in that they depend on a relatively limited number of compounds and are subject to risks associated with clinical studies. As an indicator of value, we considered the increase in value, or step-up, from the most-recent preferred round to the IPO price for each of these GPCs. We considered the median step-up as one indication of value for our equity. The values indicated by these two categories of GPCs were similar, and we assumed an average of the two values.

For the valuation at April 15, 2012, we used the OPM to allocate equity value among our preferred and common securities. Significant assumptions for the OPM included volatility, the risk-free rate, and the time to liquidity. We calculated annual rates of volatility based on weekly historical trading data for a group of guideline public companies. The estimated time to liquidity was based on a 45% probability of liquidity in 2.72 years, a 45% probability of liquidity in 3.72 years and a 10% probability of liquidity in 1.46 years. The anticipated timing and probability of a liquidity event was based on then-current plans and estimates of our board of directors and management. The weighted-average time to liquidity was 3.04 years. We used the yield on three-year U.S. Treasuries as a risk-free rate.

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We applied a discount for lack of marketability to the value indicated for our common stock. A discount is appropriate because our common stock is unregistered, and the holder of a minority interest in the common stock may not influence the timing of a liquidity event for our Company. Our estimate of the appropriate discount for lack of marketability took into consideration put option methodologies consistent with the AICPA Practice Aid. Put option models indicated discounts of 30 to 68%. We selected a smaller discount after taking into account empirical studies of restricted stock issued by publicly-traded companies. The following table summarizes the significant assumptions used to determine the fair value of our common stock of \$0.11 as of April 15, 2012:

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**April 15, 2012 valuation**

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**Key assumptions**

Liquidity date	7/5/2015
Annual volatility	72%
Risk-free interest rate	0.4%
Discount for lack of marketability (DLOM)	25%
Estimated per share present value of marketable common stock (before DLOM)	\$ 0.15

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*July 23, 2012 valuation*

For the contemporaneous valuation at July 23, 2012, we used a hybrid of the probability-weighted expected return method, or PWERM, and the OPM, which we refer to as the hybrid method. Under the PWERM, share value is derived from the probability-weighted present value of expected future investment returns, considering possible outcomes available to us, as well as the economic and control rights of each share class. Our July 23, 2012 valuation considers two possible outcomes: an IPO and a later, unspecified liquidity event. The hybrid method is a PWERM where the values in one of the scenarios is calculated using an OPM. The hybrid method considers one IPO scenario and one OPM scenario. For the OPM scenario, the type of liquidity event, or outcome is undefined. In order to estimate the investment return for the IPO scenario, we considered the increase in value, or step-up, from the most-recent preferred round to the IPO price for a group of drug development companies which completed IPOs in the year preceding the appraisal date. We calculated the step-up as an annual rate of return. We applied this rate of return to our Series D preferred price to estimate its future value in the event of an IPO. For the IPO scenario, we assumed a future equity value equal to the product of the future value of Series D preferred stock times the number of common equivalent shares outstanding. The future equity value at the expected IPO date was allocated to each class of preferred stock and the common stock assuming conversion of all preferred classes to common. We estimated the time to an IPO date as 2.44 years based on our board of directors' assessment of our prospects, our investors' motivations and market conditions. We then discounted the values of each class of equity in the IPO scenarios at an appropriate risk-adjusted rate. We assumed risk-adjusted rates of 25% for the preferred shares and 30% for the common shares. We selected these risk-adjusted rates based on studies of the rates of return expected by venture capital investors, as presented in the AICPA Practice Aid.

In the OPM scenario, we applied the OPM back-solve method to solve for the equity value and corresponding value of common stock based on the \$0.4983 per share price for the sale of Series D preferred stock in July 2012. Given the proximity to the Series D preferred stock financing, and the fact that the Series D preferred stock financing included and was led by

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unrelated investors, we believe the per share issuance price of the Series D preferred stock provides an indication of the fair value of our equity as of July 23, 2012. The values indicated for the preferred and common shares by the IPO scenario and the OPM scenario were probability weighted to calculate the weighted value as of the July 23, 2012 valuation date.

For the July 23, 2012 valuation, we estimated the fair value of our common stock by assigning an 85% weighting to the estimated fair value using the OPM back-solve method and a 15% weighting to the estimated fair value under the IPO scenario. We believe that the 85% weighting on the OPM back-solve method is appropriate due to the proximity of the issuance of our Series D preferred stock in July 2012 to the valuation date and the fact that the issuance included and was led by unrelated investors. The 15% weighting for the IPO scenario was deemed appropriate because at the time of the valuation, we believed that there was the possibility of following a successful Series D financing with an IPO.

Significant assumptions for the OPM include volatility, the risk-free rate, and the time to liquidity. We calculated annual rates of volatility based on weekly historical trading data for a group of guideline public companies. For the OPM scenario, the estimated time to liquidity was 3 years. The anticipated timing of a liquidity event was management's estimate in the event our planned IPO does not occur. We used the yield on three-year U.S. Treasuries as a risk-free rate.

We applied a discount for lack of marketability to the value indicated for our common stock. We lowered our estimate of the discount for lack of marketability to 20% based on our perception of our improved prospects for an IPO.

The following table summarizes the significant assumptions used in the hybrid method to determine the fair value of our common stock of \$0.14 as of July 23, 2012:

<b>July 23, 2012 valuation</b>	<b>IPO</b>	<b>OPM</b>
<b>Key assumptions</b>		
Probability weighting	15%	85%
Liquidity date	1/1/2015	7/23/2015
Weighted-average cost of capital	25%	NA
Annual volatility	NA	70%
Risk-free interest rate	NA	0.3%
Discount for lack of marketability (DLOM)	20%	20%
Estimated per share present value of marketable common stock (before DLOM and probability weighting)	\$ 0.31	\$ 0.15

The estimated per share fair value of our common stock calculated in our valuation as of July 23, 2012 of \$0.14 per share increased from the April 15, 2012 valuation of \$0.11 per share primarily due to the following factors:

- our improved financial position resulting from the issuance of 120.4 million shares in July 2012 of our Series D preferred stock for an aggregate purchase price of \$60.0 million;
- regulatory feedback from the FDA on the design of our Phase II/III Lenti-D study;
- regulatory feedback from the FDA on the nonclinical, manufacturing and clinical design of our Phase I/II LentiGlobin study;

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- filing of our clinical trial application, or CTA, in France for our Phase I/II LentiGlobin study; and
- receipt of orphan drug designation for our Lenti-D program in the United States and European Union.

*December 31, 2012 valuation*

For the contemporaneous valuation at December 31, 2012, we used the hybrid method with one IPO scenario and one OPM scenario. As an indicator of value for the IPO scenario, we considered the increase in value, or step-up, from the most recent preferred round to the IPO price for a group of drug development companies which completed IPOs in the year preceding the appraisal date. We calculated the step-up as an annual rate of return. We applied this rate of return to our Series D preferred price to estimate its future value in the event of an IPO. For the IPO scenario, we assumed a future equity value equal to the product of the future value of Series D preferred stock times the number of common equivalent shares outstanding. The future equity value at the expected IPO date was allocated to each class of preferred stock and the common stock assuming conversion of all preferred classes to common. We estimated the time to an IPO date as one year based on our board of directors' assessment of our prospects, our investors' motivations and market conditions. We then discounted the values of each class of equity in the IPO scenarios at an appropriate risk-adjusted rate. We assumed risk-adjusted rates of 25% for the preferred shares and 30% for the common shares. We selected these risk-adjusted rates based on studies of the rates of return expected by venture capital investors, as presented in the AICPA Practice Aid. In the OPM scenario, we assumed an equity value equal to the present value of our equity in a future IPO.

For the December 31, 2012 valuation, we estimated the fair value of our common stock by assigning a 60% weighting to the estimated fair value using the OPM and a 40% weighting to the estimated fair value under the IPO scenario. We deemed the 40% weighting of our IPO scenario appropriate because of our progress since July 2012 in preparing for a potential IPO, which included advancements of our negotiations with a potential partner, completion of GMP-grade vector lots, qualification of a transduction manufacturing facility, advancement of our IND and CTA applications and engagement in initial discussions with underwriters.

Significant assumptions for the OPM include volatility, the risk-free rate, and the time to liquidity. We calculated annual rates of volatility based on weekly historical trading data for a group of guideline public companies. For the OPM scenario, the estimated time to liquidity was 2.56 years. The anticipated timing of a liquidity event was management's estimate in the event our planned IPO does not occur. We used the yield on three-year U.S. Treasuries as a risk-free rate.

We applied a discount for lack of marketability to the value indicated for our common stock. We lowered our estimate of the discount for lack of marketability to 10% based on our perception of the company's improved prospects for an IPO.

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The following table summarizes the significant assumptions used in the hybrid method to determine the fair value of our common stock of \$0.29 as of December 31, 2012:

<b>December 31, 2012 valuation</b>	<b>IPO</b>	<b>OPM</b>
<b>Key assumptions</b>		
Probability weighting	40%	60%
Liquidity date	12/31/2013	7/23/2015
Weighted-average cost of capital	25%	NA
Annual volatility	NA	71%
Risk-free interest rate	NA	0.4%
Discount for lack of marketability (DLOM)	10%	10%
Estimated per share present value of marketable common stock (before DLOM and probability weighting)	\$ 0.48	\$ 0.22

The estimated per share fair value of our common stock calculated in our valuation as of December 31, 2012 of \$0.29 per share increased significantly from the July 23, 2012 valuation of \$0.14 per share. This is primarily due to the following factors:

- potential partnership with a leading pharmaceutical company that would extend our platform into oncology indication;
- increased probability of taking our Company public;
- successful manufacturing of two GMP-grade vector lots for our Lenti-D and LentiGlobin programs;
- successful completion of our LentiGlobin transduction manufacturing qualification at a centralized CRO;
- CTA approval of our  $\beta$ -thalassemia and SCD study in France;
- filing of an IND for our  $\beta$ -thalassemia program in the United States; and
- receipt of a \$9.3 million award from CIRM to fund our U.S. LentiGlobin study.

*March 31, 2013 valuation*

For the contemporaneous valuation at March 31, 2013, we used the hybrid method with one IPO scenario and one OPM scenario. As an indicator of value for the IPO scenario, we considered the increase in value, or step-up, from the most recent preferred round to the IPO price for a group of drug development companies which completed IPOs in the five quarters preceding the appraisal date. We calculated the step-up as an annual rate of return. We applied this rate of return to our Series D preferred price to estimate its future value in the event of an IPO. For the IPO scenario, we assumed a future equity value equal to the product of the future value of Series D preferred stock times the number of common equivalent shares outstanding. The future equity value at the expected IPO date was allocated to each class of preferred stock and the common stock assuming conversion of all preferred classes to common. We estimated the time to an IPO date as 0.42 years based on our board of directors' assessment of our prospects, our investors' motivations and market conditions. We then discounted the values of each class of equity in the IPO scenarios at an appropriate risk-adjusted rate. We assumed risk-adjusted rates of 25% for the

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preferred shares and 30% for the common shares. We selected these risk-adjusted rates based on studies of the rates of return expected by venture capital investors, as presented in the AICPA Practice Aid. In the OPM scenario, we assumed an equity value equal to the present value of our equity in a future IPO.

For the March 31, 2013 valuation, we estimated the value of our common stock by assigning a 30% weighting to the estimated value using the OPM and a 70% weighting to the estimated fair value under the IPO scenario. We deemed the 70% weighting of our IPO scenario appropriate because of our progress since December 2012 in preparing for a potential IPO which included entering into a strategic collaboration with Celgene, further advancement of our IND applications, including effectiveness of the IND for our LentiGlobin program, and the filing of our initial registration statement.

Significant assumptions for the OPM include volatility, the risk-free rate, and the time to liquidity. We calculated annual rates of volatility based on weekly historical trading data for a group of guideline public companies. For the OPM scenario, the estimated time to liquidity was 2.31 years. The anticipated timing of a liquidity event was management's estimate in the event our planned IPO does not occur. We used the yield on two-year U.S. Treasuries as a risk-free rate.

We applied a discount for lack of marketability to the value indicated for our common stock. We estimated the discount for lack of marketability to be 10% based on our perception of our prospects for an IPO.

The following table summarizes the significant assumptions used in the hybrid method to determine the fair value of our common stock of \$0.43 as of March 31, 2013:

<b>March 31, 2013 valuation</b>	<b>IPO</b>	<b>OPM</b>
<b>Key assumptions</b>		
Probability weighting	70%	30%
Liquidity date	8/31/2013	7/23/2015
Weighted-average cost of capital	25%	NA
Annual volatility	NA	72%
Risk-free interest rate	NA	0.3%
Discount for lack of marketability (DLOM)	10%	10%
Estimated per share present value of marketable common stock (before DLOM and probability weighting)	\$ 0.56	\$ 0.27

The estimated per share fair value of our common stock calculated in our valuation as of March 31, 2013 of \$0.43 per share increased from the December 31, 2012 valuation of \$0.29 per share. This is primarily due to the following factors:

- effectiveness of the IND for our LentiGlobin program in the United States;
- filing of an IND for our Lenti-D program in the United States;
- initial submission of our confidential draft registration statement on Form S-1 that increases the likelihood of a near-term liquidity event; and
- entering into a strategic collaboration with Celgene in March 2013 to discover, develop and commercialize novel, disease-altering gene therapies in oncology.



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*Initial public offering price*

In consultation with the underwriters for this offering, we determined the estimated price range for this offering, as set forth on the cover page of this prospectus. The midpoint of the price range is \$ \_\_\_\_\_ per share. In comparison, our estimate of the fair value of our common stock was \$ \_\_\_\_\_ per share as of \_\_\_\_\_. We note that, as is typical in IPOs, the estimated price range for this offering was not derived using a formal determination of fair value, but was determined by negotiation between us and the underwriters. Among the factors that were considered in setting this range were the following:

- an analysis of the typical valuation ranges seen in recent IPOs for companies in our industry;
- the general condition of the securities markets and the recent market prices of, and the demand for, publicly traded common stock of generally comparable companies;
- an assumption that there would be a receptive public trading market for pre-commercial biotechnology companies such as us; and
- an assumption that there would be sufficient demand for our common stock to support an offering of the size contemplated by this prospectus.

The midpoint of the estimated price range for this offering reflects an increase over the estimated valuation as of \_\_\_\_\_ of \$ \_\_\_\_\_ per share. Investors should be aware of this difference and recognize that the price range for this offering is in excess of our prior valuations. Further, investors are cautioned not to place undue reliance on the valuation methodologies discussed above as an indicator of future stock prices. We believe the difference may be due to the following factors:

- The initial offering price range necessarily assumes that this offering has occurred, a public market for our common stock has been created and that our preferred stock has converted into common stock in connection with this offering and, therefore, excludes the marketability or illiquidity discounts associated with the timing or likelihood of an initial public offering, the superior rights and preferences of our preferred stock and the alternative scenarios considered in the contemporaneous valuations over the past two years.
- In the public markets we believe there are investors who may apply more qualitative valuation criteria to certain of our clinical assets than the valuation methods applied in our valuations.
- The price that investors are willing to pay in this offering, for which the price range is intended to serve as an estimate, may take into account other things that have not been expressly considered in our prior valuations, are not objectively determinable and that valuation models are not able to quantify.

Investors should be cautioned that the midpoint of the price range set forth on the cover of this prospectus does not necessarily represent the fair value of our common stock, but rather reflects an estimate of the offer price determined in consultation with the underwriters.

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There are significant judgments and estimates inherent in the determination of these valuations. These judgments and estimates include assumptions regarding our future performance, including the successful enrollment and completion of our clinical studies as well as the determination of the appropriate valuation methods. If we had made different assumptions, our stock-based compensation expense could have been different. The foregoing valuation methodologies are not the only methodologies available and they will not be used to value our common stock once this offering is complete. We cannot make assurances as to any particular valuation for our common stock. Accordingly, investors are cautioned not to place undue reliance on the foregoing valuation methodologies as an indicator of future stock prices.

***Convertible preferred stock warrants***

As of March 31, 2013, we had warrants outstanding to purchase shares of Series A-1 and Series B preferred stock. Freestanding warrants that are related to the purchase of redeemable preferred stock are classified as liabilities and recorded at fair value regardless of the timing of the redemption feature or the redemption price or the likelihood of redemption. The warrants are subject to re-measurement at each balance sheet date and any change in fair value is recognized as a component of other income (expense), net. We measure the fair value of our warrant liability using a Black-Scholes option pricing model. Any modifications to the warrant liability are recorded in earnings during the period of the modification. The significant assumptions used in estimating the fair value of our warrant liability include the exercise price, volatility of the stock underlying the warrant, risk-free interest rate, estimated fair value of the preferred stock underlying the warrant, and the estimated life of the warrant.

As a result of the revision of the terms of our Series A-1 preferred stock upon the Series D financing, the redemption feature in the Series A-1 preferred stock is no longer present. Due to this change, we re-evaluated whether the warrants to purchase Series A-1 preferred stock represented a liability. Because the Series A-1 preferred stock does not contain any redemption feature or preference in liquidation, we concluded that the warrant should be classified as permanent equity. On the date of reclassification, we performed a final valuation of the Series A-1 warrants, with the change in value recorded to other income (expense), net. The fair value of the warrants was then reclassified to additional paid in capital.

Pursuant to the terms of these warrants, upon the conversion of the class of preferred stock underlying the warrant, the warrants automatically become exercisable for shares of our common stock based upon the conversion ratio of the underlying class of preferred stock. The consummation of this offering will result in the conversion of all classes of our preferred stock into common stock. Upon such conversion of the underlying classes of preferred stock, the remaining warrants to purchase Series B preferred stock will be classified as a component of equity and no longer be subject to re-measurement.

***Emerging growth company status***

The Jumpstart our Business Startups Act of 2012, or the JOBS Act, permits an “emerging growth company” such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies. We are choosing to “opt out” of this provision and, as a result, we will comply with new or revised accounting standards as required when they are adopted. This decision to opt out of the extended transition period under the JOBS Act is irrevocable.

### **Recently adopted accounting pronouncements**

In February 2013, the FASB issued guidance to provide information about the amounts reclassified out of accumulated other comprehensive income, or AOCI, by component. An entity is required to present, either on the face of the financial statements or in the notes, significant amounts reclassified out of AOCI by the respective line items of net income, but only if the amount reclassified is required to be reclassified in its entirety in the same reporting period. For amounts that are not required to be reclassified in their entirety to net income, an entity is required to cross-reference to other disclosures that provide additional details about those amounts. On January 1, 2013, we adopted this standard, which had no impact on our financial position or results of operations.

In June 2011, the FASB issued an amendment to the accounting guidance for presentation of comprehensive income. Under the amended guidance, a company may present the total of comprehensive income, the components of net income, and the components of other comprehensive income either in a single continuous statement of comprehensive income or in two separate but consecutive statements. In either case, a company is required to present each component of net income along with total net income, each component of other comprehensive income along with a total for other comprehensive income, and a total amount for comprehensive income. The amendment is effective for fiscal years ending, and interim periods within those years, beginning after December 15, 2011, and is applied retrospectively. We adopted this amendment in the accompanying financial statements by presenting comprehensive income in one consecutive statement along with net loss.

In May 2011, the FASB issued amended guidance on fair value measurements. This newly issued accounting standard clarifies the application of certain existing fair value measurement guidance and expands the disclosures for fair value measurements that are estimated using significant unobservable (Level 3) inputs. This accounting standard was effective on a prospective basis for annual and interim reporting periods beginning on or after December 15, 2011. The adoption of this standard has not had a material impact on our financial position or results of operations.

### **Results of operations**

#### **Comparison of the three months ended March 31, 2012 and 2013 (unaudited)**

<b>Three months ended March 31, (in thousands)</b>	<b>2012</b>	<b>2013</b>	<b>Increase (Decrease)</b>
	<b>(unaudited)</b>		
Revenue:			
Collaboration revenue	\$ —	\$ 1,042	\$ 1,042
Research and license fees	85	85	—
Total revenue	85	1,127	1,042
Expenses:			
Research and development	3,858	5,284	1,426
General and administrative	1,363	2,324	961
Total expenses	5,221	7,608	2,387
Loss from operations	(5,136)	(6,481)	(1,345)
Other income (expense), net	68	(63)	(131)
Net loss	\$ (5,068)	\$ (6,544)	\$ (1,476)

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**Revenue.** Revenue was \$1.0 million for the three months ended March 31, 2013, compared to \$0.1 million for the three months ended March 31, 2012. The increase of \$1.0 million from \$0.1 million is due to the Celgene collaboration. In the three months ended March 31, 2013, we recorded \$1.0 million in recognition of amounts allocated to research and development services from the Celgene collaboration, which was entered into in March 2013 and is expected to be recognized on a straight-line basis through March 2016, and \$0.1 million of research fees.

**Research and development expenses.** Research and development expenses were \$5.3 million for the three months ended March 31, 2013, compared to \$3.9 million for the three months ended March 31, 2012. The increase was primarily due to a \$0.4 million increase in employee- and contractor-related expenses to support increased development activities associated with three clinical studies planned to commence in 2013 and an \$0.8 million increase in clinical start-up activities related to our LentiGlobin program.

**General and administrative expenses.** General and administrative expenses were \$2.3 million for the three months ended March 31, 2013, compared to \$1.4 million for the three months ended March 31, 2012. The increase in spending is primarily due to \$0.6 million of employee- and contractor-related expenses to support corporate operational activities, including \$0.3 million of consultant costs incurred in connection with preparing for this offering.

**Other income (expense), net.**

Other income (expense), net was \$(0.1) million for the three months ended March 31, 2013, compared to \$0.1 million for the three months ended March 31, 2012. The decrease was primarily due to the re-measurement of the redeemable convertible preferred stock warrants and foreign currency losses.

**Comparison of the years ended December 31, 2011 and 2012**

<b>Year ended December 31, (in thousands)</b>	<b>2011</b>	<b>2012</b>	<b>Increase (Decrease)</b>
Revenue	\$ 882	\$ 340	\$ (542)
Expenses:			
Research and development	11,409	17,210	5,801
General and administrative	4,615	6,846	2,231
Total expenses	16,024	24,056	8,032
Loss from operations	(15,142)	(23,716)	(8,574)
Other income (expense), net	(456)	46	502
Net loss	\$ (15,598)	\$ (23,670)	\$ (8,072)

**Revenue.** We recorded \$0.3 million research fee revenue for the year ended December 31, 2012. For the year ended December 31, 2011, we recorded \$0.9 million in revenue consisting of \$0.3 million research fees, \$0.3 million license fees, and \$0.2 million grant revenue (a tax incentive from the Commonwealth of Massachusetts).

**Research and development expenses.** Research and development expenses were \$17.2 million for the year ended December 31, 2012, compared to \$11.4 million for the year ended December 31, 2011, an increase of \$5.8 million. The increase was primarily due to:

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- \$2.8 million increase for clinical supply manufacturing and drug product process development activities in preparation for the ALD-102, HGB-204, and HGB-205 clinical studies planned for 2013;
- \$1.1 million increase to employee and contractor-related expenses to support the increased development activities in 2012 in anticipation of the three clinical studies planned for 2013;
- \$0.8 million increase in lab supplies, assay transfer and validation activities to support clinical supply and process development activities;
- \$0.7 million increase in consulting fees to support regulatory filing and other clinical start-up activities; and
- \$0.3 million increase in license and milestone fees paid to third parties.

**General and administrative expenses.** General and administrative expenses were \$6.8 million for the year ended December 31, 2012, compared to \$4.6 million for the year ended December 31, 2011. The increase of \$2.2 million was due primarily to an increase of \$1.4 million in professional fees, \$0.6 million in employee and contractor-related expenses to support corporate operational and business development activities and \$0.5 million in office and facility expenses, which was partially offset by a decrease in market study-related expenses.

**Other income (expense), net.** Other income (expense), net, was \$0.05 million for the year ended December 31, 2012, compared to \$(0.5) million for the year ended December 31, 2011, an increase of approximately \$0.5 million. The increase was primarily due to revaluation of the redeemable convertible preferred stock warrants of \$0.4 million and \$0.1 million of currency losses.

## **Liquidity and capital resources**

We have incurred losses and cumulative negative cash flows from operations since our inception in April 1992, and as of March 31, 2013, we had an accumulated deficit of \$79.9 million. We anticipate that we will continue to incur losses for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our operations, which we may raise through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements.

We have funded our operations principally from the sale of common stock, preferred stock, convertible notes and warrants to purchase common stock. In addition, in October 2012, we were awarded a \$9.3 million grant from CIRM to fund our U.S. LentiGlobin study. This grant will be issued in quarterly installments and is expected to be utilized over a four-year period starting in the second quarter of 2013. In March 2013, we entered into a strategic collaboration with Celgene to discover, develop and commercialize novel, disease-altering gene therapies in oncology. This collaboration has an initial term of three years, and Celgene has made a \$75 million up-front, non-refundable cash payment to us as consideration for entering into the collaboration. As of March 31, 2013, we had cash and cash equivalents of approximately \$131.8 million. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation. Currently, our funds are held in money market mutual funds consisting of U.S. government-backed securities.

**Cash flows**

The following table sets forth the primary sources and uses of cash for each of the periods set forth below:

(in thousands)	Year ended December 31,		Three months ended March 31,	
	2011	2012	2012	2013
			(unaudited)	
Net cash provided by (used in):				
Operating activities	\$ (12,217)	\$ (21,044)	\$ (6,200)	\$ 66,018
Investing activities	(3,964)	2,599	3,175	(812)
Financing activities	32,435	59,852	—	(381)
Net (decrease) increase in cash and cash equivalents	\$ 16,254	\$ 41,407	\$ (3,025)	\$ 64,825

**Operating activities.** The significant increase in cash provided by operating activities for the three months ended March 31, 2013, compared to the three months ended March 31, 2012, is primarily due to the up-front payment related to the Celgene collaboration agreement. The significant increase in cash used in operating activities for the year ended December 31, 2012, compared to the year ended December 31, 2011, is primarily due to an increase in research and development expenses as we continue the development of our Lenti-D and LentiGlobin product candidates, which includes an increase in personnel related costs, process development and manufacturing activities. In addition, general and administrative expenses increased due to an increase in administrative personnel as well as professional and facility-related spending, offset by an increase in accrued expenses. The use of cash in all periods resulted primarily from our net losses adjusted for non-cash charges and favorable changes in components of working capital.

The net cash provided by operating activities was \$66.0 million for the three months ended March 31, 2013, and consisted primarily of a net loss of \$6.5 million adjusted for non-cash items including stock-based compensation expense of \$0.7 million and depreciation of \$0.1 million and a net increase in operating assets and liabilities of \$71.7 million. The significant items in the change in operating assets and liabilities include an increase in deferred revenue of \$73.9 million due to the up-front payment related to the Celgene collaboration partially offset by an increase in prepaid expenses and other current assets of \$1.1 million and a decrease in accounts payable of \$0.6 million and a decrease in accrued expenses and deferred rent of \$0.5 million.

The net cash used in operating activities was \$6.2 million for the three months ended March 31, 2012, and consisted primarily of a net loss of \$5.1 million adjusted for non-cash items including stock-based compensation expense of \$0.2 million and depreciation of \$0.1 million and a net decrease in operating assets and liabilities of \$1.3 million. The significant items in the change in operating assets and liabilities include decreases in accounts payable of \$1.1 million and deferred revenue of \$0.1 million and an increase in prepaid expenses and other current assets of \$0.5 million slightly offset by an increase in accrued expenses and other liabilities of \$0.4 million.

The net cash used in operating activities was \$21.0 million for the year ended December 31, 2012, and consisted primarily of a net loss of \$23.7 million adjusted for non-cash items including stock-based compensation expense of \$0.8 million and depreciation of \$0.3 million and a net increase in operating assets and liabilities of \$1.5 million. The significant items in the change in

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operating assets and liabilities include an increase in accounts payable of \$0.4 million and accrued expenses and other liabilities of \$1.4 million and a decrease in prepaid expenses and current assets of \$0.1 million, offset by a decrease in deferred revenue of \$0.3 million.

The net cash used in operating activities was \$12.2 million for the year ended December 31, 2011, and consisted primarily of a net loss of \$15.6 million adjusted for non-cash items including stock-based compensation expense of \$0.8 million, re-measurement of warrants of \$0.4 million, and depreciation of \$0.2 million and a net increase in operating assets and liabilities of \$1.9 million. The significant items in the change in operating assets and liabilities include increases in accounts payable of \$0.9 million, accrued expenses and other liabilities of \$0.4 million, and deferred revenues of \$1.0 million, slightly offset by a decrease in prepaid expenses and other current assets of \$0.3 million.

**Investing activities.** Net cash provided by (used in) investing activities consisted of purchases of fixed assets, purchases of marketable securities, and proceeds from the sale of marketable securities. Net cash used in investing activities for the three months ended March 31, 2013 was \$0.8 million and consisted primarily of purchases of property and equipment. Net cash provided by investing activities for the three months ended March 31, 2012 was \$3.2 million and consisted of proceeds from the sale of marketable securities of \$3.5 million slightly offset by purchases of property and equipment of \$0.3 million. Net cash provided by investing activities for the year ended December 31, 2012 was \$2.6 million and consisted primarily of proceeds from the sale of marketable securities of \$3.5 million slightly offset by purchases of property and equipment of \$0.9 million. Net cash used in investing activities for the year ended December 31, 2011, was \$4.0 million and was comprised primarily of purchases of marketable securities of \$5.3 million, slightly offset by proceeds from the sale of marketable securities of \$1.8 million and the purchases of property and equipment of \$0.4 million.

**Financing activities.** Net cash used in financing activities for the three months ended March 31, 2013 was \$0.4 million and consisted primarily of accumulated issuance costs related to our planned initial public offering. Net cash provided by financing activities for the year ended December 31, 2012 is the result of the sale of 120.4 million shares of our Series D preferred stock for net proceeds of \$59.8 million. Net cash provided by financing activities for the year ended December 31, 2011 is the result of the issuance and sale of 39.9 million shares of our Series C preferred stock for net proceeds of \$14.9 million, and the issuance and sale of 53.6 million shares of the second tranche of our Series B preferred stock for net proceeds of \$17.5 million for aggregate net proceeds of \$32.4 million.

**Operating capital requirements**

To date, we have not generated any revenue from product sales. We do not know when, or if, we will generate any revenue from product sales. We do not expect to generate significant revenue from product sales unless and until we obtain regulatory approval of and commercialize one of our current or future product candidates. We anticipate that we will continue to generate losses for the foreseeable future, and we expect the losses to increase as we continue the development of, and seek regulatory approvals for, our product candidates, and begin to commercialize any approved products. We are subject to all of the risks incident in the development of new gene therapy products, and we may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. Upon the closing of this offering, we expect to incur additional costs associated with

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operating as a public company. We anticipate that we will need substantial additional funding in connection with our continuing operations.

We believe that the net proceeds from this offering and our existing cash and cash equivalents will be sufficient to fund our projected operating requirements through at least the end of 2015. However, we may require additional capital for the further development of our existing product candidates and may also need to raise additional funds sooner to pursue other development activities related to additional product candidates.

Until we can generate a sufficient amount of revenue from our products, if ever, we expect to finance future cash needs through public or private equity or debt offerings. Additional capital may not be available on reasonable terms, if at all. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of one or more of our product candidates. If we raise additional funds through the issuance of additional debt or equity securities, it could result in dilution to our existing stockholders, increased fixed payment obligations and these securities may have rights senior to those of our common stock. If we incur indebtedness, we could become subject to covenants that would restrict our operations and potentially impair our competitiveness, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. Any of these events could significantly harm our business, financial condition and prospects.

Our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement and involves risks and uncertainties, and actual results could vary as a result of a number of factors. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the initiation, progress, timing, costs and results of clinical studies for our products, including our Phase II/III Lenti-D study and our Phase I/II LentiGlobin studies;
- the outcome, timing and cost of regulatory approvals by the FDA and comparable foreign regulatory authorities, including the potential for the FDA or comparable foreign regulatory authorities to require that we perform more studies than those that we currently expect;
- the ability of our product candidates to progress through clinical development successfully;
- the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights;
- our need to expand our research and development activities;
- our need and ability to hire additional personnel;
- our need to implement additional infrastructure and internal systems;
- the effect of competing technological and market developments; and
- the cost of establishing sales, marketing and distribution capabilities for any products for which we may receive regulatory approval.



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If we cannot expand our operations or otherwise capitalize on our business opportunities because we lack sufficient capital, our business, financial condition and results of operations could be materially adversely affected.

**Contractual obligations and commitments**

The following table summarizes our contractual obligations at December 31, 2012.

<b>(in thousands)</b>	<b>Total</b>	<b>Less than 1 Year</b>	<b>1 to 3 Years</b>	<b>3 to 5 Years</b>	<b>More than 5 Years</b>
Operating lease obligations(1)	\$1,885	\$ 831	\$ 841	\$ 213	\$ —

(1) We lease office space at 840 Memorial Drive in Cambridge, Massachusetts under a noncancelable operating lease that expires on March 31, 2015.

We also have obligations to make future payments to third parties that become due and payable on the achievement of certain development, regulatory and commercial milestones (such as the start of a clinical trial, filing of an NDA, approval by the FDA or product launch). We have not included these commitments on our balance sheet or in the table above because the achievement and timing of these milestones is not fixed and determinable. These commitments include:

- Under a license agreement with Inserm-Transfert pursuant to which we license certain patents for use in human adrenoleukodystrophy therapy, we will be required to make payments based upon development, regulatory and commercial milestones for any products covered by the in-licensed intellectual property. The maximum aggregate payments we may be obligated to pay for each of these milestone categories per product is €0.3, €0.2 and €1.6 million, respectively. We will also be required to pay a royalty on net sales of products covered by the in-licensed intellectual property in the low single digits. The royalty is subject to reduction for any third-party payments required to be made, with a minimum floor in the low single digits.
- Under a license agreement with Institut Pasteur pursuant to which we license certain patents for use in *ex vivo* gene therapy, we will be required to make payments per product covered by the in-licensed intellectual property upon the achievement of development and regulatory milestones, depending on the indication and the method of treatment. The maximum aggregate payments we may be obligated to pay for each of these milestone categories per product is €1.5 and €2.0 million, respectively. We will also be required to pay a royalty on net sales of products covered by the in-licensed intellectual property in the low single digits, which varies slightly depending on the indication of the product. We have the right to sublicense our rights under this agreement, and we will be required to pay a percentage of such license income varying from the low single digits to mid-double digits depending on the nature of the sublicense. Starting in 2016, we will be required to make an annual maintenance payment, which is creditable against royalty payments on a year-by-year basis.
- Under a license agreement with the Board of Trustees of the Leland Stanford Junior University, or Stanford, pursuant to which we license the HEK293T cell line for use in gene therapy products, we are required to pay a royalty on net sales of products covered by the in-licensed intellectual property in the low single digits that varies with net sales. The royalty is reduced for each third-party license that requires payments by us with respect to

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a licensed product, provided that the royalty to Stanford is not less than a specified percentage that is less than one percent. We are required to pay Stanford an annual maintenance fee based on net sales of licensed products, which is creditable against our royalty payments.

- Under a license agreement with the Massachusetts Institute of Technology, or MIT, pursuant to which we license various patents, we will be required to make a payment of \$0.1 million based upon a regulatory filing milestone. We will also be required to pay a royalty on net sales of products covered by the in-licensed intellectual property by us or our sublicensees. The royalty is in the low single digits and is reduced for royalties payable to third parties, provided that the royalty to MIT is not less than a specified percentage that is less than one percent. We have the right to sublicense our rights under this agreement, and we will be required to pay a percentage of such license income varying from the mid-single digits to low double digits. We are required to pay MIT an annual maintenance fee based on net sales of licensed products, which is creditable against our royalty payments.
- Under a license agreement with Research Development Foundation pursuant to which we license patents that involve lentiviral vectors, we will be required to make payments of \$1.0 million based upon a regulatory milestone for each product covered by the in-licensed intellectual property. We will also be required to pay a royalty on net sales of products covered by the in-licensed intellectual property in the low single digits, which is reduced by half if during the ten year following first marketing approval the last valid claim within the licensed patent that covers the licensed product expires or ends.

We enter into contracts in the normal course of business with CROs for preclinical research studies, research supplies and other services and products for operating purposes. These contracts generally provide for termination on notice, and therefore are cancelable contracts and not included in the table of contractual obligations and commitments.

### **Off-balance sheet arrangements**

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the Securities and Exchange Commission.

### **Quantitative and qualitative disclosures about market risks**

We are exposed to market risk related to changes in interest rates. As of March 31, 2012 and 2013, we had cash and cash equivalents of \$22.6 million and \$131.8 million, respectively, primarily money market mutual funds consisting of U.S. government-backed securities. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are in short-term securities. Our available for sale securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

# Business

## Overview

We are a clinical-stage biotechnology company focused on transforming the lives of patients with severe genetic and orphan diseases using gene therapy. Many diseases have a genetic aspect whereby a mutated gene linked to a disease is passed down from generation to generation and causes the disease. Gene therapy seeks to introduce a functional copy of the defective gene into a patient's own cells, a process called gene transfer. We believe that gene therapy has the potential to change the way these patients are treated by correcting the underlying genetic defect that is the *cause* of their disease, rather than offering solutions that only address their *symptoms*. Accordingly, we believe gene therapy has the potential to provide transformative disease modifying effects with life-long clinical benefits based on a single therapeutic administration.

Each person's hereditary genetic material, or genome, is encoded by deoxyribonucleic acid, or DNA, in sequences of genetic code called genes. Genes, in turn, through a process called gene expression, produce proteins that perform a vast array of functions within all living organisms. A mutation, or alteration, in the gene or in sequences that control the expression of that gene can cause proteins to be produced aberrantly in the cell – for example, too little or too much protein can be produced in the cell – which can cause disease. Through gene transfer, a functional copy of the mutated gene is delivered to the patient's cells, thereby correcting the underlying genetic defect that causes aberrant gene expression.

In the gene transfer process, a functional gene is delivered and incorporated into a patient's cells through a delivery system called a vector, which are most commonly based on naturally-occurring viruses that have been modified to take advantage of the virus' natural ability to introduce genes into cells. However, unlike naturally-occurring viruses, which replicate following infection of a target cell and have the capacity to infect new cells, viral vectors are modified to be non-replicating by deleting that portion of the viral genome responsible for replication. Gene transfer using a viral vector is called transduction and the resulting gene-modified cells are described as transduced cells. Transduction can be accomplished either via *ex vivo* or *in vivo* delivery. In the *ex vivo* approach, cells are gene-modified outside of the patient's body and the modified cells are transplanted back into the patient. In the *in vivo* approach, vectors are introduced directly into the patient's body to deliver the desired gene to the target cell.

A growing body of gene therapy-based clinical data, the establishment of regulatory guidelines to govern the development and approval of gene therapy products and increased investment from the biopharmaceutical industry suggest that the time is now for gene therapy to emerge as an important new therapeutic modality for patients with significant unmet medical need. We believe we are particularly well-positioned to drive the continued advancement of gene therapy technology for the treatment of severe genetic and orphan diseases. We have assembled extensive expertise in viral vector design, manufacturing and gene transfer, a broad intellectual property estate, an experienced management team and a world-class group of scientific advisors and key opinion leaders. We refer to our viral vector and gene transfer technology and know-how as our gene therapy platform.

We and our scientific collaborators have generated what we believe is human proof-of-concept data for our gene therapy platform in two underserved diseases, each of which has been granted orphan drug status by U.S. and European regulatory authorities. We expect to initiate in late 2013 a Phase II/III clinical study of our most advanced product candidate, Lenti-D, to evaluate

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its safety and efficacy in subjects with childhood cerebral adrenoleukodystrophy, or CCALD, a rare, hereditary neurological disorder affecting young boys that is often fatal. We also expect to initiate in the second or third quarter of 2013, or mid-2013, Phase I/II clinical studies in the United States and Europe of our next most advanced product candidate, LentiGlobin, to evaluate its safety and efficacy in subjects with  $\beta$ -thalassemia major and, in the European clinical study, sickle cell disease, or SCD, which are rare, hereditary blood disorders that often lead to severe anemia and shortened lifespans. We refer to the initiation of a clinical study as the time by which we have received all regulatory approvals necessary to commence a clinical study in accordance with a defined clinical protocol, we are under agreement with at least one clinical site to conduct the clinical study and we have begun to screen patients for enrollment in the clinical study. In addition, in March 2013, we announced a global strategic collaboration with Celgene Corporation to discover, develop and commercialize novel, disease-altering gene therapies in oncology.

Our gene therapy platform is based on viral vectors that utilize a modified, non-replicating version of the Human Immunodeficiency Virus Type 1, or HIV-1 virus, that has been stripped of all of the components required for it to self-replicate and infect additional cells. The HIV-1 virus is part of the lentivirus family of viruses, as a result of which we refer to our vectors as lentiviral vectors. Our lentiviral vectors are used to introduce a functional copy of a gene to the patient's own isolated blood stem cells, called hematopoietic stem cells, or HSCs, which reside in a patient's bone marrow and are capable of differentiating into a wide range of cell types. HSCs are dividing cells, thus our approach allows for sustained expression of the modified gene as we are able to take advantage of a lifetime of replication of the gene-modified HSCs. Additionally, we have developed a proprietary cell-based vector manufacturing process that is both reproducible and scalable. We believe our innovations in viral vector design and related manufacturing processes are important steps towards advancing the field of gene therapy and in realizing its full potential on a commercial scale, a concept we refer to as the industrialization of gene therapy.

Utilizing our industrialized gene therapy platform, we are developing product candidates comprising the patient's own gene-modified HSCs. Clinical proof-of-concept already exists for allogeneic hematopoietic stem cell transplant, or HSCT, an approach of treating a patient with HSCs contributed by a donor other than the patient that contain the properly functioning copy of the gene whose mutation has caused the underlying disease. However, this approach has significant limitations, including difficulties in finding appropriate genetically-matched donors and the risk of transplant-related rejection and mortality, and is therefore typically only offered on a limited basis. Our approach is intended to address the significant limitations of allogeneic HSCT while utilizing existing stem cell transplant infrastructure and processes. Also, because our approach has the potential to drive sustained expression of the functional protein encoded by the gene insert to provide a potentially single-administration, transformative therapy, we believe the value proposition offered by our product candidates for patients, families, providers and payors would be significant.

Although our initial focus is in CCALD,  $\beta$ -thalassemia and SCD, we believe our gene therapy platform has broad therapeutic potential in a variety of indications. We believe that our vectors can be used to introduce virtually any gene and have the potential to be manufactured on a commercial scale reproducibly and reliably, as each new vector is produced using substantially the same process. We also take advantage of lentivirus' ability to transduce HSCs more efficiently than other vectors, such as those derived from another virus used in gene therapy approaches, called adeno-associated virus, or AAV, which gives us the potential to address diseases in a variety of cell lineages that are derived from HSCs, such as microglia (useful for CCALD), red blood cells (useful for  $\beta$ -thalassemia and SCD), T cells (useful for cancer and immunology) and others.

## The potential of gene therapy to address severe genetic and orphan diseases

### *Gene therapy—the time is now*

Gene therapy has been an evolving field for the last 20 years that has been characterized by great hope and potential. Gene therapy is an approach to treating disease through the introduction of a desired gene or gene sequence into a patient's own cells to modulate or enhance the activity of such cells. Each person's hereditary genetic material is encoded by deoxyribonucleic acid, or DNA, in sequences of genetic code called genes. Collectively, our gene expression patterns influence cell functionality by controlling protein production, either directly or through other indirect regulatory mechanisms. A mutation, or alteration, in the gene or in sequences that control the expression of that gene can cause proteins to be produced aberrantly in the cell, which can cause disease.

Gene therapy represents a unique opportunity to change the way patients with severe genetic and orphan diseases are treated by addressing the underlying *cause* of their disease, rather than offering solutions that focus only on their *symptoms*. By correcting the underlying genetic defect, we believe gene therapy can provide transformative disease modifying effects—potentially with life-long clinical benefits based on a single therapeutic administration.

Our belief in the potential of gene therapy to become a viable therapeutic modality is supported by several recent developments, including the following:

- **Growing body of promising clinical results** . Over the last several years, a number of clinical studies of gene therapies have shown promising efficacy and safety results in conditions such as retinal disease, adrenoleukodystrophy, or ALD,  $\beta$ -thalassemia, chronic lymphoid leukemia, hemophilia and Parkinson's disease.
- **Significant design, manufacturing and process improvements** . In recent years, we and others have designed new viral vectors with improved safety profiles over earlier generation vectors. Improvements in viral vector manufacturing techniques have also enabled the production of more potent and efficient viral vectors on a commercially viable scale.
- **Growing support from regulators for gene therapy** . Although the U.S. Food and Drug Administration, or the FDA, has not yet approved any human gene therapy product for sale, it has provided guidance for the development of gene therapy products. For example, the FDA has established the Office of Cellular, Tissue and Gene Therapies, or OCTGT, within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee, or CTGTAC, to advise CBER on its reviews. In addition, the FDA has issued a growing body of clinical guidelines, chemical, manufacturing and control, or CMC, guidelines and other guidelines, all of which are intended to facilitate industry's development of gene therapy products.
- **First regulatory approval of a gene therapy product in the Western world** . In 2012, the European Medicines Agency, or EMA, approved a gene therapy product called Glybera, which is the first gene therapy product approved by regulatory authorities anywhere in the Western world.
- **Growing investment from the pharmaceutical and biotechnology industries** . Companies such as GlaxoSmithKline plc, Sanofi/Genzyme Corporation and BioMarin Pharmaceutical Inc. are currently advancing programs in gene therapy, and in 2012 Novartis AG

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announced a broad collaboration with the University of Pennsylvania to develop gene therapy products. In addition, Sanofi/Genzyme and Shire plc have made equity investments of \$8.0 million in the aggregate in our Company, and we have partnered with Celgene Corporation in the field of oncology.

- **Increased interest in genetic screening.** The growing market for both clinical and direct-to-consumer genetic testing and screening, including newborn screening initiatives for known hereditary diseases, points to increasing interest from patients and clinicians in therapeutic approaches that target specific genetic defects to treat disease.

Encouraged by these developments, we believe we are particularly well-positioned to drive the continued advancement of gene therapy technology in treating severe genetic and orphan diseases. We have assembled a leading position in the fields of gene therapy and severe genetic and orphan diseases, including extensive expertise in viral vector design, manufacturing and transduction, a broad intellectual property estate, an experienced management team and a world-class group of scientific advisors and key opinion leaders. Leveraging these capabilities, we have developed new, proprietary lentiviral vectors designed to more safely deliver our product candidates to patients, as well as improved transduction techniques to more efficiently effect gene transfer. We refer to our viral vector and transduction technology and know-how as our gene therapy platform. Our initial focus is on our two lead clinical programs in CCALD and  $\beta$ -thalassemia major. However, we believe our gene therapy platform has broad applicability in a variety of severe genetic and orphan diseases beyond these initial indications, which we intend to explore selectively, either alone or through partnerships, such as our recently-announced collaboration with Celgene in the field of oncology.

### **Our gene therapy platform and process**

Our gene therapy platform and product candidates are being developed based on a simple notion: *to genetically modify a patient's own cells to fundamentally correct or address the genetic basis underlying a disease*. Although the notion of gene transfer to a patient's own cells is simple, the processes of developing viral vectors capable of delivering the genetic material and inserting gene sequences safely into a patient's target cells is highly technical and demands significant expertise, experience and know-how. Leveraging our extensive expertise in viral vector design and manufacturing and transduction, we have developed a gene therapy platform that we believe is broadly applicable in a variety of indications of significant unmet medical need.

The historical challenges for gene therapy relate to the three factors on which the success of a gene therapy product is primarily based—potency, efficiency and safety. The potency of a particular gene therapy product is measured by its effectiveness, which is based on successfully introducing the gene of interest into the target cells at a high enough frequency to achieve expression of the desired protein at a level sufficient to exert a therapeutic benefit. The efficiency of a gene therapy product is measured by the amount of product that is required to create the desired effect, the period of time it takes for the therapy to go into effect, and also the period of time over which the therapy is effective for a given dosage. Safety is evaluated based on the nature and severity of any side effects, complications, conditions or diseases that may result from introducing foreign materials into a person's body and cells. Until recently, there has been a lack of manufacturing and transduction infrastructure that would enable the delivery of these therapies in a reliable and reproducible manner and at a commercially viable scale.

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However, over the last several years, we have focused on and made significant investments in developing improved, “next generation,” viral vectors and manufacturing processes and procedures to address each of these issues.

These improvements include the following:

- We have developed proprietary viral vectors with improved potency, efficiency and safety over those vectors used historically, which in some cases raised serious safety concerns.
- We have developed proprietary vector manufacturing processes and techniques that produce a more purified and concentrated end product, as evidenced by the approximately 25 to 30-fold reduction in non-infectious viral particles as compared to viral vectors used in previous clinical studies (both ours and of others).
- We are investing in the development of mid- to large-scale manufacturing systems designed to be both reproducible and sustainable, with a view towards supporting our product candidates, if approved, at commercial scale.

We refer to these improvements as the “industrialization” of gene therapy manufacturing and production. We believe these improvements and our continuing investment in our manufacturing platform will enable us to develop best-in-class, next generation gene therapy products for severe genetic and orphan diseases.

### ***Our proprietary lentiviral vectors***

The success of a gene therapy platform is highly dependent on the type of delivery system used. Our platform is based upon an *ex vivo* viral delivery system whereby a certain type of virus delivers the DNA that it is carrying into a cell and inserts this DNA into the cell's existing DNA. We have developed significant expertise in designing a particular type of vector delivery system employing a lentivirus for use in gene therapy and have also developed and in-licensed relevant intellectual property, including know-how, related to lentiviral vectors. Our lentiviral construct design includes only the minimal viral components of the HIV-1 virus required to enable the vector to undergo one round of replication within the cell during manufacturing and subsequently to enter the target cells and deliver the gene that it is carrying.

We believe that our lentiviral vectors are particularly well-suited for treating a number of diseases and have certain advantages over other viral vectors used in developing gene therapy products, including:

- **Sustained expression**—Unlike other viral vectors based on other viruses, such as AAV, lentiviral vectors are capable of integrating the functional gene they carry into the DNA of the target cell's chromosome. As such, they are well-suited to introduce a sustained therapeutic effect in dividing cells because the gene sequence introduced by the lentiviral vector will be replicated during cell division along with the rest of the cell's chromosomal DNA. Therefore, subsequent dividing cells resulting from the originally transduced cell will also carry the newly inserted gene sequence. The power of lentiviral vectors is sustained expression: a single insertion of a functional gene into a dividing cell can have a multiplying effect on multiple downstream cells. Other vector platforms that take advantage of different viruses introduce genes into cells but they don't integrate into a cell's DNA and thus require many viral events to transform a cell.

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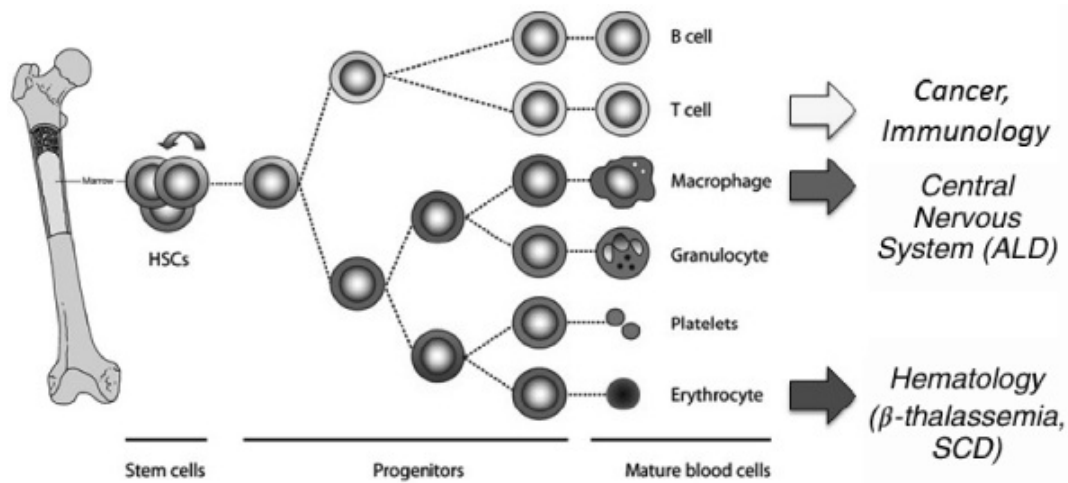
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- **Safety**—In clinical studies of gene therapy product candidates conducted by other entities, earlier generations of integrating viral vectors based on a mouse gamma-retrovirus were shown to preferentially integrate into certain regulatory regions of genes (such as the promoter regions) and in some instances inappropriately activate the cell to divide uncontrollably, leading to cancer through a process called insertional oncogenesis. These genetic alterations have led to several well-publicized adverse events, including several reported cases of leukemia, and highlighted the need to develop new gene therapy vectors with improved safety profiles. Next generation, lentiviral vectors, unlike gamma retroviruses, have a distinct pattern of integrating into regions that provide instructions for making proteins rather than preferentially integrating into regions that can lead to cell proliferation and cancer. We believe this difference in integration patterns is a critical factor in improving the safety profile of the vector, and distinguishes them from earlier generations of integrating viral vectors. This integration pattern difference has been published in several studies, showing that lentiviral vectors have demonstrated an improved safety profile over gamma-retrovirus vectors, with no known clinical events of insertional oncogenesis or cancer.
- **Carrying capacity**—Unlike AAV, the lentivirus is able to carry large therapeutic gene sequences (up to 8,000 base pairs) into a host cell. This may limit the utility of AAV in some diseases where the required gene sequences will be too large to fit into an AAV construct. In this regard, lentiviral vectors offer more flexibility.

***Our focus on Hematopoietic Stem Cells (HSCs)***

Our gene therapy platform takes advantage of lentiviral vectors' ability to stably integrate into the target cell's chromosome by focusing on diseases we can treat through genetic modification of hematopoietic stem cells, or HSCs, which when reintroduced back into the patient, differentiate into numerous other cell lineages, as depicted below. We believe our initial clinical indications —CCALD,  $\beta$ -thalassemia major and SCD—can all be treated by introducing a specific functional gene into HSCs taken from the patient to correct the gene defect responsible for the disease.

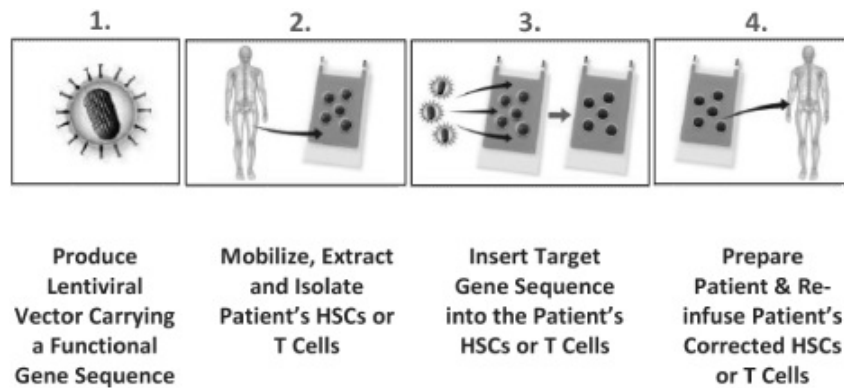




HSCs are dividing stem cells that are permanently found in a patient’s bone marrow and are an ongoing replacement source of mature cell types as they die off. HSCs produce progeny cells, called progenitors, that differentiate into all of the cellular elements that compose the blood, including microglia (useful for CCALD), red blood cells (useful for β-thalassemia and SCD), T cells (useful for cancer and immunology) and others. As such, all progenitors derived from a single gene therapy-modified HSC will carry the same corrective genetic modification, which we believe gives our approach the potential to deliver life-long clinical benefits based on a single therapeutic administration. We believe there are numerous diseases associated with genetic abnormalities in cell types derived from HSCs that we can target using our gene therapy platform.

**Our therapeutic approach**

The delivery of a gene therapy product requires several steps, as illustrated in the figure below. Importantly, our approach seeks to leverage cell transplant procedures and infrastructure already widely used in the clinic for allogeneic HSCT.



1. We produce our lentiviral vector by co-transfecting a packaging cell line with multiple plasmids that separately encode the various components of the virus as well as the

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functional gene sequence the viral vector will carry. The use of multiple plasmids is an important safety step designed to further prevent the resulting lentiviral vectors from being able to replicate and cause infection on their own.

2. A sample of the patient's own HSCs is extracted and isolated through a standard process known as apheresis, where HSCs are first mobilized into the blood stream from the bone marrow using a routinely-used pharmaceutical agent and then collected from the patient's blood. In some cases, HSCs are extracted directly from the patient's bone marrow.
3. The lentiviral vector is mixed with the patient's isolated HSCs *ex vivo*. This leads to the insertion of the functional gene into the HSCs' existing DNA, thus creating a pool of the patient's own, or autologous, gene-modified cells. The cells are then washed to remove any remnants of the viral vector or culture media. These gene-modified HSCs are the therapeutic drug product that is delivered back into the patient.
4. Prior to administering our drug product, the patient undergoes a standard myeloablation procedure (also used in allogeneic HCST) to remove all endogenous bone marrow cells. The modified HSCs are then re-infused back into the patient (approximately one to two months after initial extraction of the patient's HSCs) and begin re-populating a portion of the bone marrow as permanently modified HSCs in a process known as engraftment. The engrafted HSCs will go on to give rise to progenitor cell types with the corrected gene sequences. Following successful engraftment, we anticipate that clinical benefits for Lenti-D in CCALD, indicated by prevention of major functional disabilities, stabilization of NFS and Loes score and resolution of gadolinium enhancement, will begin to become evident within 24 months of transplant, and that clinical benefits for LentiGlobin in  $\beta$ -thalassemia and SCD, indicated by reduction or elimination of blood transfusion requirements, number of in-patient hospitalization days (post-transplant discharge) and, for SCD, several additional endpoints, will begin to become evident within 12-24 months of transplant.

We believe that our approach has several potential advantages over current treatment options for CCALD,  $\beta$ -thalassemia and SCD, including the following:

- **Single administration with potential life-long benefit** . Our process allows us to potentially arrest, correct or treat a disease with a single therapeutic administration as many of the corrected cells will live in the patient's body in perpetuity and have the potential to deliver long-term, and possibly life-long, effects.
- **We know exactly what gene to insert** . We are initially pursuing diseases where the genetic abnormality is known and is found in a single gene, known as monogenic diseases. We therefore know what we are correcting and exactly what gene sequence to insert into the patient's cells, thus mitigating against the uncertainty of the disease biology.
- **Allogeneic HSCT provides proof-of-concept for our approach** . We are currently pursuing clinical indications for which allogeneic HSCT is already a proven therapeutic option . Clinical proof-of-concept already exists for the diseases we are targeting via allogeneic HSCT, an approach of treating a patient with HSCs contributed by a donor other than the patient that contain the properly functioning copy of the gene whose mutation has caused the underlying disease.
- **We use the patient's own cells** . By using the patient's own isolated HSCs, we believe our approach will eliminate many of the challenges associated with allogeneic HSCT, such as

the limited availability of optimally matched donors and risks of transplant rejection that often result in serious adverse events, such as graft-versus-host disease. Even where allogeneic HSCT is deemed successful, many patients are required to comply with prolonged immunosuppressive drug regimens that increase the risk of opportunistic infections and other adverse events.

- **We modify our target cells *ex vivo*.** By inserting the new functional DNA into the cells *ex vivo*, we reduce the risk of adverse events and remove one of the key biological complexities of any therapeutic—getting a drug directly to the target cells.
- **Administration of our drug product is consistent with existing stem cell transplant practices.** The final step of our process, in which patients are myeloablated and then transfused with the finished drug product, is consistent with widely adopted stem cell transplant clinical practices and infrastructure already in use.
- **Value proposition to patients, families, providers and payors.** Given the potentially dramatic clinical and life-long benefits anticipated from such therapies delivered through potentially a single administration, we believe the value proposition for patients, families, providers and payors would be significant.

Put simply, we believe we have developed next generation vectors with improved potency, efficiency and safety using a reproducible, scalable manufacturing process to address a variety of severe genetic and orphan diseases.

## Our strategy

Our objective is to develop and commercialize a next generation of products based on the transformative potential of gene therapy to treat patients with severe genetic and orphan diseases. Central to this effort is a collective determination within our Company to provide these patients with hope for a better life in the face of limited or no long-term safe and effective treatment options. Specifically, our business strategy is based on the following principles:

- **Relentlessly focus on serving our patients.** Our culture is rooted in a shared motivation to bring the transformative potential of gene therapy to patients in need. Our initial focus is on patients suffering from monogenic diseases such as CCALD,  $\beta$ -thalassemia and SCD, as well as cancer; however, we believe there are many additional indications for which our technology may be applicable.
- **Be the world's biggest gene therapy geeks.** We believe our people and our culture (based on the principles: b colorful, b cooperative, b yourself) will continue to be fundamental to our success. We will continue to build a professional team of employees, advisors and collaborators with deep and industry-leading experience in the discovery, development, manufacturing and commercialization of gene therapy technologies to treat severe genetic and orphan diseases. We believe our expertise in this field—in terms of lentiviral vector design and gene therapy process industrialization—will allow us to continue developing next generation technologies that will overcome some of the challenges that have historically complicated the use of gene therapy on a broader scale and allow for deployment in many underserved severe genetic and orphan disease markets. We will continue our efforts, which over the last several years have resulted in the production of early clinical proof-of-concept results in two diseases, the industrialization of the gene therapy process and the generation of significant intellectual property.

- **Leverage our platform and technical expertise to build a gene therapy product engine for severe genetic and orphan diseases.** We will continue to take advantage of the adaptability of our gene therapy platform in creating viral vectors and gene therapy products to address a broad range of genetically-defined diseases. Unlike other gene modification approaches that may require extensive optimization for each gene target or disease indication, each of our lentiviral vectors is produced using the same modified vector backbone and manufacturing system. This enables us to generate new product candidates relatively quickly by essentially swapping in the new gene of interest and assessing its potency and purity using standardized assays and tests. We believe our specific ability to design and manufacture lentiviral vectors quickly and reproducibly on a commercial scale will differentiate us from other gene therapy technologies and provides a strong competitive advantage in the long term.
- **Develop and commercialize drugs in our core disease areas and partner selectively to expand the scope of our pipeline.** Our core disease areas are severe genetic and orphan diseases, such as CCALD and  $\beta$ -thalassemia, that we believe to be good candidates for treatment with gene therapy. Given the relatively low prevalence of these diseases and the strong key opinion leader communities and patient advocacy groups around them, we believe we can serve these markets with a small targeted commercial infrastructure. The broad potential of our platform also presents an opportunity for us to selectively form collaborative alliances to expand our capabilities and product offerings into other therapeutic areas and potentially accelerate the development and commercialization of our products. For example, we recently announced a global strategic collaboration with Celgene to discover, develop and commercialize novel, disease-altering gene therapies in oncology.
- **Pursue indications with high unmet medical need and greater probability of clinical, regulatory and commercial success.** Each of our three current core indications are severe diseases with high unmet medical need. We believe there is a strong rationale for treating diseases like these with gene therapy because their underlying genetic abnormality is well-characterized and can be addressed by correcting or inserting a single gene. Given the poor prognosis and current lack of corrective treatment options for these diseases, we believe our gene therapy product candidates may offer a potential single-treatment alternative for these patients and their families. Our gene therapy products, if successful, may offer a potentially superior long-term value proposition for our patients and the healthcare system more broadly, which will allow us to drive premium value while delivering patients life-altering treatments.

## Our product candidate pipeline

The following table summarizes key information on our development programs.

Product/ Territories	Program Area	Preclinical	Phase I/II	Phase II/III	Status
Lenti-D Worldwide	CNS Diseases				
	Childhood Cerebral ALD - ALD-102 Study*				• IND Active • Initiate Late 2013
	Adult Cerebral ALD				
LentiGlobin® Worldwide	Hematologic Diseases				
	B-Thalassemia/SCD (France) - HGB-205 Study**				• CTA Active • Initiate Mid-2013
	B-Thalassemia (U.S.) - HGB-204 Study**				• IND Active • Initiate Mid-2013
CAR T Cells Global Celgene Collaboration	Oncology				
	Hematologic Malignancies				
	Solid Tumors				

\* The Phase II/III ALD-102 Study is our first clinical study of our current Lenti-D viral vector and product candidate. See “Business—Our Lenti-D product candidate.”

\*\* The Phase I/II HGB-205 and HGB-204 Studies are our first clinical studies of our current LentiGlobin viral vector and product candidate. See “Business—Our LentiGlobin product candidate.”

Our most advanced product candidate is called Lenti-D, which we are developing to treat patients with ALD. We plan to initiate a Phase II/III clinical study of Lenti-D in the United States in late 2013, which we refer to as the ALD-102 Study, to examine the feasibility, safety and efficacy of Lenti-D in preserving neurological function and stabilizing cerebral demyelination in subjects with CCALD, the most severe form of ALD. We also expect to initiate sites outside the United States, pending approvals from the applicable regulatory authorities. If successful, and pending further discussion with the FDA, the results from the ALD-102 Study could potentially form the basis of a Biologics License Application, or BLA, submission to the FDA and a Marketing Authorization Application, or MAA, to the EMA for this product candidate. However, there can be no assurance that the FDA and the EMA will not require additional studies before the approval of a BLA or MAA, respectively. Initial proof-of-concept data from a clinical study utilizing an approach similar to Lenti-D with an earlier generation lentiviral vector supplied by a third party were published in *Science* (2009).

Our next most advanced product candidate is called LentiGlobin, which we are developing to treat patients with  $\beta$ -thalassemia and SCD. We are currently conducting a Phase I/II clinical study in France evaluating an earlier generation of our LentiGlobin vector for the treatment of  $\beta$ -thalassemia major and SCD. Initial proof-of-concept data from this study were published in *Nature* (2010). We plan to initiate an extension of this study under a revised protocol for LentiGlobin, which we refer to as the HGB-205 Study, in mid-2013. We also plan to initiate a second Phase I/II clinical program in the United States for LentiGlobin, which we refer to as the HGB-204 Study, for  $\beta$ -thalassemia major in mid-2013.

In March 2013, we announced a global strategic collaboration with Celgene Corporation to discover, develop and commercialize novel disease-altering gene therapies in oncology. The collaboration will focus on applying gene therapy technology to genetically modify a patient's own T cells to target and destroy cancer cells. Such modified T cells, called chimeric antigen receptor, or CAR, T cells have been shown to have beneficial effects in human clinical trials for patients with B cell lymphomas. The multi-year research and development collaboration has the potential to lead to the development and commercialization of multiple CAR T cell products. See “—Our strategic alliance with Celgene.”

## **Our Lenti-D opportunity**

### ***Adrenoleukodystrophy***

Adrenoleukodystrophy is a rare X-linked, inherited, neurological disorder that is often fatal. ALD is caused by mutations in the ABCD1 gene which encodes for a protein called the ALD protein, or ALDP, which plays a critical role in the breakdown and metabolism of very long-chain fatty acids, or VLCFA. Without functional ALDP, VLCFA accumulate in cells including neural cells in which they cause damage to the myelin sheath, a protective and insulating membrane that surrounds nerve cells in the brain. This damage can result in decreased motor coordination and function, visual and hearing disturbances, the loss of cognitive function, dementia, seizures, adrenal dysfunction and other complications, including death. The incidence rate for ALD is approximately one in 20,000 newborn males, and the U.S. National Institute of Health, or NIH, estimates a prevalence of one in 20,000.

ALD is divided into various sub-segments with three main phenotypes that impact brain function:

- **CCALD (Childhood cerebral adrenoleukodystrophy)**: The most severe form of ALD is CCALD. CCALD accounts for about 30-40% of patients diagnosed with ALD and presents in young boys. CCALD is characterized by progressive destruction of myelin, leading to severe loss of neurological function and eventual death. In boys affected by CCALD, learning and behavioral problems are often observed in mid-childhood between the ages of 3 and 15 years (median age 7). In the absence of intervention, boys affected by CCALD typically experience rapid degeneration into vegetative state, and ultimately death within a decade of diagnosis.
- **AMN (Adrenomyeloneuropathy)**: AMN which typically develops in adults aged 21 years and older, is the most common neurological form of ALD, accounting for 40-45% of all patients diagnosed with ALD. All patients with AMN present with more slowly progressive symptoms resulting from (non-inflammatory) disruption of the axons (which are a fundamental component of the central nervous system that allows nerve signals to be transmitted) in the spinal cord. Approximately 40% of these patients have or will develop cerebral disease similar to CCALD, with varying degrees of associated inflammation.
- **ACALD (Adult Cerebral ALD)**: ACALD typically develops in males aged 15 years and older. It is also very severe, with progression of neurologic symptoms that parallels the course of CCALD. ACALD accounts for approximately 5% of all patients diagnosed with ALD.

### ***Limitations of current treatment options***

There is a clear unmet medical need for patients with the neurologic forms of ALD. Currently, the only effective treatment option for boys with CCALD is allogeneic HSCT. In this procedure, the patient is treated with HSCs containing the properly functioning copy of the gene

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contributed by a donor other than the patient. Allogeneic HSCT has also been shown to have potential clinical benefit in other forms of ALD including AMN and ACALD.

Allogeneic HSCT is typically performed early in the course of the disease, ideally using an unaffected matched sibling HSC donor to minimize complications. However, the majority of allogeneic HSCT procedures for CCALD are carried out with non-sibling matched donor cells, partially matched related or unrelated donor cells and umbilical cord blood cells because a matched sibling donor is not available in most cases. The difficulty of finding a suitable sibling-matched donor is one of the primary drawbacks of this approach. Allogeneic HSCT is associated with significant morbidity and mortality, particularly in patients who undergo non-sibling-matched allogeneic HSCT. Complications of allogeneic HSCT include a 10-30% risk of engraftment failure in unrelated Human-Leukocyte-Antigen, or HLA, matched patients, a 12-16% incidence of life-threatening infection, and an approximately 30% risk of graft-versus-host-disease, or GVHD, a common complication in which donor immune cells (white blood cells in the graft) recognize the cells of the recipient (the host) as “foreign” and attack them. As a result of these safety challenges, allogeneic HSCT in CCALD patients whose donor is not a matched sibling result in significant mortality rates. In addition, because of the need for long-term immunosuppression following allogeneic HSCT, there is a prolonged risk of opportunistic infections and other serious side effects associated with immunosuppressive drugs.

Moreover, of the approximately 80 boys who are born with CCALD each year in the United States and European Union, we estimate that between 20% and 50% may have disease so advanced at the time of diagnosis that a beneficial outcome from treatment would be unlikely. This is attributed to rapid disease progression and difficulty with early diagnosis, as the initial presentation of the signs and symptoms of CCALD are frequently misdiagnosed, for example as attention deficit hyperactivity deficit disorder. Newborn screening through a simple and inexpensive blood test is being developed to enable earlier detection of CCALD, but is not yet widely available. Based in part on the fact that several states are currently considering universal newborn screening for ALD, it is our expectation that newborn screening will be widely adopted in the United States within the next five years, and potentially elsewhere, providing for the opportunity to identify more boys for proactive monitoring of disease symptoms and early disease intervention.

## **Our Lenti-D product candidate**

We are developing our Lenti-D product candidate as a potential one-time treatment to halt the progression of CCALD. Our approach involves the *ex vivo* insertion of a functional copy of the ABCD1 gene via an HIV-1 based lentiviral vector into the patient's own HSCs to correct the aberrant expression of ALDP in patients with CCALD. HSCs derived from the patient's own body are called autologous HSCs. We refer to autologous HSCs that have been modified to carry the functional copy of the ABCD1 gene as the final Lenti-D drug product, or our Lenti-D product candidate. Upon successful engraftment of our Lenti-D product candidate, we expect that microglia in the brain derived from the transduced HSCs will correct the metabolic abnormalities resulting from excess VLCFA and stabilize the demyelination and cerebral inflammation characteristic of CCALD.

We have had and continue to have extensive dialogue with the FDA, the EMA and other regulatory authorities and advisory bodies concerning the clinical advancement of our Lenti-D product candidate. These interactions include the following:

- our Lenti-D product candidate has been granted orphan drug status by the FDA and the EMA for the treatment of CCALD;

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- in 2010, the NIH's Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC, reviewed our draft protocol and its recommendations were incorporated into the final protocol and informed consent;
- a type B pre-IND meeting with the FDA in 2010, during which meeting the FDA recommended we initiate a retrospective natural history of disease study to inform future clinical studies and provide guidance on the manufacturing, nonclinical and clinical development of our Lenti-D product candidate;
- receipt of Scientific Advice regarding the design of the planned ALD-102 Study from the French agence nationale de sécurité du médicament et des produits de santé, or ANSM, in February 2011, from the EMA in May 2011, and from the United Kingdom Medicines and Healthcare Products Regulatory Agency, or MHRA, in May 2012;
- a type C pre-IND meeting with the FDA in 2012, focused on the design of the planned ALD-102 Study;
- an agreed Pediatric Investigation Plan, or PIP, with the EMA Pediatric Committee, or PDCO, in March 2013; and
- an IND submission for our ALD-102 Study in March 2013, which IND is active as of April 2013.

We expect to initiate the ALD-102 Study in the United States in late 2013. We also expect to initiate sites outside the United States, pending approvals from the applicable regulatory authorities. If successful, and pending further discussion with the regulatory authorities, the results from the ALD-102 Study could potentially form the basis of a BLA submission to the FDA and an MAA to the EMA for this product candidate. However, there can be no assurance that the FDA and the EMA will not require additional studies before the approval of a BLA or MAA, respectively. The FDA has advised us that the ALD-102 Study may not be deemed to be a pivotal study or may not provide sufficient support for a BLA submission. The FDA normally requires two pivotal clinical studies to approve a drug or biologic product, and thus the FDA may require that we conduct additional clinical studies of Lenti-D prior to a BLA submission.

## **Clinical development of our Lenti-D product candidate**

### ***Completed non-interventional retrospective study (the ALD-101 Study)***

Due to the rarity of CCALD, and the fact that allogeneic HSCT has historically not been subject to extensive systematic analysis in controlled clinical studies, the amount of clinical data necessary to precisely characterize progression of the disease and the efficacy and safety profile of allogeneic HSCT is largely absent from the current scientific literature. Accordingly, in order to properly design future clinical studies of Lenti-D and interpret the efficacy and safety results thereof, at the recommendation of the FDA, we performed a non-interventional retrospective data collection study to assess the natural course of disease in CCALD patients that were left untreated, which we refer to as the untreated group or cohort, in comparison to the efficacy and safety data obtained from patients that received allogeneic HSCT, which we refer to as the treated cohort. A non-interventional retrospective data collection study involves an examination of historical clinical records from patients with the pertinent condition in order to assess the typical course of the condition and the efficacy and safety of treatment options. In the study, we collected neurologic



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and neuropsychological assessments and neuroimaging data for both treated and untreated patients, as available; however, given the retrospective nature of the study, we were not able to collect comprehensive data for all subjects.

For this study, we collected data from four U.S. sites and one French site on a total of 137 subjects, 72 of whom were untreated and 65 of whom were treated with allogeneic HSCT. To our knowledge, the ALD-101 Study is the most comprehensive study ever conducted to characterize clinical outcomes in untreated versus allogeneic HSCT-treated CCALD patient populations. The ALD-101 Study report was completed in March 2013.

*Three primary clinical measurements of CCALD disease progression*

The findings from the ALD-101 Study suggest that, although there are a wide number of cognitive, behavioral, functional and radiological modalities utilized to assess patients with CCALD, three are utilized most widely and consistently:

- **The Neurological Function Score (NFS)**. The NFS is a 25-point neurological function score that assesses fifteen neurological abnormalities typically caused by ALD. These neurological abnormalities are summarized below:

Symptoms	Score
Loss of communication*	3
No voluntary movement*	3
Cortical blindness*	2
Tube feeding*	2
Wheelchair required*	2
Total incontinence*	2
Swallowing/other CNS dysfunctions	2
Spastic gait (needs assistance)	2
Hearing/auditory processing problems	1
Aphasia/apraxia	1
Visual impairment/fields cut	1
Running difficulties/hyperreflexia	1
Walking difficulties/spasticity/spastic gait (no assistance)	1
Episodes of incontinency	1
Nonfebrile seizures	1
<b>Total</b>	<b>25</b>

\* Major Functional Disabilities (MFDs)

Among the 15 functional domains in the NFS scale, we consider six to be of particular clinical importance because when these neurological abnormalities occur, the patient's ability to function independently is severely compromised. These particular deficiencies, which we define as Major Functional Disabilities, or MFDs, are loss of communication, complete loss of voluntary movement, cortical blindness, requirement for tube feeding, wheelchair dependence and total incontinence.

- **The Loes score**. The Loes score is a 34-point scale specifically designed to objectively measure the extent of central nervous system disease burden based on brain magnetic resonance imaging, or MRI, studies. The Loes score measures the extent and location of brain abnormalities such as the presence of white matter changes, degree of demyelination and the presence of focal or global atrophy. A Loes score of one or more

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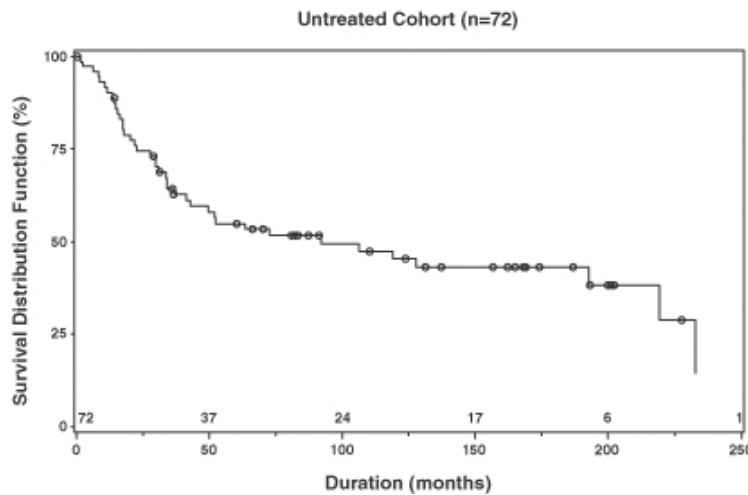
(i.e., the presence of any such abnormalities) indicates significant disease, and patients with a Loes score of 10 or more generally are not considered to be good candidates for transplant therapy due to the advanced stage of the disease.

- **Gadolinium enhancement.** One of the hallmarks of inflammatory disease in ALD patients is the presence of a compromised blood-brain barrier behind the leading edge of demyelinating lesions in the brain. This can be assessed using a contrast agent called gadolinium in brain MRI studies. Evidence of gadolinium enhancement in the brain in a MRI study, referred to by clinicians as a gadolinium positive result, suggests that neuroinflammation is present and the blood-brain barrier has been compromised, which in published studies has been shown to be a predictive biomarker of ALD disease progression.

*Summary of findings*

Key findings from the ALD-101 Study are summarized below:

- **Untreated, CCALD patients progress to dismal outcomes .** In the untreated cohort, the median overall survival was 92 months (7.7 years) and the estimated probability of survival at five years was 55%. Although informative, survival data must be considered in light of the fact that supportive measures may be used to sustain life after progression to a vegetative state.



- **Baseline disease severity, as assessed by NFS and Loes scores, were good predictors of survival .** In both the untreated and treated cohorts, significantly lower mortality rates were seen in patients with lower baseline NFS and Loes scores than in those with higher scores.

	Mortality Rate*			
	NFS ≤ 1	NFS > 1	Loes ≥ 1 ≤ 9	Loes > 9
Untreated Cohort	42%	85%	46%	76%
Treated Cohort	12%	29%	13%	28%

\* Mortality rate determined by the number of deaths that occurred at any time through the observation period post-CCALD diagnosis.

As a consequence of this observation, and consistent with entry criteria that have been used in studies of allogeneic HSCT, the entry criteria for the ALD-102 Study excludes subjects with evidence of advanced disease on NFS and Loes score to prevent enrollment of subjects whose disease would be expected to progress to a poor outcome despite treatment.

- **MFDs occurred in the majority of the untreated cohort who showed evidence of gadolinium enhancement in brain MRI.** Among the 72 patients in the untreated cohort, data were available regarding the presence of MFDs at 24 months post-CCALD diagnosis in 56 of these patients. Among these 56 patients, 29 patients (52%) developed at least one MFD throughout the data collection period. Of the 18 cases in the untreated cohort who were gadolinium positive, 13 (72%) had developed at least one MFD at 24 months from the time of their first gadolinium positive scan. We believe the finding that a large proportion of the untreated cohort with gadolinium enhancement progress to an MFD at 24 months provides an important reference point by which to assess the success of treatment with our Lenti-D product candidate. These observations support the requirement that subjects enrolled in the ALD-102 Study demonstrate gadolinium enhancement at baseline and support a primary endpoint based on the prevention of MFDs.
- **Gadolinium enhancement appears to be an objective, predictive measure of the likelihood of rapid progression.** In the untreated cohort, of the 15 patients with scans that were gadolinium-positive and had repeat NFS assessments during the applicable observation period, most (12 of the 15 patients) showed rapid progression of NFS scores, defined as an increase of greater than five points over the applicable observation period, with all 12 showing decline within six to 18 months. This observation supports the requirement that subjects enrolled in the ALD-102 Study demonstrate gadolinium enhancement at baseline. These patients would be expected to develop progressive disease without therapeutic intervention.
- **Allogeneic HSCT was associated with disease stabilization.** Despite the significant risk of morbidity and mortality associated with allogeneic HSCT, successful transplantation was shown to provide clinically meaningful benefit to patients with CCALD, particularly those with early-stage disease. For the majority of patients in the treated cohort (63%), no MFD was present at 24 months post-HSCT. Allogeneic HSCT was also associated with resolution of gadolinium enhancement. Of those patients who would meet eligibility criteria for the ALD-102 study (baseline NFS of zero or one, gadolinium-positive at baseline, baseline Loes between 0.5 and nine, inclusive), three of 20 (15%) patients developed an MFD within 24 months post-allogeneic HSCT.
- **Consistent with published literature, allogeneic HSCT, particularly with unmatched/unrelated donors, was associated with clinically significant morbidity and mortality.**
  - **Morbidity:** Post-allogeneic HSCT, engraftment failure occurred in 12 of 65 (18%) patients, 10 of whom (83%) were transplanted with unrelated donor cells. Despite prophylaxis, the GVHD rate was 54%, including acute GVHD in 27 (42%) patients and chronic GVHD in 12 (18%) patients. Due to the requirement for myeloablation prior to HSCT, the occurrence of GVHD and the requirement for immunosuppressive therapy post-allogeneic HSCT, allogeneic HSCT is associated with a substantial risk of life-threatening infection. Infections were the most commonly reported serious adverse

event, with at least one serious infection reported in 19 (29%) patients post-allogeneic HSCT. The substantial morbidity associated with allogeneic HSCT for CCALD supports evaluating Lenti-D in the ALD-102 Study as an alternative therapeutic option that is expected to avoid the issues of immune incompatibility seen with allogeneic HSCT.

- **Mortality:** Post-allogeneic HSCT, the 100-day mortality rate was 8% and the overall one-year mortality rate was 19%. The estimated probability of two and five year survival rates post-allogeneic HSCT were 82% and 74%, respectively. As anticipated from the published literature, analysis of survival by type of donor (matched sibling versus other) showed that the proportion of deaths through the observation period post- allogeneic HSCT was lower in matched-sibling donor cases than in other allogeneic HSCT cases. The majority of allogeneic HSCT patients (46 patients; 71%) were transplanted with unrelated donor cells given the limited availability of HLA-matched sibling donors. As a result of this analysis, we determined to exclude patients with a sibling-matched donor from the ALD-102 Study.

We believe the results from the ALD-101 Study support the proposition that, while the approach of treating a patient with genetically corrected HSCs can stabilize the progression of disease in patients with CCALD, there remains a significant unmet medical need for safer therapies, particularly for patients without the option of a sibling-matched donor. We believe that many of the issues that contribute to the mortality and morbidity associated with allogeneic HSCT could be avoided using a patient's own gene-modified HSCs. Importantly, the results from this study were also used to inform the criteria for patient and endpoint selection for our planned ALD-102 Study, which we describe below.

#### ***Previous clinical experience with lentiviral gene therapy for CCALD (the TG04.06.01 Study)***

Between September 2006 and September 2010, four boys with a confirmed diagnosis of CCALD were treated in Paris, France, in a Phase I/II study with autologous HSCs transduced *ex vivo* with a lentiviral vector carrying a functional ABCD1 gene before reinfusion. Short-term clinical data and biological experience with the first two treated boys was first reported in *Science* (2009). The study is ongoing although no new subjects are expected to be enrolled beyond the initial four boys.

The TG04.06.01 Study is sponsored by the institut national de la santé et de la recherche médicale (French Institute of Health and Medical Research), or Inserm, in Paris, and the lentiviral vector was supplied by a third party company not affiliated with bluebird bio. We are party to a strategic collaboration agreement with Inserm for the development of HSC gene therapies in this patient population, pursuant to which we are collaborating with Patrick Aubourg, the Principal Investigator of the TG04.06.01 Study.

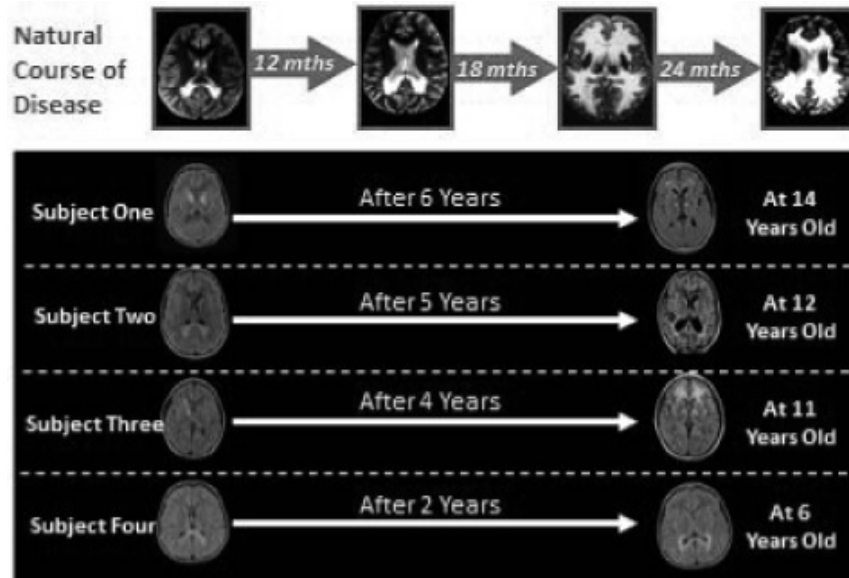
In the TG04.06.01 Study, all four subjects had cerebral demyelinating lesions with Loes scores ranging from two to seven prior to treatment. Gadolinium contrast enhancement indicated that the lesions were active and inflammatory in all four subjects. At the time of enrollment, each subject had a normal neurologic examination with NFS equal to zero.

Below is a summary of the efficacy results for each of the four subjects in the TG04.06.01 Study.

- **Subject One:** Loes score stabilized at month 30 and remained stable through month 75.

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- **Subject Two:** Loes score stabilized at month 30 and remained stable through month 64. Gadolinium enhancement was initially positive, resolved, reappeared in the parietal area and then resolved and has remained negative.
- **Subject Three:** Loes score stabilized at month 33 but gadolinium enhancement has persisted. Subject Three had active, progressive disease post-transplant resulting in the development of significant cognitive deficits with the loss of ability for new learning consistent with a frontal lobe syndrome, including the loss of spontaneous speech by month 33 and urinary incontinence. As of 54 months post-transplant, he had no further decline in NFS or Loes scores since his month 33 evaluation.
- **Subject Four:** Loes score stabilized at month 16 and remained stable at 24 months. Gadolinium enhancement disappeared 45 days post-transplant and was still not detectable at month 12.



At the top of the figure is a series of brain MRI images showing an example of progressive white matter disease in an untreated patient with CCALD. The expanding "white" in the images from left to right illustrates increasing demyelination in the brain and represents severe disease. The images below represent the baseline (left) and recent (right) brain MRI images from the four boys treated in the TG04.06.01 Study. In contrast to the extensive progressive white matter disease that might be seen in untreated CCALD, as shown at the top of the figure, the progression of white matter disease following treatment in the TG04.06.01 Study is more limited.

We believe these efficacy results are consistent with outcomes that would be expected following successful allogeneic HSCT. All four boys are alive two years or more after treatment, while the ALD-101 Study would suggest an expected mortality rate of approximately 20% in the same two-year window post-allogeneic HSCT. As assessed by NFS and brain MRI, Subjects One, Two and Four have shown encouraging evidence of disease stabilization. Additionally, gadolinium enhancement resolved in Subjects One, Two and Four, suggesting a reduction of neuroinflammation. These results also contrast with the natural history of disease in untreated patients, which is characterized by continuous and rapid progression of cerebral demyelination in the majority of cases, particularly those with gadolinium enhancement on brain MRI. All four subjects demonstrated some deterioration of neurologic function within the second year after

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transplant, which is expected as it is also frequently seen following allogeneic HSCT, given the time it takes for transplant-derived microglial cells to populate the brain. Although neurologic deficits have occurred in these subjects post-treatment, we are encouraged by the fact that neurologic disease has stabilized in all four subjects.

Importantly, there have also been no reported incidents of gene therapy-related safety concerns in the TG04.06.01 Study. The infusion procedure was clinically uneventful for all four subjects, with all achieving successful engraftment within 15 days post-transplant. In addition, none of these subjects experienced adverse events due to immune incompatibility issues typically associated with allogeneic HSCT, such as graft rejection or GVHD.

We believe the efficacy and safety results of the TG04.06.01 Study provide clinical proof-of-concept, as the lentiviral vector used in the study shares many features with our Lenti-D vector. In addition, the results of the TG04.06.01 Study were helpful in informing the design of our future ALD-102 Study. The design of the ALD-102 Study is built upon the observations made in the TG04.06.01 Study, but will enroll a larger number of subjects, is a multi-center, international trial with a different primary endpoint determined by analysis of the ALD-101 Study data and in consultation with experts in the field, and has a predefined criterion for clinical success. Additionally, with improvements we have introduced into the vector manufacturing and transduction processes, we expect to obtain a higher frequency of gene-modified HSCs in subjects treated in the ALD-102 Study compared to what was achieved in the TG04.06.01 Study, which we believe will translate into improved clinical benefit by virtue of the increased expression of normally-functioning ALDP.

**Phase II/III clinical study (the ALD-102 Study)**

In April 2013, the FDA informed us that the IND we filed in March 2013 with the FDA for a Phase II/III clinical study to examine the feasibility, safety and efficacy of our Lenti-D product candidate is now active. We refer to this study as the ALD-102 Study. The study is designed as a single-dose, open-label, non-randomized, international, multi-center Phase II/III study to test the safety and efficacy of our Lenti-D product candidate in preserving neurological function and stabilizing cerebral demyelination in subjects with CCALD. Subjects will be followed for 24 months post-transplant under this protocol. Per the *FDA Guidance for Industry: Gene Therapy for Clinical Trials – Observing Subjects for Delayed Adverse Events*, we will be monitoring study subjects in a long-term follow up protocol to evaluate safety for up to 15 years, and will also monitor efficacy endpoints to demonstrate a sustained treatment effect.

Our clinical trial recruitment plans involve a multi-faceted approach, including:

- clinical site community outreach programs;
- global patient referral and support programs to bring patients from across the world to existing clinical sites;
- gene therapy patient, family and physician education tools, including general gene therapy and ALD-specific websites and materials;
- ALD patient advocacy engagement and support; and
- continued publication of existing and future scientific and clinical ALD data.

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Up to 15 subjects will be enrolled in the study to obtain at least 12 evaluable subjects that have been transplanted with the Lenti-D drug product. In the study, subjects must be age fifteen years or younger with a confirmed diagnosis of active CCALD, including elevated levels of plasma VLCFA, a brain MRI Loes score of 0.5 to nine, inclusive, evidence of gadolinium enhancement and an NFS  $\leq$  one. Subjects with a willing matched sibling HSCT donor will be excluded from the study. We expect to initiate the ALD-102 Study in late 2013.

Based on results from our retrospective ALD-101 Study and consultation with leading clinicians in the field of ALD, we have defined the primary efficacy endpoint in the ALD-102 Study as the proportion of subjects who have no MFDs, as measured by NFS, at 24 months ( $\pm$ two months) post-transplant. Secondary efficacy evaluations, in each case measured at 24 months ( $\pm$ two months) post-transplant, capture the key assessments of CCALD disease status, including the change from baseline in NFS and Loes score, resolution of gadolinium enhancement on MRI and determination of MFD-free survival and overall survival. The sample size for this study was not determined by formal statistical methods, but we believe it may be sufficient to demonstrate a robust effect on the binary response endpoint, where a responder is defined as a subject with no MFD at 24 months ( $\pm$ two months) following transplant. Thus, we expect the FDA will make a qualitative assessment of the efficacy and safety data from this study to evaluate whether the results are sufficient to support a BLA.

Safety evaluations will be performed during the study and will include evaluation of the following: success and kinetics of HSC engraftment; incidence of transplant-related mortality through 100 and 180 days post-transplant; detection of vector-derived replication of the HIV-1 virus; and characterization and quantification of events related to the location of insertion of the functional gene in target cells.

If successful, we believe that the results from the ALD-102 Study would form the basis of a BLA and an MAA. However, given the number of subjects and design of the study and the qualitative/subjective assessment of the data, there can be no assurance the FDA will not require one or more additional clinical studies as a precursor to a BLA application. The FDA has advised us that the ALD-102 Study may not be deemed to be a pivotal study or may not provide sufficient support for a BLA submission. The FDA normally requires two pivotal clinical studies to approve a drug or biologic product, and thus the FDA may require that we conduct additional clinical studies of Lenti-D prior to a BLA submission.

***Preclinical evaluation of our Lenti-D product candidate***

We have completed a single-dose toxicology study of our Lenti-D product candidate in immunodeficient mice following a single intravenous administration. This study investigated the engraftment of normal human HSCs transduced with our Lenti-D vector and the reversibility of any toxicity following a 28 and 91 day post-treatment recovery period. The assessment of toxicity was based on mortality, clinical observations, body and organ weights, and anatomic pathology. In addition, engraftment of the HSCs was analyzed in the bone marrow of all the interim and main sacrifice animals by fluorescence-activated cell sorting and by polymerase chain reaction procedures.

Study results from the single dose toxicology study found no product candidate-related effects in body and organ weight, hematology or clinical chemistry parameters. In addition, histopathological evaluation revealed that there were no product candidate-related microscopic findings. There were no significant group differences (aside from slight individual animal

variation) in cellularity of the bone marrow in treated control and test animals, as determined by light microscopy. Based upon the evaluation criteria used for the study, the Lenti-D drug product appeared to be well tolerated after single intravenous injection.

### ***Additional potential clinical indications for Lenti-D***

The ACALD and AMN subsets of the broader ALD patient population represent potential additional opportunities for our Lenti-D product candidate. Allogeneic HSCT has shown some early reported success in ACALD patients, suggesting autologous gene therapy with our Lenti-D product candidate may also be used to address these patients. AMN represents a population of heterogeneous patients with about 40% presenting with cerebral symptoms, however no known allogeneic HSCT studies have been conducted in the AMN population to provide evidence for a gene therapy based approach in the treatment of this disease. The risk-reward balance and safety risks associated with allogeneic HSCT have limited its use in treating ACALD and AMN patients, which may provide an opportunity to expand the use of our Lenti-D gene therapy product in these indications to increase interest in gene therapy for the treatment of other forms of ALD.

## **Our LentiGlobin opportunity**

### ***$\beta$ -thalassemia***

#### *Overview*

$\beta$ -thalassemia is a rare hereditary blood disorder caused by a genetic abnormality of the  $\beta$ -globin gene resulting in defective red blood cells, or RBCs. Genetic mutations cause the absence or reduced production of the beta chains of hemoglobin, or  $\beta$ -globin, thereby preventing the proper formation of hemoglobin A, which normally accounts for greater than 95% of the hemoglobin in the blood of adults. Hemoglobin is an iron-containing protein in the blood that carries oxygen from the respiratory organs to the rest of the body. Hemoglobin A consists of four chains—two chains each of  $\alpha$ -globin and  $\beta$ -globin. Normally existing at an approximate 1:1 ratio, genetic mutations that impair the production of  $\beta$ -globin can lead to a relative excess of  $\alpha$ -globin, leading to premature death of red blood cells. The clinical implications of the  $\alpha$ -globin/ $\beta$ -globin imbalance are two-fold: first, patients lack sufficient RBCs and hemoglobin to effectively transport oxygen throughout the body and can become severely anemic; and second, the shortened life span and ineffective production of RBCs can lead to other complications such as splenomegaly, marrow expansion, bone deformities, and iron overload in major organs.

The clinical course of  $\beta$ -thalassemia correlates with the degree of globin chain imbalance. Nearly 200 different mutations have been described in patients with  $\beta$ -thalassemia. Symptoms of  $\beta$ -thalassemia can include severe anemia, splenomegaly, marrow expansion, bone deformities and iron overload in major organs. The clinical presentation varies widely, dependent largely upon the number and type of inherited mutation. Mutations can be categorized as those which result in little or no functional  $\beta$ -globin production ( $\beta^0$ ) and those which result in decreased functional  $\beta$ -globin production ( $\beta^+$ ).  $\beta$ -thalassemia major refers to any mutation pairing that results in the need for chronic transfusions due to severe anemia, and is the clinical finding in patients with  $\beta^0\beta^0$  genotype as well as many with the  $\beta^0\beta^+$  genotype. Affected patients produce as little as one to seven g/dL of hemoglobin (while a normal adult produces 12-18 g/dL of hemoglobin). Hemoglobin E, which is another  $\beta$ -globin mutation and is usually asymptomatic, can also result in  $\beta$ -thalassemia major when paired with the  $\beta^0$  or  $\beta^+$  mutations.



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$\beta$ -thalassemia is concentrated in populations of Mediterranean, South and Southeast Asian and Middle Eastern descent. The total annual incidence of symptomatic individuals is estimated at one in 100,000 throughout the world and one in 10,000 people in the European Union. Due to the rarity of this disease in the United States, published research on the prevalence of  $\beta$ -thalassemia in the United States is limited, although it is estimated that due to changing immigration patterns, 1.8 in 100,000 births in California are affected by  $\beta$ -thalassemia. This data is derived from a mandatory screening program for hemoglobinopathies in that state.

### *Limitations of current treatment options*

In geographies where treatment is available, patients with  $\beta$ -thalassemia major receive chronic blood transfusion regimens aimed at maintaining steady state hemoglobin levels of approximately 9-10 g/dL. These regimens consist of infusions with units of pRBC every three to five weeks, the timing of which is based predominantly on monitoring hemoglobin levels. Chronic blood transfusions can be effective at preventing the hallmark symptoms of childhood  $\beta$ -thalassemia major, however, often lead to a large iron overload, which over time leads to mortality through iron-associated heart and liver toxicity. To prevent iron overload-associated risks, patients must adhere to therapeutic iron chelation regimens to reduce the iron overload. Poor compliance with chelation regimens remains a key challenge; it is estimated that with typical compliance, the overall life expectancy for a patient with transfusion-dependent  $\beta$ -thalassemia is only 28 years. Even patients who are compliant with transfusion and iron chelation regimens can experience a reduced quality of life due to the burden of therapy and the fluctuating levels of hemoglobin on a month-to-month basis.

The only potentially curative therapy for  $\beta$ -thalassemia today is allogeneic HSCT. However, because of the significant risk of transplant-related morbidity and mortality, transplants are offered primarily to pediatric patients with a matched sibling donor, which occurs in less than 25% of all cases. Allogeneic HSCT carries a significant risk of morbidity and mortality related to myeloablation (which decreases or eliminates the cells in the bone marrow and blood), immunosuppressive medications, graft failure, GVHD and opportunistic infections. Overall,  $\beta$ -thalassemia major remains a devastating disease with an unmet medical need.

In many developing countries where  $\beta$ -thalassemia is more prevalent, such as Thailand, the lack of readily available chronic blood transfusions and optimal iron chelation regimens represents a significant societal challenge. In these countries, children with  $\beta$ -thalassemia major have a poor prognosis and experience growth retardation, hepatosplenomegaly, or enlargement of the spleen, and skeletal deformities resulting from extra-medullary hematopoiesis. Ultimately, most die in childhood. We believe that safer therapies, such as those represented by our gene therapy approach, could offer a potential solution to the challenges of treating  $\beta$ -thalassemia patients across the world.

## **Sickle cell disease**

### *Overview*

Sickle cell disease, or SCD, is a hereditary blood disorder resulting from a mutation in the  $\beta$ -globin gene that causes polymerization of hemoglobin proteins and abnormal red blood cell function. The disease is characterized by anemia, vaso-occlusive pain crisis (a common complication of SCD in which there is severe pain due to obstructed blood flow in the bones, joints, lungs, liver, spleen, kidney, eye, or central nervous system), infections, stroke, overall poor quality of life and

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early death in a large subset of patients. Under low-oxygen conditions, which are exacerbated by the red blood cell abnormalities, the mutant hemoglobin aggregates causing the RBCs to take on a sickle shape (sickle cells), which causes them to aggregate and obstruct small blood vessels, thereby restricting blood flow to organs resulting in pain, cell death and organ damage. If oxygen levels are restored, the hemoglobin can disaggregate and the RBCs will return to their normal shape, but over time, the sickling damages the cell membrane and the cells fail to return to the normal shape even in high-oxygen conditions. Additionally, the sickle-shaped RBCs tend to rupture more easily, often resulting in damage to the blood vessels and iron overload that can ultimately lead to organ failure and death.

SCD is concentrated in populations of African, Middle Eastern and South Asian descent. The global incidence of SCD is estimated to be 250,000-300,000 births annually, and the global prevalence of the disease is estimated to be about 20-25 million. In the United States, where SCD is a standard part of mandatory newborn screening, the incidence is more than 1,600 births annually with an estimated prevalence of 100,000 individuals.

#### *Limitations of current treatment options*

Where adequate medical care is available, common treatments for patients with SCD include chronic blood transfusions and hydroxyurea. As is the case with  $\beta$ -thalassemia, chronic transfusions pose a compliance burden and are associated with significant risks that often leads to mortality through iron-associated heart and liver toxicity. Patients must also adhere to daily iron chelation regimens. A significant number of patients with SCD find it difficult to adhere to hydroxyurea treatment regimens due in part to drug-related toxicities.

The only potentially curative therapy currently available for SCD is allogeneic HSCT, however because of the significant risk of transplant-related morbidity and mortality, this option is usually offered primarily to pediatric patients with available sibling-matched donors. It is particularly difficult to find suitable donors for individuals of African descent, and it is estimated that approximately 10% of eligible patients do so. In light of these factors, we believe SCD is a devastating disease with a significant unmet medical need.

### **Our LentiGlobin product candidate**

We are developing our LentiGlobin product candidate as a potential one-time treatment for both  $\beta$ -thalassemia and SCD. Our approach involves the *ex vivo* insertion of a single codon variant of the normal  $\beta$ -globin gene via an HIV-1 based lentiviral vector into the patient's own HSCs to enable formation of normally functioning hemoglobin A and normal RBCs in patients with  $\beta$ -thalassemia or SCD. Importantly, this codon variant, referred to as T87Q, also serves as a distinct biomarker used to quantify expression levels of the functional  $\beta$ -globin protein in patients with  $\beta$ -thalassemia and SCD, while also providing strong anti-sickling properties in the context of SCD. We refer to the gene-modified HSCs as the final LentiGlobin drug product, or our LentiGlobin product candidate.

We have had and continue to have a comprehensive dialogue with the FDA, the EMA and other regulatory authorities and advisory bodies concerning the clinical advancement of our LentiGlobin product candidate. These interactions include the following:

- our LentiGlobin product candidate has been granted orphan drug designation by the FDA and the EMA and Fast Track status by the FDA;

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- in 2012, the NIH's RAC reviewed our draft protocol and its recommendations were incorporated into the final protocol and informed consent;
- a type B pre-IND meeting with the FDA in 2012 focused on the design of our planned HGB-204 Study and provided guidance on the manufacturing and nonclinical development with a view towards a future IND filing;
- an IND submission for our HGB-204 Study in December 2012, which IND is effective as of January 2013;
- a meeting with ANSM in November 2011 regarding the submission of a Clinical Trial Application, or CTA, with a revised clinical protocol to support the use of our current LentiGlobin vector in our planned HGB-204 Study, and confirming that no additional *in vivo* toxicology data would be required for the CTA submission; and
- submission and approval of the CTA for the HGB-205 Study in 2012.

We expect to initiate our HGB-204 and HGB-205 clinical studies in mid-2013.

## Clinical development of our LentiGlobin product candidate

### ***Previous clinical experience with lentiviral gene therapy for $\beta$ -thalassemia major (the LG001 Study)***

Between September 2006 and November 2011, three subjects with  $\beta$ -thalassemia major were treated in France by our scientific collaborators in a Phase I/II study with autologous HSCs transduced *ex vivo* with an earlier generation of our LentiGlobin vector, called HPV569. We refer to the HSCs transduced *ex vivo* with the HPV569 vector as the HPV569 drug product. Clinical data and biological experience with one subject in this study (Subject Three) were first reported in *Nature* (2010).

Four subjects were enrolled in the LG001 Study, although only three subjects were actually treated with the HPV569 drug product—Subject One was ineligible due to pre-transplant complications. The other three subjects were successfully transplanted, however Subject Two received a dose of HPV569 drug product with cell counts well below current standards in transplant practice and failed to engraft. All subjects enrolled in the study required significant transfusion support prior to treatment. Below is a summary of the results for the two subjects with successful engraftment:

- **Subject Three:** During the first year post-transplant, Subject Three experienced a decline in both the volume and frequency of transfusion requirements and eventually became transfusion-independent approximately one year post-treatment. Subject Three has remained transfusion-independent ever since (more than four years), even in light of regular blood withdrawals to eliminate iron accumulation in the body. Adverse events considered to be treatment related were all attributable to study procedures or myeloablative conditioning, but not the HPV569 drug product. One notable observation was the detection of partial clonal dominance of a common myeloid progenitor bearing an integrated vector in the third intron of the HMGA2 gene, which resulted in a relatively large proportion of the gene therapy modified cells being derived from a single clone in which the lentiviral vector had inserted into the HMGA2 gene. There was some initial concern that the observed clonal dominance might represent a pre-leukemic event, however there have been no adverse clinical consequences of this event, or any signs of

cancer, in over five years since the observation was made. In fact, the presence of the HMGA2 clone has steadily declined over time to the point that it is no longer the most common clone.

- **Subject Four:** After transplant, Subject Four experienced delayed recovery of platelets and required platelet transfusion thrice weekly until day 100, with the last transfusion on day 122. Therapeutic hemoglobin in reticulocytes was detectable by one month post-transplant. At two- and six-months post-transplant, therapeutic hemoglobin was expressed in 4.0% and 3.1% of reticulocytes, respectively. Subject Four is clinically stable, has fully engrafted and feels well. However, transfusion requirements remain unchanged at approximately monthly intervals with T87Q corrected globin stably expressed at levels substantially below those demonstrated by Subject Three at similar time points. Further follow-up is required to establish the complete trajectory of T87Q globin production and vector copy number. Adverse events considered to be treatment related were all attributable to study procedures or myeloablative conditioning, but not the HPV569 drug product.

We believe that achieving transfusion independence in Subject Three is a direct benefit of treatment with the HPV569 drug product, as we are not aware of any reported cases of spontaneous transfusion independence in patients with  $\beta$ -thalassemia major. While successful allogeneic HSCT may achieve transfusion independence, the mortality risk of allogeneic HSCT in adults with  $\beta$ -thalassemia major exceeds 20%, and for that reason it is not a standard therapeutic intervention for adult patients. The approach of using autologous gene-modified HSCs avoids the adverse consequences of immune incompatibility that are responsible for much of the mortality and morbidity associated with allogeneic HSCT.

We believe the efficacy and safety results of the LG001 Study provide clinical proof-of-concept, as the lentiviral vector used in the study shares many features with our current LentiGlobin vector. In addition, the results of the LG001 Study were helpful in informing the design of our HGB-205 and HGB-204 clinical studies. Additionally, with improvements we have introduced into the vector manufacturing and transduction processes, we expect to obtain a higher frequency of gene-therapy modified HSCs in the patients treated in the HGB-205 and HGB-204 clinical studies compared to what was achieved in the LG001 Study, which we believe will translate into improved clinical efficacy and in improved clinical benefit by virtue of increased production of normally functioning hemoglobin.

#### ***Phase I/II clinical study for $\beta$ -thalassemia major and sickle cell disease (the HGB-205 Study)***

At the request of ANSM, in 2012 we submitted a CTA with a revised clinical protocol for the LG001 Study as a result of our decision to use our newer LentiGlobin BB305 vector for our clinical studies going forward. A preclinical evaluation of LentiGlobin BB305 showed that transduction efficiency was higher with the LentiGlobin BB305 vector as compared to the HPV569 vector used in the LG001 Study, resulting in higher expression of the therapeutic  $\beta$ -globin protein in transduced cells, despite unchanged expression levels per vector copy. The CTA was accepted in 2012, resulting in an active study, now called the HGB-205 study, which we expect to initiate in France in mid-2013. This continuation study is a Phase I/II clinical study to examine the safety and efficacy of our LentiGlobin product candidate in up to seven additional subjects with a diagnosis of  $\beta$ -thalassemia major or SCD. Study subjects must be between five and 35 years of age with a diagnosis of  $\beta$ -thalassemia major or SCD. Those with  $\beta$ -thalassemia must have received at least 100 mL/kg/year of pRBCs per year for the past two years. Those with SCD must have failed to

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achieve clinical benefit from treatment with hydroxyurea and have an additional poor prognostic risk factor (e.g., recurrent veno-occlusive crises or acute chest syndromes). All subjects must be eligible for allogeneic HSCT, but without a matched related donor. Subjects with a matched sibling allogeneic HSCT donor will be excluded from the study.

Our clinical trial recruitment plans for the HGB-205 Study involve a multi-faceted approach, including:

- clinical site community outreach programs;
- global patient referral and support programs to bring patients to existing clinical sites;
- clinical site expansion in areas of high epidemiology;
- gene therapy patient, family and physician education tools, including general gene therapy and  $\beta$ -thalassemia and SCD specific websites and materials;
- $\beta$ -thalassemia and SCD patient advocacy engagement; and
- support and continued publication of existing and future  $\beta$ -thalassemia and SCD scientific and clinical data.

For all subjects, efficacy will be measured by RBC transfusion requirements per month and per year, post-transplant and the number of total in-patient hospitalization days (post-transplant discharge) at six, 12 and 24 months. For SCD patients only, efficacy will be measured by the number of vaso-occlusive crises or acute chest syndrome events at six, 12 and 24 months and evaluation of changes in the nature or frequency of the subject-specific main inclusion criteria.

Safety evaluations to be performed during the study include success and kinetics of HSC engraftment, incidence of transplant-related mortality post-treatment, overall survival, detection of vector-derived replication-competent lentivirus in any subject and characterization of events of insertional mutagenesis leading to clonal dominance or leukemia.

***Phase I/II clinical study for  $\beta$ -thalassemia major (the HGB-204 Study)***

In December 2012, we submitted an IND with the FDA for a Phase I/II clinical study to examine the feasibility, safety and efficacy of our LentiGlobin product candidate. We refer to this study as the HGB-204 Study. The study is a single-dose, open-label, non-randomized, multi-site Phase I/II clinical study in the United States to evaluate the safety and efficacy of the LentiGlobin product candidate in increasing hemoglobin production and eliminating or reducing transfusion dependence following treatment. In January 2013, we were cleared to commence the study and we expect to initiate this study in mid-2013.

Our clinical trial recruitment plans for the HGB-204 Study involve a multi-faceted approach, including:

- clinical site community outreach programs;
- global patient referral and support programs to bring patients to existing clinical sites;
- clinical site expansion in areas of high epidemiology;
- gene therapy patient, family and physician education tools, including general gene therapy and  $\beta$ -thalassemia specific websites and materials;

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- $\beta$ -thalassemia patient advocacy engagement; and
- support and continued publication of existing and future  $\beta$ -thalassemia scientific and clinical data.

Up to 15 adults will be enrolled in the study. Study subjects must be between 18 and 35 years of age with a diagnosis of  $\beta$ -thalassemia major and who receive at least 100 mL/kg/year of pRBCs or greater than or equal to eight transfusions of pRBCs per year in each of the two years preceding enrollment. The subjects must also be eligible for allogeneic HSCT.

Efficacy will be evaluated primarily by the production of  $\geq 2.0$  g/dL of hemoglobin A containing  $\beta^{A-T87Q}$ -globin for the six-month period between 18 and 24 months post-transplant. In order to allow for endogenous hemoglobin production following transplant, subjects will be transfused with RBCs only when total hemoglobin decreases below 7.0 g/dL. The rationale for the primary endpoint is that production of  $\geq 2.0$  g/dL of hemoglobin A containing  $\beta^{A-T87Q}$ -globin represents a clinically meaningful increase in endogenous hemoglobin production that would be expected to diminish transfusion requirements, and could result in transfusion independence in  $\beta$ -thalassemia subjects.

Exploratory efficacy endpoints include RBC transfusion requirements (measured in milliliters per kilogram) per month and per year, post-transplant. Safety evaluations to be performed during the study include success and kinetics of HSC engraftment, incidence of transplant-related mortality post-treatment, overall survival, detection of vector-derived replication-competent lentivirus in any subject and characterization of events of insertional mutagenesis leading to clonal dominance or leukemia. Subjects will be monitored by regular screening. Each subject will remain on study for approximately 26 months from time of consent and then will be enrolled in a long-term follow-up protocol that will assess safety and efficacy beyond 24 months.

### **Preclinical evaluation of our LentiGlobin product candidate**

Several nonclinical studies have been performed to support the use of our LentiGlobin BB305 vector. These studies were conducted in human HSCs isolated from patients with SCD and in *in vivo* mouse transplant models. In these studies, transduction efficiency was shown to be higher with the LentiGlobin BB305 vector as compared to the HPV569 vector, based on higher expression levels of the therapeutic  $\beta$ -globin protein in cells transduced with this vector despite unchanged protein expression levels per vector copy. *In vivo* pharmacology and safety studies carried out in a mouse model for  $\beta$ -thalassemia provided no evidence that our lentiviral vectors caused any adverse effects or alteration of bone marrow homeostasis in animals treated with cells transduced with either the HPV569 or BB305 vector. In two independent *in vitro* immortalization, or IVIM, assays, LentiGlobin BB305 vector showed a reduced risk of IVIM and genotoxicity in murine HSCs as compared to positive control vectors known to have significant oncogenic potential. Results of integration site analyses in mice treated with syngeneic bone marrow cells transduced with either LentiGlobin BB305 or HPV569 vectors revealed no signs for clonal outgrowth. The integration site profile of the two vectors was comparable and typical for HIV-1 based lentiviral vectors. Both vectors showed a large overlap of integration sites in identical common integration site regions. Although integration near oncogenes was, in general, increased in the analyzed vector samples compared to the theoretical random integration site data, there was no increase of integration sites near oncogenes in the post-transplant samples isolated from the bone marrow at necropsy compared to pre-transplant samples of transduced bone marrow.

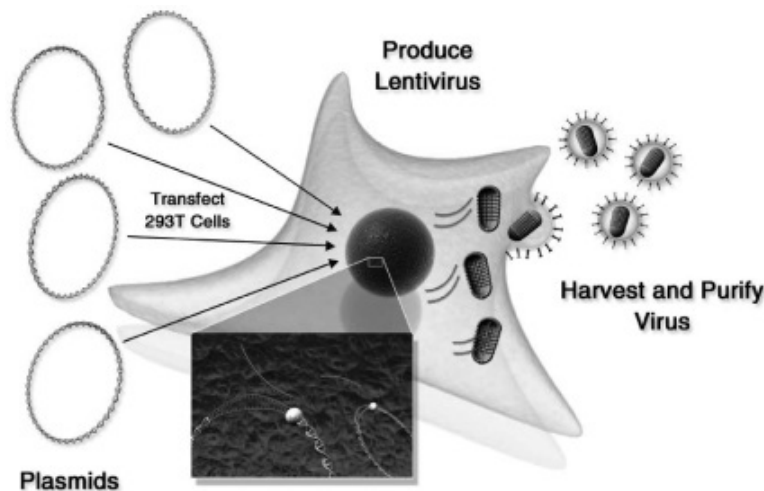
### **Previous preclinical experience with lentiviral gene therapy for sickle cell disease**

In 2001, a preclinical proof-of-concept study, led by our scientific founder Dr. Philippe Leboulch and scientists at Harvard Medical School and the Massachusetts Institute of Technology, corrected sickle cell disease in mice using gene therapy. In the study, mice were bioengineered to contain a human gene that produced defective hemoglobin, causing SCD. HSCs containing the defective gene were removed from the bioengineered mice and gene-modified by the addition of an anti-sickling gene using a lentiviral vector. The modified gene (T87Q) produced  $\beta$ -globin that gave rise to a modified normal hemoglobin molecule that prevented the sickling process. This gene construct is the same construct we use in our LentiGlobin product candidate. After adding the anti-sickling gene, the corrected marrow was then transplanted into other mice with SCD whose bone marrow had been removed by radiation. Ten months later, blood samples from the transplanted mice showed a high level of expression of the anti-sickling  $\beta$ -hemoglobin gene. The results from this preclinical proof-of-concept study for SCD were published in *Science* (2001).

### **Manufacturing**

Our gene therapy platform has two main components: lentiviral vector production and the target cell transduction process, which results in our finished drug product.

#### **Our lentiviral manufacturing process**



Our lentiviral vectors are assembled using a human cell line called HEK293T. The HEK293T cells are maintained in disposable flasks until sufficient cell mass has been generated to fill approximately 40 ten tray cell factories, or TTCFs, then transferred and allowed to adhere to the bottom of the trays. Adherent cells are transfected with multiple plasmids encoding all the genetic material required to assemble the lentiviral vector carrying such functional gene of interest. The genetic material is delivered on multiple plasmids to reduce the odds of generating a replication-competent virus and improve the overall safety of this step of the procedure. The transfected HEK293T cells then assemble our lentiviral vectors packaged with the functional gene of interest, which bud off into the cell culture media. The media containing the assembled vectors is harvested, purified by a single chromatography step, concentrated and formulated

prior to freezing for storage. These finished lentiviral vectors are what is ultimately used to transduce the HSCs isolated from the patient.

We believe that our lentiviral vectors have broad applicability, since the majority of the viral production system can remain the same, while we change only the therapeutic gene “cassette” depending on the disease. In other words, the vector “backbone” stays the same, while only the therapeutic gene and related sequences are changed. If we were to undertake drug development in an additional indication, we believe we could rapidly move forward using this lentiviral vector backbone and associated assays, simply by switching the therapeutic gene insert and associated control elements.

Although we intend to continue manufacturing our Lenti-D vectors in TTCFs, we are currently in the process of adapting our LentiGlobin vector production technology to a larger, suspension-based bioreactor process with the potential to scale from 100 to upwards of 1,000 liters in a single production run. So far, we have demonstrated successful production of LentiGlobin vectors on a small scale and are currently transferring the new process to a contract manufacturer in compliance with Good Manufacturing Practices, or GMP, to accommodate future demand for our drug candidates, if approved, in their current indications as well as those beyond our initial focus.

***Our target cell transduction process—creating the gene-modified cells (our drug product)***

The ultimate product of our manufacturing processes is the patient’s own gene-modified cells, which we refer to as our drug product. The process for producing our drug product is as follows:



1. **Selection:** We extract HSCs from peripheral blood mononuclear cells obtained from the patient’s blood by apheresis (or alternatively, by bone marrow harvest) following mobilization via a colony stimulating factor. The process is carried out using existing hospital infrastructure and standard protocols currently in place for stem cell transplant procedures.
2. **Pre-stimulation:** The isolated HSCs are treated with a mixture of growth factors and additional proprietary processes that help enable an efficient transduction process.
3. **Transduction:** The isolated, purified and pre-treated HSCs are exposed to our lentiviral vectors containing the appropriate functional gene for up to 40 hours to facilitate transduction and insertion of the therapeutic DNA into the chromosomes of the target cells.
4. **Final harvest:** Once transduction is complete, the gene-modified HSCs are washed and re-suspended into cell culture media to remove any residual impurities. A portion of the harvested cells is removed for quality control release testing, which includes ensuring that transduction was successful and the functional gene delivered by the vector is adequately expressed by the target cells.
5. **Formulation and freeze:** The remaining cells are appropriately formulated and cryopreserved.



The final step is to return the gene-modified HSCs to the patient. Just prior to dosing, the drug product is thawed and sampled for cell number and viability to ensure the dose administered meets a pre-defined minimum.

Of note, our proprietary lentiviral vector manufacturing and HSC transduction processes utilize operations and equipment that are common to the biopharmaceutical industry. We rely exclusively on the use of contract manufacturing organizations to manufacture our Lenti-D and LentiGlobin vectors and drug product candidates, and do not own or operate any of our own facilities for these purposes. However, we believe our team of technical personnel has extensive manufacturing, analytical and quality experience as well as strong project management discipline to effectively oversee these contract manufacturing and testing activities, and to compile manufacturing and quality information for our regulatory submissions.

## Future applications and opportunities

The investments that we have made to industrialize our gene therapy platform, processes and manufacturing may have application to other severe genetic and orphan diseases. We believe that we have the opportunity to pursue other disease indications that would take advantage of our know-how and other intellectual property, and expertise in three main areas:

- **Other lentiviral *ex vivo* applications:** We believe our current gene therapy platform will enable us to develop and test new vectors based on similar viral vector backbones that carry different gene sequences for other hereditary diseases without the need for significant research work. In this way, we can move products rapidly through preclinical into clinical development. We may consider research and development programs targeting other monogenic, hereditary diseases that involve cells derived from HSCs. These programs may involve hereditary orphan diseases that could be developed and potentially commercialized on our own.

We also plan to pursue gene therapy programs that target other cell types, such as T cells, that leverage the unique properties of lentiviral vectors. Through our global partnership with Celgene, we are now developing gene therapy products by inserting novel gene sequences into a patient's own T cells using lentiviral vectors for oncology. This represents a direct application of our expertise in gene therapy and our capabilities, know-how and patents associated with lentiviral gene therapy for *ex vivo* applications. As we further develop this program, we will investigate the opportunity to expand the application to T cells and other cell types for new potential indications.

- **Lentiviral *in vivo* applications:** Our expertise in lentiviral vector production and cell transduction also provides an opportunity to develop new lentiviral products for use in the *in vivo* setting. In this case, lentiviral vectors carrying certain gene sequences would be delivered directly to the disease site (e.g., to the brain or eye) or into the bloodstream of the patient and, in each case, the vector would need to find the target cell *in vivo* and deliver the genetic material into those target cells. Although this represents a less controlled environment in which to transduce cells and deliver genetic material, it opens up additional orphan and large market indications where this approach is more appropriate for the disease and targeted cells.
- **Adeno-associated viral (AAV) vector platform targeting other diseases:** Our team has extensive historic experience with AAV research and development programs. There is extensive evidence in the scientific literature supporting the use of these vectors for *in vivo*

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applications. The unique properties of AAV vectors may offer advantages in some indications where lentiviral vectors might be less suited. For example, AAV vectors may be better suited for use in products delivered *in vivo* systematically. Our experience and know-how could be useful with an AAV platform in these additional disease settings and we expect to explore cautiously and opportunistically AAV product candidates that could provide a bolt-on platform and capability for us.

The graphic below represents an example of the breadth of potential applications of our gene therapy platform.

LENTIVIRAL PLATFORM				AAV PLATFORM		
Central Nervous System	Hematology	Oncology	Immunology	Hemophilia B	Ocular	Central Nervous System
ALD	β-thalassemia	Hematologic Tumors				
Lysosomal Storage Disorders	Sickle Cell Disease	Solid Tumors				
Other Central Nervous System	Hemophilia A					

**Strategic collaborations**

Our objective is to develop and commercialize a next generation of products based on the transformative potential of gene therapy to treat patients with severe genetic and orphan diseases. To access the substantial funding and other resources required to develop and commercialize gene therapy products, we have formed, and intend to seek other opportunities to form, strategic alliances with collaborators who can augment our industry leading gene therapy expertise. To date, we have focused on forging a limited number of significant strategic alliances with leading pharmaceutical partners and academic laboratories where both parties contribute expertise to enable the discovery and development of potential gene therapy product candidates.

**Our strategic alliance with Celgene**

In March 2013, we announced a strategic collaboration with Celgene Corporation to discover, develop and commercialize novel disease-altering gene therapies in oncology. The collaboration will focus on applying gene therapy technology to genetically modify a patient’s own T cells, to target and destroy cancer cells. Such modified T cells, which are called chimeric antigen receptor, or CAR, T cells, have been shown to have beneficial effects in human clinical trials for patients with B cell lymphomas. The multi-year research and development collaboration has the potential to lead to the development and commercialization of multiple CAR T cell products.

Under the terms of the collaboration, for any product candidate selected for development under the collaboration, we will be responsible for conducting and funding all research and

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development activities performed up through completion of the initial Phase I clinical study, if any, of such product candidate. This collaboration will be governed by a joint steering committee, or JSC, formed by representatives from us and Celgene. The JSC will, among other activities, review the collaboration program, review and evaluate product candidates and approve regulatory plans.

On a product candidate-by-product candidate basis, up through a specified period following completion of an initial Phase I clinical study for such product candidate, we have granted Celgene an option to obtain an exclusive worldwide license to develop and commercialize such product candidate pursuant to a written agreement, the form of which we have already agreed upon. If Celgene elects to exercise this option, it must pay us an option fee, subject to reduction if we elect to co-develop and co-promote that product candidate in the United States. In addition to the option fee, Celgene would also be obligated to pay us additional amounts based upon achievement of specified development and regulatory milestones and a percentage of net sales as a royalty, however, if we elect to co-develop and co-promote in the United States, this royalty only applies to sales outside the United States. The maximum option fee payable to us under these agreements, together with the maximum additional payments payable to us upon achievement of specified development and regulatory milestones, is \$225 million, and the royalties payable to us range from the mid-single digits to mid-teens. The royalties payable to us are subject to certain reductions, including for any royalty payments required to be made by Celgene to acquire patent rights, with an aggregate minimum floor. Celgene will assume certain development obligations and must report on their progress in achieving these milestones on a quarterly basis. If we do elect to co-develop and co-promote the product candidate within the United States, we would share equally in all costs relating to developing, commercializing and manufacturing the product candidate within the United States and we would share equally in the United States profits.

Celgene will be solely responsible for all costs and expenses of manufacturing and supplying any optioned product candidates. Subject to customary "back-up" supply rights granted to Celgene, we have the sole right to manufacture or have manufactured supplies of vectors and associated payloads manufactured for incorporation into the optioned product candidate. We would do so under a written agreement, the form of which has not yet been agreed upon, although we have agreed upon certain material terms for such manufacturing and supply agreement. Celgene would reimburse us for our costs to manufacture and supply such vectors and associated payloads, plus a modest mark-up.

If Celgene does not exercise its option with respect to any product candidate prior to expiration of the applicable option period, then we have the right to develop that product candidate outside the scope of the collaboration, subject to a Celgene opt-in right to obtain a license to that product candidate, which right exists through completion of a pivotal study for that product candidate.

We received an up-front payment of \$75.0 million from Celgene in connection with the collaboration. The collaboration term ends in March 2016, unless extended at Celgene's option. Celgene may elect to extend the term twice, first for a period of two years and then for an additional period, in each case in consideration of a specified payment to us. Either party may terminate the agreement upon written notice to the other party in the event of the other party's uncured material breach. Celgene may terminate the agreement for any reason upon prior written notice to us. If the agreement is terminated, rights to product candidates in development at the time of such termination will be allocated to the parties through a mechanism included in the agreement. In addition, if Celgene terminates the agreement for our breach, any then-existing co-development and co-promotion agreement will be automatically terminated and

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replaced with a license agreement for such product candidate and any amounts payable by Celgene under any then-existing product license agreements will be reduced.

*Baylor College of Medicine*

Simultaneous with entering into the collaboration agreement with us, Celgene entered into a strategic collaboration with the Baylor College of Medicine, or Baylor, to discover, develop and commercialize CAR T cell products. We are not a party to this collaboration agreement, although, by virtue of our agreements with Celgene, the joint steering committee under the Baylor-Celgene collaboration agreement will include representatives selected by us, together with representatives selected by each of Celgene and Baylor. Under our collaboration agreement with Celgene, we may develop product candidates covered by the intellectual property rights of Baylor in this field, which intellectual property rights would be in-licensed by Celgene pursuant to its collaboration agreement with Baylor.

*Call Option and Target Antigen License*

Our agreement with Celgene provides that, effective upon completion of this offering, during the initial three-year term of the collaboration and, if extended, during the first two-year extension term of the collaboration, in the event that we engage in a change in control transaction, including for such purposes a merger or consolidation of bluebird bio or the sale of all or substantially all of our assets, or if another person or entity or group of persons or entities acquires at least 50% of our voting capital stock, then Celgene has the right, but not the obligation, to terminate the collaboration agreement and obtain perpetual, non-terminable, worldwide, exclusive, fully paid-up licenses to all, but not less than all, of the product candidates previously identified under the collaboration agreement. We refer to this right to acquire such licenses as the call option.

Under the call option, the product candidates to which Celgene would have the right to acquire fully paid-up licenses include any product candidate previously licensed out of the collaboration during the term of the collaboration, any product candidate for which we have exercised our right to co-develop and co-promote the product candidate within the United States, any product candidate for which Celgene previously declined its option to obtain a license and any product candidate for which at least *in vivo* efficacy studies have been initiated or authorized by the JSC. The purchase price for such fully paid-up licenses would be determined pursuant to a binding arbitration process and would be paid on or about the consummation of the change in control transaction with our acquiror.

In addition, during the initial three-year term of the collaboration, but not during any extension of the collaboration agreement, in the event that we engage in a change in control transaction described above and Celgene exercises the call option described above, then, in addition to the right to acquire the fully paid-up licenses described above, Celgene would also have the right to obtain a perpetual, non-terminable, worldwide, exclusive license to our intellectual property to develop one or more CAR T cell products targeting one or more oncology associated target antigens identified by Celgene following the third anniversary of the collaboration agreement. There is no limit to the number of oncology associated target antigens Celgene may select under this license. Upon commercialization of any such product candidate so licensed by Celgene, Celgene would be obligated to pay us a specified milestone payment upon regulatory approval and a percentage of net sales as a royalty. We refer to this license agreement to develop one or more CAR T cell products targeting one or more oncology associated target antigens as the target antigen license.

The call option and the right to acquire a target antigen license may have the effect of delaying or preventing a change in control transaction involving us, or may reduce the number of companies interested in acquiring us. See “Risk factors— Provisions in our collaboration agreement with Celgene Corporation may prevent or delay a change in control.”

## Intellectual property

We strive to protect and enhance the proprietary technology, inventions, and improvements that are commercially important to the development of our business, including seeking, maintaining, and defending patent rights, whether developed internally or licensed from third parties. We also rely on trade secrets relating to our proprietary technology platform and on know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain our proprietary position in the field of gene therapy that may be important for the development of our business. We additionally rely on regulatory protection afforded through orphan drug designations, data exclusivity, market exclusivity, and patent term extensions where available.

Our commercial success may depend in part on our ability to obtain and maintain patent and other proprietary protection for commercially important technology, inventions and know-how related to our business; defend and enforce our patents; preserve the confidentiality of our trade secrets; and operate without infringing the valid enforceable patents and proprietary rights of third parties. Our ability to stop third parties from making, using, selling, offering to sell or importing our products may depend on the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities. With respect to both licensed and company-owned intellectual property, we cannot be sure that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our commercial products and methods of manufacturing the same.

We have developed or in-licensed numerous patents and patent applications and possess substantial know-how and trade secrets relating to the development and commercialization of gene therapy products. Our proprietary intellectual property, including patent and non-patent intellectual property, is generally directed to, for example, certain genes, methods of transferring genetic material into cells, processes to manufacture our lentivirus-based product candidates and other proprietary technologies and processes related to our lead product development candidates. As of the date of this prospectus, our patent portfolio includes the following:

- approximately 176 patents or patent applications that we own or have exclusively in-licensed from academic institutions and third parties related to lentiviral vectors and vector systems;
- approximately 58 patents or patent applications that we have non-exclusively in-licensed or optioned from academic institutions and third parties related to lentiviral vectors and vector systems;
- approximately 18 patents or patent applications that we own, including eight that are co-owned with MIT, related to vector manufacturing or production;
- approximately seven patents or patent applications that have been non-exclusively in-licensed from academic institutions and third parties related to vector manufacturing or production; and

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- approximately 12 patents or patent applications that we own or have exclusively in-licensed from academic institutions and third parties related to therapeutic cellular products.

Our objective is to continue to expand our portfolio of patents and patent applications in order to protect our gene therapy product candidates and lentiviral manufacturing process. Examples of the products and technology areas covered by our intellectual property portfolio are described below. See also “—License agreements.”

### ***Childhood Cerebral Adrenoleukodystrophy (CCALD)***

The CCALD platform includes three patent portfolios, described below.

- **Pasteur Institute.** The Pasteur patent portfolio contains patent applications directed to FLAP/cPPT elements and lentiviral vectors utilized to produce our Lenti-D product candidate for CCALD. As of March 31, 2013, we had an exclusive license (from Pasteur Institute) to four issued U.S. patents and four pending U.S. applications. Corresponding foreign patents and patent applications include pending applications or issued patents in Australia, Canada, China, Europe, Hong Kong, Israel, and Japan. We expect the issued composition of matter patents to expire from 2019-2023 in the United States, and from 2019-2020 in the rest of the world (excluding possible patent term extensions). Further, we expect composition of matter patents, if issued from the pending patent applications and if the appropriate maintenance, renewal, annuity or other governmental fees are paid, to expire in 2019-2020 (excluding possible patent term extensions). We expect the patents and patent applications in this portfolio other than composition of matter patents, if issued, and if the appropriate maintenance, renewal, annuity, or other governmental fees are paid, to expire in 2019-2020 (worldwide, excluding possible patent term extensions).
- **RDF.** The in-licensed patent portfolio from Research Development Foundation, or RDF, in part, contains patents and patent applications directed to aspects of our lentiviral vectors utilized to produce our Lenti-D product candidate for CCALD. As of March 31, 2013, we had an exclusive license (from RDF) to three issued U.S. patents and one pending U.S. application related to our lentiviral vector platform. Corresponding foreign patents and patent applications related to our lentiviral vector platform include pending applications or issued patents in Canada, Europe, and Israel. We expect the issued composition of matter patents to expire from 2022-2023 (excluding possible patent term extensions). Further, we expect composition of matter patents, if issued from the pending patent applications and if the appropriate maintenance, renewal, annuity or other governmental fees are paid, to expire in 2021-2022 (excluding possible patent term extensions). We expect the patents and patent applications in this portfolio other than composition of matter patents, if issued, and if the appropriate maintenance, renewal, annuity, or other governmental fees are paid, to expire in 2021-2022 (worldwide, excluding possible patent term extensions).
- **bluebird bio.** The bluebird bio patent portfolio contains patent applications directed to compositions of matter for CCALD gene therapy vectors and compositions and methods of using the vectors and compositions in cell-based gene therapy of adrenoleukodystrophy or adrenomyeloneuropathy. As of March 31, 2013, we owned one pending U.S. application and one pending Patent Cooperation Treaty, or PCT, application that is due for national stage entry in December 2013. We expect the composition of matter patent for the CCALD gene therapy vectors, if issued from the pending patent application and if the appropriate

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maintenance, renewal, annuity or other governmental fees are paid, to expire in 2032 (worldwide, excluding possible patent term extensions). We expect the other patents and patent applications in this portfolio, if issued, and if the appropriate maintenance, renewal, annuity, or other governmental fees are paid, to expire in 2032 (worldwide, excluding possible patent term extensions).

***β-thalassemia/SCD***

The β-thalassemia/SCD platform includes three patent portfolios, described below.

- **Pasteur Institute.** The in-licensed Pasteur patent portfolio contains the patents and patent applications described above directed towards aspects of our lentiviral vectors utilized to produce our LentiGlobin product candidate for β-thalassemia and SCD.
- **RDF.** The in-licensed RDF patent portfolio contains the patents and patent applications described above directed towards aspects of our lentiviral vectors utilized to produce our LentiGlobin product candidate for β-thalassemia and SCD.
- **MIT/bluebird bio.** The co-owned patent portfolio contains patents and patent applications directed to certain specific compositions of matter for lentiviral β-globin expression vectors. As of March 31, 2013, we co-owned one issued U.S. patent and two pending U.S. applications, as well as corresponding foreign patents issued in Europe and Hong Kong. We expect the issued composition of matter patents to expire in 2023 (excluding possible patent term extensions). Further, we expect composition of matter patents, if issued from the pending patent applications and if the appropriate maintenance, renewal, annuity or other governmental fees are paid, to expire in 2023 (excluding possible patent term extensions). We expect the other patents and patent applications in this portfolio, if issued, and if the appropriate maintenance, renewal, annuity, or other governmental fees are paid, to expire in 2023 (worldwide, excluding possible patent term extensions). We note that we have an exclusive license to MIT's interest in this co-owned intellectual property.

***Lentiviral platform (e.g., vectors, manufacturing, and cell therapy products)***

The lentiviral platform, which is potentially applicable to the CCALD, β-thalassemia, SCD and other potential programs, includes three patent portfolios, described below.

- **Pasteur Institute.** The Pasteur patent portfolio contains the patents and patent applications described above.
- **RDF.** The in-licensed RDF patent portfolio contains the patents and patent applications described above.
- **bluebird bio.** One aspect of the bluebird bio patent portfolio contains patents and patent applications directed to certain specific compositions of matter and improved methods for selecting and delivering transduced cells. As of March 31, 2013, we owned one pending PCT application that is due for national stage entry in July 2013. We expect any composition of matter or methods patents, if issued from a corresponding nonprovisional national stage application, and if the appropriate maintenance, renewal, annuity or other governmental fees are paid, to expire in 2031 (worldwide, excluding possible patent term extensions). We expect the other patents and patent applications in this portfolio, if issued, and if the appropriate maintenance, renewal, annuity, or other governmental fees

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are paid, to expire in 2031 (worldwide, excluding possible patent term extensions). Another component of the bluebird bio patent portfolio includes the vector manufacturing platform and is potentially applicable to the CCALD,  $\beta$ -thalassemia, SCD and other programs. This portion of the portfolio contains patents and patent applications directed to compositions of matter for improved packaging cells and cell lines and improved methods for transfection and transduction of therapeutic cells. As of March 31, 2013, we owned two U.S. provisional applications, which have nonprovisional filing bar dates in 2013, and two pending PCT applications, which are due for national stage entry in December 2013 and March 2014. We expect composition of matter and method patents, if issued from a corresponding nonprovisional national stage application, and if the appropriate maintenance, renewal, annuity or other governmental fees are paid, to expire in 2032 (worldwide, excluding possible patent term extensions).

In addition to the above, we have established expertise and development capabilities focused in the areas of preclinical research and development, manufacturing and manufacturing process scale-up, quality control, quality assurance, regulatory affairs and clinical trial design and implementation. We believe that our focus and expertise will help us develop products based on our proprietary intellectual property.

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the date of filing the non-provisional application. In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office in granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier-filed patent.

The term of a patent that covers an FDA-approved drug may also be eligible for patent term extension, which permits patent term restoration of a U.S. patent as compensation for the patent term lost during the FDA regulatory review process. The Hatch-Waxman Act permits a patent term extension of up to five years beyond the expiration of the patent. The length of the patent term extension is related to the length of time the drug is under regulatory review. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug may be extended. Moreover, a patent can only be extended once, and thus, if a single patent is applicable to multiple products, it can only be extended based on one product. Similar provisions are available in Europe and other foreign jurisdictions to extend the term of a patent that covers an approved drug. When possible, depending upon the length of clinical trials and other factors involved in the filing of a new drug application, or NDA, we expect to apply for patent term extensions for patents covering our product candidates and their methods of use.

We may rely, in some circumstances, on trade secrets to protect our technology. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our



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consultants, contractors or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

## **License agreements**

### ***Inserm-Transfert***

In May 2009, we entered into an exclusive license with Inserm-Transfert, which is a wholly-owned subsidiary of Institut national de la santé et de la recherche médicale, for use of certain patents and know-how related to the ABCD1 gene and corresponding protein, for use in the field of human ALD therapy. This agreement was amended once in 2012 and again in 2013. Inserm-Transfert is referred to herein as Inserm. The Inserm licensed patent portfolio includes at least three U.S. and foreign patents and patent applications. This portfolio has no pending applications. Inserm retains the right to practice the intellectual property licensed under the agreement for educational, clinical and preclinical studies purposes.

Upon commercialization of our products covered by the in-licensed intellectual property, which we expect would include our Lenti-D product candidate, we will be obligated to pay Inserm a percentage of net sales as a royalty for the longer of the life of any patents covering the product or 10 years from first commercial sale. This royalty is in the low single digits. The royalties payable to Inserm are subject to reduction for any third party payments required to be made, with a minimum floor in the low single digits.

We are required to use all commercially reasonable efforts to develop licensed products and introduce them into the commercial market as soon as practical, consistent with our reasonable business practices and judgment in compliance with an agreed upon development plan. We have assumed certain development, regulatory and commercial milestone obligations and must report on our progress in achieving these milestones on an annual basis.

We may unilaterally terminate the license agreement at any time. Either party may terminate the agreement in the event of the other party's material breach which remains uncured after 60 days of receiving written notice of such breach or in the event the other party become subject of a voluntary or involuntary petition in bankruptcy and such petition is not dismissed with prejudice within 120 days after filing. In addition, Inserm may terminate the license agreement in the event that we cannot prove within 60 days of written notice from Inserm that we have been diligent in developing the licensed products and introducing them into the commercial market.

Absent early termination, the agreement will automatically terminate upon the expiration of all issued patents and filed patent applications within the patent rights covered by the agreement or 10 years from the date of first commercial sale of a licensed product, whichever is later. The license grant ceases in connection with any such termination. The longest lived patent rights licensed to us under the agreement are currently expected to expire in 2016.

### ***Institut Pasteur***

In September 2011, we entered into a license with Institut Pasteur for certain patents relating to the use of DNA sequences, lentiviral vectors and recombinant cells in the field of *ex vivo* gene therapy in a range of indications. This agreement was amended twice in 2012. The Institut Pasteur licensed patent portfolio includes at least 23 U.S. and foreign patents and patent

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applications. Any patents within this portfolio that have issued or may yet issue would have a statutory expiration date in 2019 and 2020. The license is exclusive for products containing human (HIV-1 and HIV-2) lentiviral vector and non-exclusive for products containing non-human lentiviral vector. Institut Pasteur retains the right, on behalf of itself, its licensees and research partners, to conduct research using the licensed intellectual property.

We have the right to grant sublicenses outright to third parties under the agreement. If we receive any income (cash or non-cash) in connection with such sublicenses we must pay Institut Pasteur a percentage of such income varying from low single digits to lower to mid double digits depending on the nature of the sublicense.

Upon commercialization of our products covered by the in-licensed intellectual property, which we expect would include our Lenti-D and LentiGlobin product candidates, we will be obligated to pay Institut Pasteur a percentage of net sales as a royalty. This royalty varies depending on the indication of the product but in any event is in the low single digits. In addition, starting in 2016 we must make under this agreement an annual maintenance payment which is creditable against royalty payments on a year-by-year basis. If the combined royalties we would be required to pay to Institut Pasteur and third parties is higher than a pre-specified percentage, we may ask Institut Pasteur to re-negotiate our royalty rates under this relationship.

We are required to use all reasonable commercial efforts (as compared to a company of similar size and scope) to develop and commercialize one or more products in the license field and to obtain any necessary governmental approvals in respect of, and market the products in license field, if any. Additionally, we have assumed certain development and regulatory milestone obligations. We must report on our progress towards achieving these milestones on an annual basis.

We may unilaterally terminate the license agreement at any time by sending Institut Pasteur 90 day prior written notice. Either party may terminate the license in the event of the other party's substantial breach which remains uncured after 60 days of receiving written notice of such breach. Institut Pasteur may also terminate the agreement in the event bankruptcy proceedings are opened against us and not dismissed within 60 days.

Absent early termination, the agreement will automatically terminate upon the expiration of the last licensed patents. In the event the agreement is terminated, while the license grant would cease, we would retain the right to manufacture, import, use and sell licensed products for a certain period of time post-termination. In addition, our ownership stake in certain jointly made improvements covered by the licensed patents would survive termination of the agreement. The longest lived patent rights licensed to us under the agreement are currently expected to expire in 2023.

### ***Stanford University***

In July 2002, we entered into a non-exclusive license agreement with the Board of Trustees of the Leland Stanford Junior University, referred to herein as Stanford, which we amended and restated in April 2012. Under this agreement, we are granted a license to use the HEK293T cell line for any commercial or non-commercial use for research, non clinical and clinical development purpose and human and animal gene therapy products.

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We have the right to grant sublicenses outright to third parties under the agreement. For each such sublicense we grant, we must pay Stanford a fee (unless the sublicense is to a collaborating partner, contract manufacturer or contract research organization).

Upon commercialization of our products covered by the in-licensed intellectual property, which we expect would include our Lenti-D product candidate, we will be obligated to pay Stanford a percentage of net sales as a royalty. This royalty varies with net sales but in any event is in the low single digits and is reduced for each third-party license that requires payments by us with respect to a licensed product, provided that the royalty to Stanford is not less than a specified percentage which is less than one percent. Beginning in April 2013, we will pay Stanford an annual maintenance fee, which will be creditable against our royalty payments.

We may unilaterally terminate the agreement by giving Stanford 30 days' written notice. Stanford may also terminate the license agreement if after 30 days of providing notice we are delinquent on any report or payment, are not using commercially reasonable efforts to develop, manufacture and/or commercialize one or more licensed products, are in material breach of any provision or provide any false report. Termination of this agreement may require us to utilize different cell types for vector manufacturing, which could lead to delays.

Absent early termination, the license will expire in April 2037. We may elect to extend the term for an additional 25 years so long as we have a commercial product on the market at that time and we are in material compliance with the license agreement.

### ***Massachusetts Institute of Technology***

In December 1996, we entered into an exclusive license with the Massachusetts Institute of Technology, referred to herein as MIT, for use of certain patents in any field. This license agreement was amended in December 2003, May 2004 and June 2011. The licensed patent portfolio includes at least 26 U.S. and foreign patents and patent applications. Any patents within this portfolio that have issued or may yet issue would have a statutory expiration date in 2023. This license also has been amended to include a case jointly owned by MIT and us wherein we received the exclusive license to MIT's rights in this case. MIT retains the right to practice the intellectual property licensed under the agreement for noncommercial research purposes.

We have the right to grant sublicenses outright to third parties under the agreement. In the event we sublicense the patent rights, we must pay MIT a percentage of all payments we receive from by the sublicensee. This percentage varies from mid-single digits to low double digits.

Upon commercialization of our products covered by the in-licensed intellectual property, which we expect would include our LentiGlobin product candidate, we will be obligated to pay MIT a percentage of net sales by us or our sublicensees as a royalty. This royalty is in the low single digits and is reduced for royalties payable to third parties, provided that the royalty to MIT is not less than a specified percentage that is less than one-percent. In addition, we make under this agreement an annual maintenance payment which may be credited against the royalty payments.

We are required to use diligent efforts to market licensed products and to continue active, diligent development and marketing efforts for licensed products during the term of the agreement. We have assumed certain milestones with respect to raising capital investment and regulatory progress. We must report on our progress on achieving these milestones on an annual basis.

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We may unilaterally terminate the license agreement upon six months' notice to MIT. MIT may terminate the agreement if we cease to carry on our business, or in the event of our material breach which remains uncured after 90 days of receiving written notice of such breach (30 days in the case of nonpayment). In the event the agreement is terminated, while the license grant would cease, we would retain a right to complete manufacture of any licensed products in process and sell then-existing inventory. In addition, MIT would grant our sublicensees a direct license following such termination. With respect to jointly owned intellectual property, any termination would allow MIT to grant licenses to any third party to such intellectual property, without our approval, unless a sublicensee was already in place, in which case, MIT would grant our sublicensees a direct license.

***Research Development Foundation***

In December 2011, we entered into an exclusive license with RDF to use certain patents that involve lentiviral vectors. The RDF licensed patent portfolio includes at least 14 U.S. and foreign patents and patent applications. Any patents within this portfolio that have issued or may yet issue would have an expected statutory expiration date of 2021 or 2022. RDF retains the right, on behalf of itself and other nonprofit academic research institutions, to practice and use the licensed patents for any academic, non-clinical research and educational purposes. We have the right to grant sublicenses outright to third parties under the agreement.

Upon commercialization of our products covered by the in-licensed intellectual property, which we expect would include both our Lenti-D and LentiGlobin product candidates, we are obligated to pay RDF a percentage of net sales as a royalty. This royalty is in the low single digits and is reduced by half if during the following ten years from the first marketing approval the last valid claim within the licensed patent that covers the licensed product expires or ends.

We are required to use commercially reasonable and diligent efforts for a company of our size and resources to develop or commercialize one or more licensed products, including our first licensed product by 2016 and a second licensed product by 2018. These diligence efforts include minimum annual royalty payments to RDF, which are creditable against earned royalties otherwise due to RDF, and payments upon regulatory milestones.

RDF may terminate the agreement in the event of our material breach which remains uncured after 90 days of receiving written notice of such breach (30 days in the case of nonpayment) or in the event we become bankrupt, our business or assets or property are placed in the hands of a receiver, assignee or trustee, we institute or suffer to be instituted any procedure in bankruptcy court for reorganization or rearrangement of our financial affairs, make a general assignment for the benefit of creditors, or if we or an affiliate or a sublicensee institutes any procedure challenging the validity or patentability of any patent or patent application within the licensed patents, the agreement will immediately terminate.

Absent early termination, the agreement will continue until its expiration upon the later of there being no more valid claims within the licensed patents or the expiration of our royalty obligations on licensed products that are subject to an earned royalty, if such earned royalty is based on the minimum 10-year royalty period described above. In the event the agreement is terminated, while the license grant would cease, RDF will grant our sublicensees a direct license. The longest lived patent rights licensed to us under the agreement are in one U.S. patent currently expected to expire in 2025.

## Competition

The biotechnology and pharmaceutical industries are characterized by intense and rapidly changing competition to develop new technologies and proprietary products. While we believe that our proprietary asset estate and scientific expertise in the gene therapy field provide us with competitive advantages, we face potential competition from many different sources, including larger and better-funded pharmaceutical companies. Not only must we compete with other companies that are focused on gene therapy products but any products that we may commercialize will have to compete with existing therapies and new therapies that may become available in the future.

There are other organizations working to improve existing therapies or to develop new therapies for our initially selected indications. Depending on how successful these efforts are, it is possible they may increase the barriers to adoption and success for our Lenti-D and LentiGlobin product candidates, if approved. These efforts include the following:

- **CCALD:** The current standard of care for the treatment of CCALD is allogeneic HSCT. We understand that various academic centers around the world are seeking to develop improvements to allogeneic HSCT. In addition, some physicians recommend glyceryl trierucate—better known as Lorenzo’s Oil—to patients diagnosed with ALD or AMN. However, Lorenzo’s Oil has not been clinically proven to address the cerebral symptoms of ALD, and has not been approved by any major regulatory agency as a prescription drug. There are efforts underway to obtain FDA approval for Lorenzo’s Oil as a prescription drug. We are also aware of some early-stage, preclinical efforts in academic centers to investigate the use of anti-oxidants for patients with AMN.
- **$\beta$ -thalassemia:** The current standard of care for the treatment of  $\beta$ -thalassemia in the developed world is chronic blood transfusions to address the patient’s anemia. In addition, such patients often receive iron chelation therapy to help manage the iron overload associated with their chronic blood transfusions. We understand that established biopharmaceutical companies, such as Novartis AG and ApoPharma Inc., who provide the leading iron chelation therapy, are seeking to develop improvements to their product profile and accessibility. In addition, some patients with  $\beta$ -thalassemia receive HCST treatment, particularly if a sufficiently well-matched source of donor cells is identified. We understand that various academic centers around the world are seeking to develop improvements to allogeneic HSCT. A number of different approaches are under investigation to improve treatment options, including iron modulating agents and fetal hemoglobin regulators. There are also several different groups developing gene therapy approaches for  $\beta$ -thalassemia. Some of these groups use a similar *ex vivo* autologous approach, but make use of different vectors and different cell processing techniques. These include: Memorial Sloan Kettering, which received approval for its IND in 2012, and is actively recruiting for a Phase I/II gene therapy study; GlaxoSmithKline Plc, which has entered into an agreement with the San Raffaele Telethon Institute for Gene Therapy to advance several gene therapy programs, including one for  $\beta$ -thalassemia, although to our knowledge no clinical studies have been initiated; and Sangamo BioSciences Inc., which has announced plans to investigate the use of zinc finger nuclease-mediated gene-correction techniques in hemoglobinopathies including  $\beta$ -thalassemia, although to our knowledge no clinical studies have been initiated.

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- **Sickle cell disease:** The current standard of care for the treatment of SCD in the developed world is chronic blood transfusions or hydroxyurea (a generic drug). In addition, such patients often receive iron chelation therapy to help manage the iron overload associated with chronic blood transfusions. We are aware of ongoing studies that continue to evaluate the efficacy and safety of hydroxyurea in various populations, and it can be assumed that the data from these studies will influence future utilization of this therapeutic modality. In addition, some patients with SCD receive allogeneic HSCT treatment, particularly if a sufficiently well-matched source of donor cells is identified. We understand that various academic centers around the world are seeking to develop improvements to allogeneic HSCT. There is also considerable interest from academic centers and biopharmaceutical companies to develop new therapies for SCD. A number of different approaches are under investigation, targeting the various aspects of SCD pathophysiology, including: fetal hemoglobin regulators, including HQK-1001 in Phase II studies supported by HemaQuest Pharmaceuticals Inc., and Vorinostat in Phase II studies supported by Merck & Co.; and pan-selectin inhibitors, including GMI-1070 in Phase II studies supported by GlycoMimetics Inc. (in 2011, Pfizer Inc. and GlycoMimetics Inc. entered a global collaboration to advance this compound). There are also several different groups developing gene therapy approaches for SCD. Some of these groups use a similar *ex vivo* autologous approach, but make use of different vectors and different cell processing techniques. These include: UCLA, which has received funding from the California Institute of Regenerative Medicine to pursue a Phase I/II gene therapy study for SCD, although to our knowledge no clinical studies have been initiated and Sangamo BioSciences Inc., which has announced plans to investigate the use of zinc finger nuclease-mediated gene-correction techniques in hemoglobinopathies including SCD, although to our knowledge no clinical studies have been initiated.

Many of our competitors, either alone or with their strategic partners, have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of treatments and the commercialization of those treatments. Accordingly, our competitors may be more successful than us in obtaining approval for treatments and achieving widespread market acceptance. Our competitors' treatments may be more effective, or more effectively marketed and sold, than any treatment we may commercialize and may render our treatments obsolete or non-competitive before we can recover the expenses of developing and commercializing any of our treatments.

Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical study sites and patient registration for clinical studies, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We anticipate that we will face intense and increasing competition as new drugs enter the market and advanced technologies become available. We expect any treatments that we develop and commercialize to compete on the basis of, among other things, efficacy, safety, convenience of administration and delivery, price, the level of generic competition and the availability of reimbursement from government and other third-party payors.

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Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of generic products. If our therapeutic product candidates are approved, we expect that they will be priced at a significant premium over competitive generic products.

## **Government regulation**

Biological products, including gene therapy products, are subject to regulation under the Federal Food, Drug, and Cosmetic Act, or FD&C Act, and the Public Health Service Act, or PHS Act, and other federal, state, local and foreign statutes and regulations. Both the FD&C Act and the PHS Act and their corresponding regulations govern, among other things, the testing, manufacturing, safety, efficacy, labeling, packaging, storage, record keeping, distribution, reporting, advertising and other promotional practices involving biological products. FDA approval must be obtained before clinical testing of biological products, and each clinical study protocol for a gene therapy product is reviewed by the FDA and, in some instances, the NIH, through its RAC. FDA approval also must be obtained before marketing of biological products. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources and we may not be able to obtain the required regulatory approvals.

Within the FDA, the CBER regulates gene therapy products. The CBER works closely with the NIH and its RAC, which makes recommendations to the NIH on gene therapy issues and engages in a public discussion of scientific, safety, ethical and societal issues related to proposed and ongoing gene therapy protocols. The FDA and the NIH have published guidance documents with respect to the development and submission of gene therapy protocols. The FDA also has published guidance documents related to, among other things, gene therapy products in general, their preclinical assessment, observing subjects involved in gene therapy studies for delayed adverse events, potency testing, and chemistry, manufacturing and control information in gene therapy INDs.

Ethical, social and legal concerns about gene therapy, genetic testing and genetic research could result in additional regulations restricting or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed interest in further regulating biotechnology. More restrictive regulations or claims that our products are unsafe or pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of our product candidates under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

***U.S. biological products development process***

The process required by the FDA before a biological product may be marketed in the United States generally involves the following:

- completion of nonclinical laboratory tests and animal studies according to good laboratory practices, or GLPs, and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- submission to the FDA of an application for an IND, which must become effective before human clinical studies may begin;
- performance of adequate and well-controlled human clinical studies according to the FDA's regulations commonly referred to as good clinical practices, or GCPs, and any additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the proposed biological product for its intended use;
- submission to the FDA of a Biologics License Application, or BLA, for marketing approval that includes substantive evidence of safety, purity, and potency from results of nonclinical testing and clinical studies;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biological product is produced to assess compliance with GMP, to assure that the facilities, methods and controls are adequate to preserve the biological product's identity, strength, quality and purity and, if applicable, the FDA's current good tissue practices, or GTPs, for the use of human cellular and tissue products;
- potential FDA audit of the nonclinical and clinical study sites that generated the data in support of the BLA; and
- FDA review and approval, or licensure, of the BLA.

Before testing any biological product candidate, including a gene therapy product, in humans, the product candidate enters the preclinical testing stage. Preclinical tests, also referred to as nonclinical studies, include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the product candidate. The conduct of the preclinical tests must comply with federal regulations and requirements including GLPs.

Where a gene therapy study is conducted at, or sponsored by, institutions receiving NIH funding for recombinant DNA research, prior to the submission of an IND to the FDA, a protocol and related documentation is submitted to and the study is registered with the NIH Office of Biotechnology Activities, or OBA, pursuant to the NIH Guidelines for Research Involving Recombinant DNA Molecules, or NIH Guidelines. Compliance with the NIH Guidelines is mandatory for investigators at institutions receiving NIH funds for research involving recombinant DNA, however many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. The NIH is responsible for convening the RAC, a federal advisory committee, that discusses protocols that raise novel or particularly important scientific, safety or ethical considerations at one of its quarterly public meetings. The OBA will notify the FDA of the RAC's decision regarding the necessity for full public review of a gene therapy protocol. RAC proceedings and reports are posted to the OBA web site and may be accessed by the public.



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The clinical study sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical study on a clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical study can begin. With gene therapy protocols, if the FDA allows the IND to proceed, but the RAC decides that full public review of the protocol is warranted, the FDA will request at the completion of its IND review that sponsors delay initiation of the protocol until after completion of the RAC review process. The FDA may also impose clinical holds on a biological product candidate at any time before or during clinical studies due to safety concerns or non-compliance. If the FDA imposes a clinical hold, studies may not recommence without FDA authorization and then only under terms authorized by the FDA. Accordingly, we cannot be sure that submission of an IND will result in the FDA allowing clinical studies to begin, or that, once begun, issues will not arise that suspend or terminate such studies.

Clinical studies involve the administration of the biological product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the study sponsor's control. Clinical studies are conducted under protocols detailing, among other things, the objectives of the clinical study, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical study will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND. Clinical studies must be conducted and monitored in accordance with the FDA's regulations comprising the GCP requirements, including the requirement that all research subjects provide informed consent. Further, each clinical study must be reviewed and approved by an independent institutional review board, or IRB, at or servicing each institution at which the clinical study will be conducted. An IRB is charged with protecting the welfare and rights of study participants and considers such items as whether the risks to individuals participating in the clinical studies are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical study subject or his or her legal representative and must monitor the clinical study until completed. Clinical studies also must be reviewed by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees basic and clinical research conducted at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

Human clinical studies are typically conducted in three sequential phases that may overlap or be combined:

- *Phase I.* The biological product is initially introduced into healthy human subjects and tested for safety. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.
- *Phase II.* The biological product is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.

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- *Phase III.* Clinical studies are undertaken to further evaluate dosage, clinical efficacy, potency, and safety in an expanded patient population at geographically dispersed clinical study sites. These clinical studies are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product labeling.

Post-approval clinical studies, sometimes referred to as Phase IV clinical studies, may be conducted after initial marketing approval. These clinical studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up. The FDA recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by ten years of annual queries, either in person or by questionnaire, of study subjects.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical study investigators. Annual progress reports detailing the results of the clinical studies must be submitted to the FDA. Written IND safety reports must be promptly submitted to the FDA, the NIH and the investigators for serious and unexpected adverse events, any findings from other studies, tests in laboratory animals or in vitro testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information. Phase I, Phase II and Phase III clinical studies may not be completed successfully within any specified period, if at all. The FDA or the sponsor or its data safety monitoring board may suspend a clinical study at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical study at its institution if the clinical study is not being conducted in accordance with the IRB's requirements or if the biological product has been associated with unexpected serious harm to patients.

Human gene therapy products are a new category of therapeutics. Because this is a relatively new and expanding area of novel therapeutic interventions, there can be no assurance as to the length of the study period, the number of patients the FDA will require to be enrolled in the studies in order to establish the safety, efficacy, purity and potency of human gene therapy products, or that the data generated in these studies will be acceptable to the FDA to support marketing approval. The NIH and the FDA have a publicly accessible database, the Genetic Modification Clinical Research Information System which includes information on gene transfer studies and serves as an electronic tool to facilitate the reporting and analysis of adverse events on these studies.

Concurrent with clinical studies, companies usually complete additional animal studies and must also develop additional information about the physical characteristics of the biological product as well as finalize a process for manufacturing the product in commercial quantities in accordance with GMP requirements. To help reduce the risk of the introduction of adventitious agents with use of biological products, the PHS Act emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the sponsor must develop methods for testing the identity, strength, quality,

potency and purity of the final biological product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the biological product candidate does not undergo unacceptable deterioration over its shelf life.

### ***U.S. review and approval processes***

After the completion of clinical studies of a biological product, FDA approval of a BLA, must be obtained before commercial marketing of the biological product. The BLA must include results of product development, laboratory and animal studies, human studies, information on the manufacture and composition of the product, proposed labeling and other relevant information. In addition, under the Pediatric Research Equity Act, or PREA, a BLA or supplement to a BLA must contain data to assess the safety and effectiveness of the biological product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to any biological product for an indication for which orphan designation has been granted. The testing and approval processes require substantial time and effort and there can be no assurance that the FDA will accept the BLA for filing and, even if filed, that any approval will be granted on a timely basis, if at all.

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA must be accompanied by a user fee. The FDA adjusts the PDUFA user fees on an annual basis. According to the FDA's fee schedule, effective through September 30, 2013, the user fee for an application requiring clinical data, such as a BLA, is \$1,958,800. PDUFA also imposes an annual product fee for biologics (\$98,380) and an annual establishment fee (\$526,500) on facilities used to manufacture prescription biologics. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on BLAs for products designated as orphan drugs, unless the product also includes a non-orphan indication.

Within 60 days following submission of the application, the FDA reviews a BLA submitted to determine if it is substantially complete before the agency accepts it for filing. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the BLA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the BLA. The FDA reviews the BLA to determine, among other things, whether the proposed product is safe and potent, or effective, for its intended use, and has an acceptable purity profile, and whether the product is being manufactured in accordance with GMP to assure and preserve the product's identity, safety, strength, quality, potency and purity. The FDA may refer applications for novel biological products or biological products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the biological product approval process, the FDA also will determine whether a Risk Evaluation and Mitigation Strategy, or REMS, is necessary to assure the safe use of the biological product. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

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Before approving a BLA, the FDA will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with GMP requirements and adequate to assure consistent production of the product within required specifications. For a gene therapy product, the FDA also will not approve the product if the manufacturer is not in compliance with the GTPs. These are FDA regulations that govern the methods used in, and the facilities and controls used for, the manufacture of human cells, tissues, and cellular and tissue based products, or HCT/Ps, which are human cells or tissue intended for implantation, transplant, infusion, or transfer into a human recipient. The primary intent of the GTP requirements is to ensure that cell and tissue based products are manufactured in a manner designed to prevent the introduction, transmission and spread of communicable disease. FDA regulations also require tissue establishments to register and list their HCT/Ps with the FDA and, when applicable, to evaluate donors through screening and testing. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure that the clinical studies were conducted in compliance with IND study requirements and GCP requirements. To assure GMP, GTP and GCP compliance, an applicant must incur significant expenditure of time, money and effort in the areas of training, record keeping, production, and quality control.

Notwithstanding the submission of relevant data and information, the FDA may ultimately decide that the BLA does not satisfy its regulatory criteria for approval and deny approval. Data obtained from clinical studies are not always conclusive and the FDA may interpret data differently than we interpret the same data. If the agency decides not to approve the BLA in its present form, the FDA will issue a complete response letter that usually describes all of the specific deficiencies in the BLA identified by the FDA. The deficiencies identified may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical studies. Additionally, the complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing, or dispensing in the form of a risk management plan, or otherwise limit the scope of any approval. In addition, the FDA may require post marketing clinical studies, sometimes referred to as Phase IV clinical studies, designed to further assess a biological product's safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

One of the performance goals agreed to by the FDA under the PDUFA is to review 90% of standard BLAs in 10 months and 90% of priority BLAs in six months, whereupon a review decision is to be made. The FDA does not always meet its PDUFA goal dates for standard and priority BLAs and its review goals are subject to change from time to time. The review process and the PDUFA goal date may be extended by three months if the FDA requests or the BLA sponsor otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date.

### ***Orphan drug designation***

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biological product intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making a drug or biological product available in the United States for this type of disease or condition will be recovered from sales of the product. Orphan product designation must be requested before submitting an NDA or BLA. After the FDA grants orphan product designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan product designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug or biological product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity. Competitors, however, may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan product exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval of the same biological product as defined by the FDA or if our product candidate is determined to be contained within the competitor's product for the same indication or disease. If a drug or biological product designated as an orphan product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan product exclusivity. Orphan drug status in the European Union has similar, but not identical, benefits.

### ***Expedited development and review programs***

The FDA has a Fast Track program that is intended to expedite or facilitate the process for reviewing new drugs and biological products that meet certain criteria. Specifically, new drugs and biological products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening condition and demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor of a new drug or biologic may request the FDA to designate the drug or biologic as a Fast Track product at any time during the clinical development of the product. Unique to a Fast Track product, the FDA may consider for review sections of the marketing application on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the application, the FDA agrees to accept sections of the application and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the application.

Any product submitted to the FDA for marketing, including under a Fast Track program, may be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. Any product is eligible for priority review if it has the potential to provide safe and effective therapy where no satisfactory alternative therapy exists or a significant improvement in the treatment, diagnosis or prevention of a disease compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an

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application for a new drug or biological product designated for priority review in an effort to facilitate the review. Additionally, a product may be eligible for accelerated approval. Drug or biological products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval, which means that they may be approved on the basis of adequate and well-controlled clinical studies establishing that the product has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity. As a condition of approval, the FDA may require that a sponsor of a drug or biological product receiving accelerated approval perform adequate and well-controlled post-marketing clinical studies. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product. Fast Track designation, priority review and accelerated approval do not change the standards for approval but may expedite the development or approval process.

***Post-approval requirements***

Maintaining substantial compliance with applicable federal, state, and local statutes and regulations requires the expenditure of substantial time and financial resources. Rigorous and extensive FDA regulation of biological products continues after approval, particularly with respect to GMP. We will rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of any products that we may commercialize. Manufacturers of our products are required to comply with applicable requirements in the GMP regulations, including quality control and quality assurance and maintenance of records and documentation. Other post-approval requirements applicable to biological products, include reporting of GMP deviations that may affect the identity, potency, purity and overall safety of a distributed product, record-keeping requirements, reporting of adverse effects, reporting updated safety and efficacy information, and complying with electronic record and signature requirements. After a BLA is approved, the product also may be subject to official lot release. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products, such as viral vaccines, before releasing the lots for distribution by the manufacturer. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency, and effectiveness of biological products.

We also must comply with the FDA's advertising and promotion requirements, such as those related to direct-to-consumer advertising, the prohibition on promoting products for uses or in patient populations that are not described in the product's approved labeling (known as "off-label use"), industry-sponsored scientific and educational activities, and promotional activities involving the internet. Discovery of previously unknown problems or the failure to comply with the applicable regulatory requirements may result in restrictions on the marketing of a product or withdrawal of the product from the market as well as possible civil or criminal sanctions. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant or manufacturer to administrative or judicial civil or criminal sanctions and adverse publicity. FDA sanctions could include refusal to approve pending applications, withdrawal of an approval,

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clinical hold, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, mandated corrective advertising or communications with doctors, debarment, restitution, disgorgement of profits, or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

Biological product manufacturers and other entities involved in the manufacture and distribution of approved biological products are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with GMPs and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain GMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved BLA, including withdrawal of the product from the market. In addition, changes to the manufacturing process or facility generally require prior FDA approval before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

***U.S. patent term restoration and marketing exclusivity***

Depending upon the timing, duration and specifics of the FDA approval of the use of our product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application. Only one patent applicable to an approved biological product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The U.S. PTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may intend to apply for restoration of patent term for one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical studies and other factors involved in the filing of the relevant BLA.

A biological product can obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued "Written Request" for such a study.

The Patient Protection and Affordable Care Act, or Affordable Care Act, signed into law on March 23, 2010, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009 which created an abbreviated approval pathway for biological products shown to be similar to, or interchangeable with, an FDA-licensed reference biological product. This amendment to the PHS Act attempts to minimize duplicative testing. Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and

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a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product and, for products administered multiple times, the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. However, complexities associated with the larger, and often more complex, structure of biological products, as well as the process by which such products are manufactured, pose significant hurdles to implementation that are still being worked out by the FDA.

A reference biologic is granted twelve years of exclusivity from the time of first licensure of the reference product. On April 10, 2013, President Obama released his proposed budget for fiscal year 2014 and proposed to cut this twelve-year period of exclusivity down to seven years. He also proposed to prohibit additional periods of exclusivity for brand biologics due to minor changes in product formulations, a practice often referred to as “evergreening.” The first biologic product submitted under the abbreviated approval pathway that is determined to be interchangeable with the reference product has exclusivity against other biologics submitting under the abbreviated approval pathway for the lesser of (i) one year after the first commercial marketing, (ii) 18 months after approval if there is no legal challenge, (iii) 18 months after the resolution in the applicant’s favor of a lawsuit challenging the biologics’ patents if an application has been submitted, or (iv) 42 months after the application has been approved if a lawsuit is ongoing within the 42-month period.

***Additional regulation***

In addition to the foregoing, state and federal laws regarding environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservancy and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern our use, handling and disposal of various biological, chemical and radioactive substances used in, and wastes generated by, our operations. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. We believe that we are in material compliance with applicable environmental laws and that continued compliance therewith will not have a material adverse effect on our business. We cannot predict, however, how changes in these laws may affect our future operations.

***U.S. Foreign Corrupt Practices Act***

The U.S. Foreign Corrupt Practices Act, to which we are subject, prohibits corporations and individuals from engaging in certain activities to obtain or retain business or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any foreign government official, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity.

***Government regulation outside of the United States***

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical studies and any commercial sales and distribution of our products. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.



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Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical studies or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical study application much like the IND prior to the commencement of human clinical studies. In the European Union, for example, a CTA must be submitted to each country's national health authority and an independent ethics committee, much like the FDA and the IRB, respectively. Once the CTA is approved in accordance with a country's requirements, clinical study development may proceed.

The requirements and process governing the conduct of clinical studies, product licensing, pricing and reimbursement vary from country to country. In all cases, the clinical studies are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

To obtain regulatory approval of an investigational biological product under European Union regulatory systems, we must submit a marketing authorization application. The application used to file the BLA in the United States is similar to that required in the European Union, with the exception of, among other things, country-specific document requirements. The European Union also provides opportunities for market exclusivity. For example, in the European Union, upon receiving marketing authorization, new chemical entities generally receive eight years of data exclusivity and an additional two years of market exclusivity. If granted, data exclusivity prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic application. During the additional two-year period of market exclusivity, a generic marketing authorization can be submitted, and the innovator's data may be referenced, but no generic product can be marketed until the expiration of the market exclusivity. However, there is no guarantee that a product will be considered by the European Union's regulatory authorities to be a new chemical entity, and products may not qualify for data exclusivity. Products receiving orphan designation in the European Union can receive ten years of market exclusivity, during which time no similar medicinal product for the same indication may be placed on the market. An orphan product can also obtain an additional two years of market exclusivity in the European Union for pediatric studies. No extension to any supplementary protection certificate can be granted on the basis of pediatric studies for orphan indications.

The criteria for designating an "orphan medicinal product" in the European Union are similar in principle to those in the United States. Under Article 3 of Regulation (EC) 1411/2000, a medicinal product may be designated as orphan if (1) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (2) either (a) such condition affects no more than five in 10,000 persons in the European Union when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the European Union to justify investment; and (3) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the European Union, or if such a method exists, the product will be of significant benefit to those affected by the condition, as defined in Regulation (EC) 847/2000. Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers and are, upon grant of a marketing authorization, entitled to ten years of market exclusivity for the approved therapeutic indication. The application for orphan drug designation must be submitted before the application for marketing authorization. The applicant will receive a fee reduction for the marketing authorization application if the orphan drug designation has been

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granted, but not if the designation is still pending at the time the marketing authorization is submitted. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

The 10-year market exclusivity may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, for example, if the product is sufficiently profitable not to justify maintenance of market exclusivity. Additionally, marketing authorization may be granted to a similar product for the same indication at any time if:

- The second applicant can establish that its product, although similar, is safer, more effective or otherwise clinically superior;
- The applicant consents to a second orphan medicinal product application; or
- The applicant cannot supply enough orphan medicinal product.

For other countries outside of the European Union, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical studies, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical studies are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

## **Facilities**

Our corporate headquarters are located in Cambridge, Massachusetts. The facility we lease encompasses approximately 17,600 square feet of office and laboratory space. The lease for this facility expires on March 31, 2015, subject to our option to renew for up to one additional three-year term. We believe that our facility is sufficient to meet our needs and that suitable additional space will be available as and when needed.

## **Employees**

As of March 31, 2013, we had 50 full-time employees, 13 of whom have Ph.D. or M.D. degrees. Of these full-time employees, 37 employees are engaged in research and development activities and 13 employees are engaged in finance, legal, human resources, facilities and general management. We have no collective bargaining agreements with our employees and we have not experienced any work stoppages. We consider our relations with our employees to be good.

## **Legal proceedings**

From time to time, we are subject to various legal proceedings and claims that arise in the ordinary course of our business activities. Although the results of litigation and claims cannot be predicted with certainty, as of the date of this prospectus, we do not believe we are party to any claim or litigation the outcome of which, if determined adversely to us, would individually or in the aggregate be reasonably expected to have a material adverse effect on our business. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

## Management

### Executive officers, significant employees and directors

The following table sets forth information regarding our executive officers, significant employees and directors, as of March 31, 2013:

Name	Age	Position(s)
<b>Executive Officers:</b>		
Nick Leschly	40	President, Chief Executive Officer and Director
Jeffrey T. Walsh	47	Chief Operating Officer and Secretary
Mitchell H. Finer, Ph.D.	54	Chief Scientific Officer
David Davidson, M.D.	49	Chief Medical Officer
Linda C. Bain, CPA	42	Vice President, Finance and Business Operations and Treasurer
<b>Significant Employees:</b>		
Mark D. Angelino, Ph.D.	40	Vice President, Pharmaceutical Sciences
Richard E.T. Smith, Ph.D.	42	Vice President, Investor Relations
Faraz Ali	40	Vice President, Head of Program Management and Commercial Development
Cyrus Mozayani	38	Sr. Director, Business Development
Kathleen Wilkinson	41	Sr. Director, Human Resources
<b>Non-Management Directors:</b>		
Daniel S. Lynch(1)(2)	55	Chairman of the Board
Wendy L. Dixon, Ph.D.(1)	57	Director
Steven Gillis, Ph.D.(1)	60	Director
John M. Maraganore, Ph.D.(2)	50	Director
Geert-Jan Mulder, M.D.(4)	46	Director
Dr. Axel Polack(3)	56	Director
David P. Schenkein, M.D.(3)	55	Director
Robert I. Tepper, M.D.(2)	57	Director

(1) Member of the audit committee.

(2) Member of the compensation committee.

(3) Member of the nominating and corporate governance committee.

(4) Dr. Mulder has indicated to us his intention to resign from our board of directors upon the consummation of this offering.

**Nick Leschly** has served as our president and chief executive officer since September 2010. Previously, he served as our interim chief executive officer from March 2010 to September 2010. Formerly a partner of Third Rock Ventures, L.P. since its founding in 2007, Mr. Leschly played an

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integral role in the overall formation, development and business strategy of several of Third Rock's portfolio companies, including Agios Pharmaceuticals, Inc. and Edimer Pharmaceuticals, Inc. Prior to joining Third Rock, he worked at Millennium Pharmaceuticals, Inc., leading several early-stage drug development programs and served as the product and alliance leader for VELCADE. Mr. Leschly also founded and served as chief executive officer of MedXtend Corporation. He received his B.S. in molecular biology from Princeton University and his M.B.A. from Wharton Business School. We believe that Mr. Leschly's operation and historical experience with our Company gained from serving as our president, chief executive officer and member of the board of directors, combined with his experience in the venture capital industry and drug research and development qualify him to serve as a member of our board of directors.

**Jeffrey T. Walsh** has served as our chief operating officer since May 2011 and as our secretary since March 2013. Mr. Walsh has 25 years of experience in executive leadership positions with responsibility for finance, business development, commercial and business operations, strategic planning and legal functions with established and emerging public and private life sciences companies. From November 2008 to February 2011, Mr. Walsh served as chief business officer of Taligen Therapeutics, Inc. where he played a key role in the growth of the company and the ultimate sale of Taligen Therapeutics, Inc. to Alexion Pharmaceuticals, Inc. in January 2011. Mr. Walsh started his career at SmithKline Beecham Corporation in finance and worldwide business development roles. He subsequently held senior business development, finance and operations roles at PathoGenesis Corp. (acquired by Chiron Corporation), Allscripts Healthcare Solutions Inc., EXACT Sciences Corporation and Inotek Pharmaceuticals Corp. Mr. Walsh received his B.A. in sociology and economics from Yale University and his M.B.A. from the Kellogg Graduate School of Management at Northwestern University.

**Mitchell H. Finer, Ph.D.** has served as our chief scientific officer since March 2010. Prior to joining us, Dr. Finer served as senior vice president of development and operations for Novocell, Inc. (now ViaCyte, Inc.), a stem cell engineering company researching treatments for diabetes and other chronic diseases from November 2008 through March 2010. From July 2005 through November 2008, Dr. Finer served as chief executive officer of Intracel Holdings LLC. From June 2003 to June 2005, he held the position of president and chief executive officer of Genteric Inc., or Genteric, which filed a voluntary petition for reorganization under Chapter 11 of the U.S. bankruptcy code in August 2004. Previously, he had served as Genteric's chief scientific officer from November 2002 to June 2003 and as vice president of research and development for the Gencell division of Aventis Pharma (now Sanofi) from April 2002 to November 2002. He was also a founder and vice president of research for Cell Genesys Inc., and a founder of Abgenix, Inc. and Avalanche Biotechnologies, Inc. Dr. Finer received his B.A. in biochemistry and bacteriology from the University of California at Berkeley and his Ph.D. in biochemistry and molecular biology from Harvard University. He completed a postdoctoral fellowship at the Whitehead Institute for Biomedical Research.

**David Davidson, M.D.** has served as our chief medical officer since February 2012. Prior to joining us, Dr. Davidson served as a senior medical director at Genzyme Corporation, or Genzyme, where he led clinical research for programs in Phases I through IV across a wide range of therapeutic areas for more than a decade. Most recently, Dr. Davidson was the medical leader for Genzyme's gene therapy and Pompe disease enzyme replacement therapy programs. In addition to Dr. Davidson's translational medicine experience, he has also worked on a number of commercial products, including Fabrazyme and Myozyme/Lumizyme, and was integral in crafting the new drug application that resulted in the approval of Welchol. Prior to Genzyme, Dr. Davidson was a medical director at GelTex Pharmaceuticals Inc. Previously, he completed

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clinical and research fellowships in infectious diseases at the Harvard Longwood Combined Infectious Diseases Program. Dr. Davidson received his B.A. from Columbia University and his M.D. from New York University School of Medicine. In addition, he completed an internal medicine internship, residency training and an endocrinology research fellowship at the University of Chicago Hospitals.

**Linda C. Bain, CPA** has served as our vice president of finance and business operations since October 2011 and as our treasurer since March 2013. Previously, she served as vice president of corporate finance at Genzyme from September 2008 to September 2011, at Fidelity Investments from September 2007 to September 2008 and a number of positions at AstraZeneca from May 2000 to September 2007. She received her B.S. from the University of the Orange Free State in South Africa.

**Mark D. Angelino, Ph.D.** has served as our vice president of pharmaceutical sciences since May 2012. Previously, Dr. Angelino served as senior director of research and development and Cambridge site head at Baxter Healthcare Corporation from December 2010 to May 2012 and as vice president of pharmaceutical development at Archemix Corporation from May 2008 to December 2010. Dr. Angelino received his B.S. in Chemical Engineering from The Cooper Union and his M.S. in chemical engineering practice and Ph.D. in chemical engineering from the Massachusetts Institute of Technology.

**Richard E. T. Smith, Ph.D.** has served as our vice president of investor relations since March 2013. From March 2012 to March 2013, Dr. Smith served as a consultant for a number of biotechnology companies. Previously, Dr. Smith served as vice president of investor relations and corporate communications at Pharmasset, Inc. from October 2008 until January 2012, when Pharmasset was acquired by Gilead Sciences. From May 2004 through August 2008, Dr. Smith was an equity research analyst at J.P. Morgan Securities covering biotechnology companies. Dr. Smith received his B.Sc. in Applied Zoology from the University of Leeds, his M.Sc. in Toxicology from the University of Surrey and his Ph.D. in Clinical Virology from the University of Oxford.

**Faraz Ali** has served as our vice president and head of program management and commercial development since May 2011. In 2011, he served as a consultant to Third Rock Ventures, L.P. From 2001 to 2010, Mr. Ali held a number of positions at Genzyme, including most recently, senior director of U.S. marketing and strategic planning of the personalized genetic health business unit from August 2006 to December 2010. Mr. Ali received his B.S. in electrical engineering from Stanford University and his M.B.A. from Harvard Business School.

**Cyrus Mozayeni, M.D.** has served as our senior director of business development since June 2010. Previously, he served as director of strategic/business development at PPD Dermatology (Magen Biosciences, Inc. until April 2009) from April 2007 to May 2010. Dr. Mozayeni received his B.S. in neuroscience from Brown University, his M.D. from the University of Virginia School of Medicine and his M.B.A. from the Kellogg Graduate School of Management at Northwestern University.

**Kathleen Wilkinson** has served as our senior director of human resources since November 2012. Previously, she served as human resources director of Adnexus Therapeutics from September 2009 to November 2012, consulted with Codon Devices Inc. from February 2009 to April 2009 and served as senior human resources director of Codon Devices from June 2007 to February 2009. Ms. Wilkinson received her B.A. in sociology from Harvard University.

**Wendy L. Dixon, Ph.D.** has served as a member of our board of directors since April 2013. In 2012, Dr. Dixon was a principal at Great Meadow Consulting LLC and in 2010, she served as senior

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advisor at The Monitor Group. Since 2005, Dr. Dixon has advised and consulted and in some instances served as a member of the board of director for a number of biopharmaceutical companies, including Alkermes PLC, Incyte Corporation, Orexigen Therapeutics, Furiex Pharmaceuticals and formerly on Ardea Biosciences, Inc. (sold to AstraZeneca PLC in 2012) and Dentsply International. Dr. Dixon also served as Chief Marketing Officer and President of Global Marketing for Bristol-Myers Squibb and as a member of the CEO's Executive Committee from 2001 to 2009. She has had an over 30-year career in the pharmaceutical and biotechnology business, combining a technical background and experience in drug development and regulatory affairs with commercial responsibilities in building and leading organizations and launching and growing more than 20 pharmaceutical products including Tagamet, Fosamax, Singulair, Plavix, Abilify, Reyataz and Baraclude. From 1996 to 2001, she was Senior Vice President Marketing at Merck and prior to that she held executive management positions at West Pharmaceuticals, Osteotech and Centocor, and various positions at SmithKline and French (now GlaxoSmithKline) in marketing, regulatory affairs, project management and as a biochemist. Dr. Dixon received her B.Sc., M.Sc. and Ph.D. from the University of Cambridge (UK). We believe that, among other experience, qualifications, attributes and skills, Dr. Dixon's technical background in drug development, commercialization, marketing and regulatory affairs qualify her to serve as a member of our board of directors.

**Steven Gillis, Ph.D.** has served as a member of our board of directors since April 2011. Since 2005, Dr. Gillis has been a managing director at ARCH Venture Partners, a venture capital firm. From 1994 to 2005, Dr. Gillis served as chief executive officer and chairman of the board of directors of Corixa Corporation, which he co-founded in October 1994. Previously, Dr. Gillis served as a director, head of research and development, chief scientific officer and acting chief executive officer of Immunex Corporation, which he co-founded. As a former director and chairman of Trubion Pharmaceuticals, Inc., Dr. Gillis led its acquisition by Emergent BioSolutions in the fall of 2010. Dr. Gillis currently serves as a director of Accelerator Corporation, Allozyne, Inc., Pulmatrix, Inc., VLST Corporation and VBI Vaccines and serves as director and chairman of VentiRX Pharmaceuticals, Inc., Theraclone Sciences, Inc., Lycera Corp. and PhaseRx, Inc. Dr. Gillis received his B.A. in biology and English from Williams College and his Ph.D. in biological science from Dartmouth College. We believe that Dr. Gillis's experience in the venture capital industry, particularly with biotech and pharmaceutical companies, combined with his experience in molecular and tumor immunology, qualify him to serve as a member of our board of directors.

**Daniel S. Lynch** has served as chairman of our board of directors since May 2011, when he joined Third Rock Ventures, L.P., or Third Rock, as an entrepreneur-in-residence. Since October 2007, Mr. Lynch has advised and served as executive chair or member of the board of directors for a number of private biopharmaceutical companies, which include Stromedix, Inc. (until its acquisition by Biogen Idec in February 2012), Avila Therapeutics, Inc. (until its acquisition by Celgene Corporation in February 2012), BIND Biosciences, Inc., RaNA Therapeutics, Inc., Nimbus Discovery, LLC, Edimer Pharmaceuticals, Ember Therapeutics, Inc. and Blueprint Medicines, Inc. Previously, Mr. Lynch served as chief executive and chief financial officer of ImClone Systems Corporation, or ImClone. As ImClone's chief executive officer, he led ImClone through a significant turnaround, helping to restore the company's reputation and to secure FDA approval of ERBITUX (Cetuximab), a novel cancer treatment. As its chief financial officer, Mr. Lynch led negotiations to form the major partnership between ImClone and Bristol-Myers Squibb. Earlier in his career, he served in various financial positions at Bristol-Myers Squibb over a 15-year tenure. He served on the board of directors and the audit committee of U.S. Oncology, Inc. for five years until December 2010, when it was acquired by McKesson. Mr. Lynch received his B.A. in mathematics from Wesleyan University and his M.B.A. from

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the Darden Graduate School of Business Administration at the University of Virginia. We believe that Mr. Lynch's experience as chief executive officer and chief financial officer of a public pharmaceutical company and as executive chairman and director for many other life science companies, qualify him to serve as a member of our board of directors.

**John M. Maraganore, Ph.D.** has served as a member of our board of directors since January 2012. Since December 2002, Dr. Maraganore has served as the chief executive officer and as a director of Alnylam Pharmaceuticals, Inc. From December 2002 to December 2007, Dr. Maraganore served as president of Alnylam. From April 2000 to December 2002, Dr. Maraganore served as senior vice president, strategic product development with Millennium Pharmaceuticals, Inc. Before Millennium, he served as director of molecular biology and director of market and business development at Biogen, Inc. (now Biogen Idec, Inc.). Prior to Biogen, Dr. Maraganore was a scientist at ZymoGenetics, Inc., and The Upjohn Company. Dr. Maraganore is also chairman of Regulus Therapeutics, Inc. and a director for Agios Pharmaceuticals and Tempero Pharmaceuticals. In addition, he is an advisor to Third Rock Ventures, L.P. He is also a member of the Immunology Advisory Council of Harvard Medical School and a member of the Biotechnology Industry Organization Board. Dr. Maraganore holds a B.A. in biological sciences from the University of Chicago and an M.S. and a Ph.D. in biochemistry and molecular biology from the University of Chicago. We believe that Dr. Maraganore's experience as chief executive officer and president of a public pharmaceutical company and chairman of another pharmaceutical company that just went public, qualify him to serve as a member of our board of directors.

**Geert-Jan Mulder, M.D.** has served as a member of our board of directors since May 2004. Dr. Mulder has been a general partner at Forbion Capital Partners since 2001. Prior to joining ABN AMRO Capital Life Sciences (now Forbion), he was clinical research manager of Byk Gulden (now Takeda) from 1999 to 2001 where his group was responsible for design and execution of early and late-stage clinical trials forming the basis for two global product registrations, Daxas and Alvesco in fields of COPD and asthma. For both products he was a member of the Global Medical Marketing group and supported the line extension program of Pantozol. From 1998 to 1999, he served as medical adviser in the field of arthritis and pain (COX-2 technology) and worked on the local and European positioning of Celebrex at Searle (now Pfizer). In addition to taking an active role in the Forbion Capital Partners investment process, Dr. Mulder serves on a number of boards and assists portfolio companies in their clinical development programs and overall strategy including Exosom Diagnostics, Inc., Pansgenetics B.V., Promedior, Inc. and Provesica, Ltd. He previously served on the board of Transave Inc. until its merger with Insmed in December 2010 and Acorda Therapeutics Inc. until its initial public offering in February 2006. Dr. Mulder is a certified Pharmaceutical Physician and earned both a masters in medicine and an M.D. from University of Utrecht. Before joining the pharmaceutical industry, he served as a resident in the field of obstetrics and gynecology. We believe that Dr. Mulder's experience in the venture capital industry, particularly with biotech and pharmaceutical companies, combined with his experience in clinical development, regulatory filings, Special Protocol Approval, orphan designations and dual experience in both the United States and Europe, qualify him to serve as a member of our board of directors. Dr. Mulder has indicated to us his intention to resign from our board of directors upon the consummation of this offering.

**Dr. Axel Polack**, has served as a member of our board of directors since May 2007. Dr. Polack joined TVM Capital in 2000 and is a general partner for life sciences in the firm's Munich office. He currently serves on the board of Noxxon Pharma AG, Invendo Medical, f-star and Probiodrug AG. Dr. Polack's main scientific fields of expertise are molecular and viral oncology, oncogene

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activation, gene regulation and molecular immunology. He works intensively on the assessment of new investment opportunities in those areas, while also providing support to existing portfolio companies. Before joining TVM Capital, Dr. Polack was general manager of Innovative Technologies Neuherberg GmbH (now Ascenion). Ascenion acts as a marketing partner to research institutions of the Helmholtz-Gemeinschaft, such as GSF—National Research Center for Environment and Health GmbH, which licenses patents and fosters start-up companies. In the eight years prior to joining Ascenion, Dr. Polack was the deputy head of the GSF—Institute of Clinical Molecular Biology. He holds a M.D. from the University of Freiburg and a Second Thesis (postdoctoral lecture qualification "Habilitation") in the field of virology. Dr. Polack's doctoral thesis was honored with the Goedecke Research prize for outstanding fundamental research in medicine. In 1995, he was appointed assistant professor/private lecturer by the Ludwig-Maximilian-University in Munich. Since 1984, he has co-authored more than 50 publications in peer review journals. We believe that Dr. Polack's experience in the venture capital industry, particularly with biotech and pharmaceutical companies, combined with his experience in virology, gene regulation, qualify him to serve as a member of our board of directors.

**David P. Schenkein, M.D.** has served as a member of our board of directors since April 2013. Since August 2009, Dr. Schenkein has served as the chief executive officer of Agios Pharmaceuticals. From April 2006 to July 2009, Dr. Schenkein served as senior vice president of oncology development and Genentech. Dr. Schenkein is also a director for Agios Pharmaceuticals, Foundation Medicine and Blueprint Medicine. Dr. Schenkein received his B.A. in chemistry from Wesleyan University and his M.D. from Upstate Medical School. We believe that Dr. Schenkein's experience as chief executive officer of Agios and his membership on the board of directors of a number of biopharmaceutical companies qualify him to serve as a member of our board of directors.

**Robert I. Tepper, M.D.** has served as a member of our board of directors since September 2010. Dr. Tepper is a distinguished scientist with over 25 years of experience building and operating leading R&D operations. Dr. Tepper co-founded Third Rock Ventures, L.P. in March 2007 and focuses on the formation, development and scientific strategy of the portfolio companies, as well as actively identifying and evaluating new investments. He also assumes active leadership roles in Third Rock's portfolio companies, functioning as chief scientific officer through the first 12-18 months post launch. Prior to joining Third Rock Ventures, L.P., Dr. Tepper served as president of research and development Millennium Pharmaceuticals, or Millenium, from 2003 to 2007 and was vital in its expansion from a drug discovery company to a fully integrated biopharmaceutical company. Before joining Millennium in 1994, he served as principal investigator in the laboratory of tumor biology at Massachusetts General Hospital Cancer Center. Dr. Tepper is also a founder and former member of the scientific advisory board of Cell Genesys/Abgenix. Dr. Tepper holds an A.B. in biochemistry from Princeton University and an M.D. from Harvard Medical School. Dr. Tepper serves as an adjunct faculty member at Harvard Medical School and Massachusetts General Hospital and is an advisory board member of several leading healthcare institutions, including the Partners HealthCare Center for Personalized Genetic Medicine, Harvard Medical School and Tufts Medical School. Dr. Tepper is a board member of Alcresta, Allena Pharmaceuticals, Cerulean Pharma Inc., Constellation Pharmaceuticals Inc. and Kala Pharmaceuticals, Inc. and is also on the board of overseers at Tufts University. We believe that Dr. Tepper's experience in the venture capital industry, particularly with biotech and pharmaceutical companies, combined with his experience building and operating research and development operations and as faculty and advisory board members of several healthcare institutions, qualify him to serve as a member of our board of directors.



## **Board composition**

We currently have nine directors, all of whom were elected pursuant to the terms of our voting agreement, which will terminate upon completion of this offering. Upon the termination of these provisions, we will not be bound by contractual obligations regarding the election of our directors.

Effective upon the closing of this offering, we will divide the terms of office of the directors into three classes:

- Class I, whose term will expire at the annual meeting of stockholders to be held in 2014;
- Class II, whose term will expire at the annual meeting of stockholders to be held in 2015; and
- Class III, whose term will expire at the annual meeting of stockholders to be held in 2016.

Upon the closing of this offering, Class I shall consist of Dr. Gillis, Mr. Leschly and Dr. Polack, Class II shall consist of Mr. Lynch, Dr. Maraganore and Dr. Tepper and Class III shall consist of Dr. Dixon and Dr. Schenkein. Dr. Mulder, currently a member of our board of directors, has indicated to us his intention to resign from our board of directors upon the consummation of this offering. At each annual meeting of stockholders after the initial classification, the successors to directors whose terms will then expire shall serve from the time of election and qualification until the third annual meeting following election and until their successors are duly elected and qualified. A resolution of the board of directors may change the authorized number of directors. Any additional directorships resulting from an increase in the number of directors will be distributed among the three classes so that, as nearly as possible, each class will consist of one-third of the directors. This classification of the board of directors may have the effect of delaying or preventing changes in control or management of our company.

Following the closing of this offering, our nominating and corporate governance committee and board of directors may consider a broad range of factors relating to the qualifications and background of nominees, which may include diversity and is not limited to race, gender or national origin. We have no formal policy regarding board diversity. Our nominating and corporate governance committee's and board of directors' priority in selecting board members is identification of persons who will further the interests of our company through his or her established record of professional accomplishment, the ability to contribute positively to the collaborative culture among board members, and professional and personal experiences and expertise relevant to our growth strategy.

## **Board committees**

Our board of directors has established three standing committees: the audit committee, the compensation committee and the nominating and corporate governance committee.

### ***Audit committee***

Effective upon this offering, our audit committee will be composed of Dr. Dixon, Dr. Gillis and Mr. Lynch, with Dr. Gillis serving as chairman of the committee. Our board of directors has determined that each member of the audit committee meets the independence requirements of Rule 10A-3 under the Exchange Act and the applicable listing standards of Nasdaq. Our board of directors

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has determined that Mr. Lynch is an “audit committee financial expert” within the meaning of the SEC regulations and applicable listing standards of Nasdaq. The audit committee’s responsibilities upon completion of this offering will include:

- appointing, approving the compensation of, and assessing the independence of our independent registered public accounting firm;
- approving audit and permissible non-audit services, and the terms of such services, to be provided by our independent registered public accounting firm;
- reviewing the internal audit plan with the independent registered public accounting firm and members of management responsible for preparing our financial statements;
- reviewing and discussing with management and the independent registered public accounting firm our annual and quarterly financial statements and related disclosures as well as critical accounting policies and practices used by us;
- reviewing the adequacy of our internal control over financial reporting;
- establishing policies and procedures for the receipt and retention of accounting-related complaints and concerns;
- recommending, based upon the audit committee’s review and discussions with management and the independent registered public accounting firm, whether our audited financial statements shall be included in our Annual Report on Form 10-K;
- monitoring the integrity of our financial statements and our compliance with legal and regulatory requirements as they relate to our financial statements and accounting matters;
- preparing the audit committee report required by the rules of the Securities and Exchange Commission, or SEC, to be included in our annual proxy statement;
- reviewing all related party transactions for potential conflict of interest situations and approving all such transactions; and
- reviewing quarterly earnings releases and scripts.

**Compensation committee**

Effective upon this offering, our compensation committee will be composed of Mr. Lynch, Dr. Maraganore and Dr. Tepper, with Mr. Lynch serving as chairman of the committee. Our board of directors has determined each member of the compensation committee is “independent” as defined under the applicable listing standards of Nasdaq. The compensation committee’s responsibilities upon completion of this offering will include:

- annually reviewing and approving corporate goals and objectives relevant to the compensation of our chief executive officer;
- evaluating the performance of our chief executive officer in light of such corporate goals and objectives and determining the compensation of our chief executive officer;
- reviewing and approving the compensation of our other executive officers;
- appointing, compensating and overseeing the work of any compensation consultant, legal counsel or other advisor retained by the compensation committee;

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- conduct the independence assessment outlined in Nasdaq rules with respect to any compensation consultant, legal counsel or other advisor retained by the compensation committee;
- annually review and reassess the adequacy of the committee charter in its compliance with the listing requirements of Nasdaq;
- reviewing and establishing our overall management compensation, philosophy and policy;
- overseeing and administering our compensation and similar plans;
- reviewing and approving our policies and procedures for the grant of equity-based awards;
- reviewing and making recommendations to the board of directors with respect to director compensation;
- reviewing and discussing with management the compensation discussion and analysis to be included in our annual proxy statement or Annual Report on Form 10-K; and
- reviewing and discussing with the board of directors corporate succession plans for the chief executive officer and other key officers.

### ***Nominating and corporate governance committee***

Effective upon this offering, our nominating and corporate governance committee will be composed of Dr. Polack and Dr. Schenkein, with Dr. Polack serving as chairman of the committee. Our board of directors has determined that each member of the nominating and corporate governance committee is “independent” as defined under the applicable listing standards of Nasdaq. The nominating and corporate governance committee’s responsibilities upon completion of this offering will include:

- developing and recommending to the board of directors criteria for board and committee membership;
- establishing procedures for identifying and evaluating board of director candidates, including nominees recommended by stockholders;
- identifying individuals qualified to become members of the board of directors;
- recommending to the board of directors the persons to be nominated for election as directors and to each of the board’s committees;
- developing and recommending to the board of directors a set of corporate governance guidelines; and
- overseeing the evaluation of the board of directors and management.

Our board of directors may establish other committees from time to time.

### **Leadership structure and risk oversight**

Our board of directors is currently chaired by Mr. Lynch. As a general policy, our board of directors believes that separation of the positions of chairman and chief executive officer reinforces the independence of the board of directors from management, creates an environment that

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encourages objective oversight of management's performance and enhances the effectiveness of the board of directors as a whole. As such, Mr. Leschly serves as our president and chief executive officer while Mr. Lynch serves as our chairman of the board of directors but is not an officer.

Our board of directors oversees the management of risks inherent in the operation of our business and the implementation of our business strategies. Our board of directors performs this oversight role by using several different levels of review. In connection with its reviews of the operations and corporate functions of our company, our board of directors addresses the primary risks associated with those operations and corporate functions. In addition, our board of directors reviews the risks associated with our company's business strategies periodically throughout the year as part of its consideration of undertaking any such business strategies.

Each of our board committees also oversees the management of our company's risk that falls within the committee's areas of responsibility. In performing this function, each committee has full access to management, as well as the ability to engage advisors. Our vice president of finance reports to the audit committee and is responsible for identifying, evaluating and implementing risk management controls and methodologies to address any identified risks. In connection with its risk management role, our audit committee meets privately with representatives from our independent registered public accounting firm and our chief financial officer. The audit committee oversees the operation of our risk management program, including the identification of the primary risks associated with our business and periodic updates to such risks, and reports to our board of directors regarding these activities.

### **Compensation committee interlocks and insider participation**

None of the members of our compensation committee has at any time during the prior three years been one of our officers or employees. None of our executive officers currently serves, or in the past fiscal year has served, as a member of the board of directors or compensation committee of any entity that has one or more executive officers serving on our board of directors or compensation committee. For a description of transactions between us and members of our compensation committee and affiliates of such members, please see "Certain relationships and related party transactions."

### **Code of business conduct and ethics**

We have adopted a code of business conduct and ethics that applies to all of our employees, officers and directors, including those officers responsible for financial reporting. Upon the closing of this offering, our code of business conduct and ethics will be available on our website. We intend to disclose any amendments to the code, or any waivers of its requirements, on our website.

## Executive and director compensation

### 2012 summary compensation table

The following table sets forth the compensation earned during the fiscal year ended December 31, 2012 to our chief executive officer and our next two highest-paid executive officers as of December 31, 2012. We refer to these officers as our named executive officers.

Name and Principal Position	Year	Salary(\$)	Bonus\$(1)	Option awards\$(2)	Non-equity incentive plan compensation\$(3)	Total(\$)
Nick Leschly <i>President and Chief Executive Officer</i>	2012	346,085	—	130,738	124,200	601,023
Jeffrey T. Walsh <i>Chief Operating Officer</i>	2012	300,758	—	—	108,000	408,758
David M. Davidson, MD <i>Chief Medical Officer</i>	2012	260,456	45,000	223,857	82,156	611,469

(1) The amount reported consists of Dr. Davidson's signing bonus.

(2) The amounts reported in the Option awards column represent the grant date fair value of the stock options granted to our named executive officers during 2012 as computed in accordance with Accounting Standards Codification, or ASC, Topic 718, not including any estimates of forfeitures. The assumptions used in calculating the grant date fair value of the stock options reported in the Option awards column are set forth in Note 12 to our consolidated financial statements included elsewhere in this prospectus. Note that the amounts reported in this column reflect the accounting cost for these stock options, and do not correspond to the actual economic value that may be received by the named executive officers from the options.

(3) Amounts represent cash bonuses earned in 2012, and paid during 2013, based on achievement of performance goals and other factors deemed relevant by our board of directors. Our 2012 company objectives related primarily to clinical development and partnering achievements.

### Narrative disclosure to summary compensation table

#### *Employment arrangements with our named executive officers*

**Nick Leschly.** We expect to enter into an amended and restated employment agreement, effective as of the closing of this offering, with Nick Leschly for the position of president and chief executive officer. Mr. Leschly currently receives a base salary of \$390,000, which is subject to adjustment at the discretion of the board of directors. Mr. Leschly is also eligible for an annual performance bonus of up to 50% of his base salary, payable at the discretion of the board of directors. Mr. Leschly is eligible to participate in our employee benefit plans, subject to the terms of those plans.

**Jeffrey T. Walsh.** We expect to enter into an amended and restated employment agreement, effective as of the closing of this offering, with Jeffrey T. Walsh for the position of chief operating officer. Mr. Walsh currently receives a base salary of \$320,000, which is subject to adjustment at the discretion of the board of directors. Mr. Walsh is also eligible for an annual performance bonus of up to 40% of his base salary, payable at the discretion of the board of directors. Mr. Walsh is eligible to participate in our employee benefit plans, subject to the terms of those plans.

**David M. Davidson, M.D.** We expect to enter into an amended and restated employment agreement, effective as of the closing of this offering, with David M. Davidson, M.D. for the position of chief medical officer. Dr. Davidson currently receives a base salary of \$315,000, which is subject to adjustment at the discretion of the board of directors. Dr. Davidson is also eligible for an annual performance bonus of up to 35% of his base salary, payable at the discretion of the board of directors. Dr. Davidson is eligible to participate in our employee benefit plans, subject to the terms of those plans.

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These employment agreements also contain provisions that provide for certain payments and benefits in the event of an involuntary termination of employment. In addition, the named executive officers may be entitled to accelerated vesting of their outstanding and unvested awards in certain circumstances. The information below describes certain compensation that may become due payable as a result of certain events. These payments and benefits are in addition to benefits available generally to salaried employees, including distributions under our Section 401(k) plan, accrued benefits under our health and welfare plans and arrangements and vacation pay or other accrued benefits under our medical and dental insurance plans, that are not generally described. Outstanding equity awards for the named executive officers as of December 31, 2012 are set forth under “Outstanding equity awards at December 31, 2012.”

***Involuntary termination of employment***

Pursuant to their employment agreements, each named executive officer is eligible to receive certain payments and benefits in the event his employment is terminated by us without “cause” (as defined in his offer letter) or in the event he terminates his employment with “good reason” (as defined in his offer letter). Upon the timely execution of a severance agreement, including a general release of claims, each named executive officer is eligible to receive the following payments and benefits:

- 12 months of base salary continuation; and
- if he elects to continue his group healthcare benefits, to the extent authorized by and consistent with COBRA, we will pay the named executive officer a monthly cash payment equal to the monthly employer contribution we would have made to provide him health insurance if he had remained employed by us until the earlier of (1) 12 months following the date of termination or (2) the end of the named executive officer’s COBRA health continuation period.

***Sale event***

Pursuant to the employment agreements and the award agreements governing equity awards granted to the named executive officers prior to the date of the employment agreements, in the event of a “sale event” of the company (as defined in the 2010 Stock Option and Grant Plan), any such unvested stock options or other stock-based awards will immediately accelerate, vest and become fully exercisable or non-forfeitable as of the effective date of the sale event.

In addition, in the event that any of the named executive officers terminates his employment with us for good reason or his employment with us is terminated by us without cause, in each case within 12 months following a “sale event” (as defined in the 2013 Stock Option and Incentive Plan), he will be entitled to receive the following payments and benefits upon the timely execution of a severance agreement, including a general release of claims:

- a lump sum cash payment equal to one times (or one and a half times in the case of Mr. Leschly) the sum of (1) the named executive officer’s then-current base salary (or base salary in effect immediately prior to the sale event, if higher) and (2) the named executive officer’s target annual incentive compensation; and
- if he elects to continue his group healthcare benefits, to the extent authorized by and consistent with COBRA, we will pay the named executive officer a monthly cash payment

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equal to the monthly employer contribution we would have made to provide him health insurance if he had remained employed by us until the earlier of (1) 12 months (or 18 months in the case of Mr. Leschly) following the date of termination or (2) the end of the named executive officer's COBRA health continuation period; and

- all stock options and other stock-based awards granted to the named executive officer after the date of his employment agreement will become fully exercisable and non-forfeitable as of the date of the named executive officer's termination.

**Definitions**

For purposes of Mr. Leschly's employment agreement, "cause" means his:

- commission of any felony or commission of any crime involving fraud, dishonesty or moral turpitude;
- commission or attempted commission of, or participation in, a fraud or act of dishonesty against us;
- material breach of any contract between Mr. Leschly and us or material breach of any legal duty Mr. Leschly owes to us;
- conduct that constitutes insubordination, incompetence or neglect of duties; or
- failure to perform the duties, functions and responsibilities of his position.

For purposes of each of the employment agreements with Mr. Walsh and Dr. Davidson, "cause" means the named executive officer's:

- dishonest statements or acts with respect to us or any of our affiliates, or any of our current or prospective customers, suppliers, vendors or other third parties with which such entity does business;
- commission of any felony or any misdemeanor involving moral turpitude, deceit, dishonesty or fraud;
- failure to perform assigned duties to our reasonable satisfaction, which failure continues, in our reasonable judgment, after written notice to the named executive officer;
- gross negligence, willful misconduct or insubordination with respect to us or any of our affiliates; or
- violation of any provision of any agreement(s) between the named executive officer and us relating to noncompetition, nondisclosure and/or assignment of inventions.

For purposes of the each of the employment agreements with the named executive officers, "good reason" means:

- a material diminution in the named executive officer's responsibilities, authority and function;
- a material reduction in base salary other than pursuant to a salary reduction program affecting substantially all of our employees (or senior executives in the case of Dr. Davidson) that does not adversely affect the named executive officer to a greater

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extent than other similarly situated employees; provided, however, that any reduction in base salary that exceeds 10% of the named executive officer's then-current base salary shall constitute good reason;

- a material change in the geographic location (of more than 30 miles in the case of Mr. Walsh) at which the named executive officer must regularly report to work or perform services, except for required travel on business (for Mr. Walsh and Dr. Davidson, to an extent substantially consistent with usual business travel obligations); and
- a material breach by us of any provision of our equity incentive plans or award agreements thereunder or any other material agreement between the named executive officer and us concerning the terms of the named executive officer's employment, benefits or compensation.

In addition, under Mr. Leschly's employment agreement, "good reason" also includes:

- an adverse change in the his job title or a change in reporting relationship as a result of which he no longer reports to our board of directors; and
- removal from, or failure to be elected to, our board of directors.

## Equity compensation

### Outstanding equity awards at December 31, 2012

The following table sets forth information concerning the outstanding equity awards held by each of the named executive officers as of December 31, 2012.

Name	Number of securities underlying unexercised options exercisable (#)	Number of securities underlying unexercised options unexercisable (#)	Number of securities underlying unexercised unearned options (#)	Option awards		Stock awards	
				Option exercise price (\$/share)	Option expiration date	Number of shares that have not vested (#)	Market value of shares that have not vested (\$)(1)
Nick Leschly	—	1,750,000(2)	—	\$ 0.11	6/4/2022	—	—
	1,023,941	1,433,518(3)	—	0.11	7/13/2021	—	—
						2,862,304(4)	\$
Jeffrey T. Walsh	—	—	497,060(5)	0.11	7/13/2021	—	—
	1,721,588	2,627,687(6)	—	0.11	7/13/2021	—	—
David M. Davidson, M.D.	—	2,982,369(7)	—	0.11	4/13/2022	—	—

(1) There was no public market for our common stock at December 31, 2012. We have estimated the market value of the unvested stock awards assuming an initial public offering price of \$ per share, the midpoint of the price range set forth on the cover of this prospectus.

(2) Represents options to purchase shares of our common stock granted on June 4, 2012. The shares underlying these options vest as follows: 25% vest on May 1, 2013, with the remainder of the shares vesting in equal monthly installments over the following three years through May 1, 2016. Vesting of all unvested shares shall accelerate in connection with an acquisition event pursuant to the terms of the option agreement.

(3) Represents options to purchase shares of our common stock granted on July 13, 2011. The shares underlying these options vest as follows: 25% vest on April 15, 2012, with the remainder of the shares vesting in equal monthly installments over the following three years through April 15, 2015. Vesting of all unvested shares shall accelerate in connection with an acquisition event pursuant to the terms of the option agreement.

(4) Under the terms of Mr. Leschly's November 15, 2010 restricted stock agreement, the remaining unvested shares will vest in equal monthly installments through October 1, 2014. Vesting of all restricted shares shall accelerate in connection with an acquisition event pursuant to the terms of the restricted stock agreement.



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- (5) Represents options to purchase shares of our common stock granted on July 13, 2011. The shares underlying these options vest as follows: 25% vest upon the one-year anniversary of the achievement of a performance-goal, with the remainder of the shares vesting in equal monthly installments over the following three years thereafter. Vesting of all unvested shares shall accelerate in connection with an acquisition event pursuant to the terms of the option agreement.
- (6) Represents options to purchase shares of our common stock granted on July 13, 2011. The shares underlying these options vests as follows: 25% vest on May 16, 2012, with the remainder of the shares vesting in equal monthly installments over the following three years through May 16, 2015. Vesting of all unvested shares shall accelerate in connection with an acquisition event pursuant to the terms of the option agreement.
- (7) Represents options to purchase shares of our common stock granted on April 13, 2012. The shares underlying these options vests as follows: 25% vest on February 13, 2013, with the remainder of the shares vesting in equal monthly installments over the following three years through February 13, 2016. Vesting of all unvested shares shall accelerate in connection with an acquisition event pursuant to the terms of the option agreement.

## Director compensation

The following table sets forth a summary of the compensation we paid to our non-employee directors during 2012. Other than as set forth in the table and described more fully below, we did not pay any compensation, reimburse any expense of, make any equity awards or non-equity awards to, or pay any other compensation to any of the other non-employee members of our board of directors in 2012. Mr. Leschly, our president and chief executive officer, receives no compensation for his service as a director, and, consequently, is not included in this table. The compensation received by Mr. Leschly as an employee during 2012 is presented in "2012 summary compensation table" above.

<b>Name(1)</b>	<b>Fees earned or paid in cash(\$)</b>	<b>Option awards(\$)(2)</b>	<b>Total(\$)</b>
Daniel S. Lynch	50,000	—	50,000
John M. Maraganore, Ph.D.	30,000	46,463	76,463

- (1) As of December 31, 2012, Mr. Lynch and Dr. Maraganore held options to purchase 1,242,500 and 637,869 shares of common stock, respectively. None of the other non-employee members of our board of directors held options to purchase common stock or any other unvested share-based awards as of that date.
- (2) The amounts reported in the Option Awards column represent the grant date fair value of the stock options granted to our non-employee directors during 2012 as computed in accordance with ASC Topic 718, not including any estimates of forfeitures. The assumptions used in calculating the grant date fair value of the stock options reported in the Option Awards column are set forth in Note 12 to our consolidated financial statements included elsewhere in this prospectus. Note that the amounts reported in this column reflect the accounting cost for these stock options, and do not correspond to the actual economic value that may be received by the non-employee directors from the options.

We have entered into offer letters with Mr. Lynch and Drs. Maraganore, Dixon and Schenkein regarding their service on our board of directors, which provide for annual cash retainers and reimbursement of expenses related to service as directors. These offer letters will terminate prior to the effectiveness of this registration statement, and each of these directors will be eligible to participate in the non-employee director compensation program described below following this offering. Each of these directors was granted an option to purchase shares of our common stock in connection with their appointment to the board of directors.

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Our board of directors has adopted a non-employee director compensation policy, effective as of the closing of this offering, that is designed to provide a total compensation package that enables us to attract and retain, on a long-term basis, high caliber non-employee directors. Under the policy, all non-employee directors will be paid cash compensation from and after the completion of this offering, as set forth below:

	<b>Annual Retainer</b>
<b>Board of Directors:</b>	
All non-employee members	\$ 35,000
Additional retainer for Non-Executive Chairman of the Board	\$ 25,000
<b>Audit Committee:</b>	
Chairman	\$ 15,000
Non-Chairman members	\$ 7,500
<b>Compensation Committee:</b>	
Chairman	\$ 10,000
Non-Chairman members	\$ 5,000
<b>Nominating and Corporate Governance Committee:</b>	
Chairman	\$ 7,000
Non-Chairman members	\$ 3,000

Under the non-employee director compensation policy, each person who is initially appointed or elected to the board of directors will be eligible for an option grant to purchase up to 260,000 shares of our common stock under our stock option plan on the date he or she first becomes a non-employee director, which will vest annually over a three-year period. In addition, on the date of the annual meeting of stockholders, each continuing non-employee director who has served on the board of directors for a minimum of six months will be eligible to receive an annual option grant to purchase up to 130,000 shares of our common stock, which will vest in full upon the earlier of the first anniversary of the date of grant or the date of the following annual meeting of stockholders. All of the foregoing options will be granted at fair market value on the date of grant.

#### ***Compensation risk assessment***

We believe that our executive compensation program does not encourage excessive or unnecessary risk taking. This is primarily due to the fact that our compensation programs are designed to encourage our executive officers and other employees to remain focused on both short-term and long-term strategic goals, in particular in connection with our pay-for-performance compensation philosophy. As a result, we do not believe that our compensation programs are reasonably likely to have a material adverse effect on us.

#### **Equity compensation plans and other benefit plans**

##### ***2013 Stock Option and Incentive Plan***

Our 2013 Stock Option and Incentive Plan, or the 2013 Plan, was adopted by our board of directors and approved by our stockholders in 2013 and will become effective immediately prior to this offering. The 2013 Plan will replace the 2010 Plan (as defined below).

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We have initially reserved \_\_\_\_\_ shares of our common stock for the issuance of awards under the 2013 Plan. The 2013 Plan provides that the number of shares reserved and available for issuance under the plan will automatically increase each January 1, beginning on January 1, 2014, by \_\_\_\_\_ % of the outstanding number of shares of our common stock on the immediately preceding December 31 or such lesser number of shares as determined by our compensation committee. This number is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

The shares we issue under the 2013 Plan will be authorized but unissued shares or shares that we reacquire. The shares of common stock underlying any awards that are forfeited, cancelled, held back upon exercise or settlement of an award to satisfy the exercise price or tax withholding, reacquired by us prior to vesting, satisfied without any issuance of stock, expire or are otherwise terminated (other than by exercise) under the 2013 Plan are added back to the shares of common stock available for issuance under the 2013 Plan.

Stock options and stock appreciation rights with respect to no more than \_\_\_\_\_ shares of stock may be granted to any one individual in any one calendar year and the maximum "performance-based award" payable to any one individual under the 2013 Plan is \_\_\_\_\_ shares of stock or \$ \_\_\_\_\_ in the case of cash-based awards. No more than \_\_\_\_\_ shares may be issued as incentive stock options in any one calendar year period.

The 2013 Plan will be administered by our compensation committee. Our compensation committee has full power to select, from among the individuals eligible for awards, the individuals to whom awards will be granted, to make any combination of awards to participants, and to determine the specific terms and conditions of each award, subject to the provisions of the 2013 Plan. Persons eligible to participate in the 2013 Plan will be those full or part-time officers, employees, non-employee directors and other key persons (including consultants) as selected from time to time by our compensation committee in its discretion.

The 2013 Plan permits the granting of both (1) options to purchase common stock intended to qualify as incentive stock options under Section 422 of the Internal Revenue Code of 1986, as amended, or the Code, and (2) options that do not so qualify. The option exercise price of each option will be determined by our compensation committee but may not be less than 100% of the fair market value of our common stock on the date of grant. The term of each option will be fixed by our compensation committee and may not exceed ten years from the date of grant. Our compensation committee will determine at what time or times each option may be exercised.

Our compensation committee may award stock appreciation rights subject to such conditions and restrictions as it may determine. Stock appreciation rights entitle the recipient to shares of common stock, or cash, equal to the value of the appreciation in our stock price over the exercise price. The exercise price of each stock appreciation right may not be less than 100% of fair market value of the common stock on the date of grant.

Our compensation committee may award restricted shares of common stock and restricted stock units to participants subject to such conditions and restrictions as it may determine. These conditions and restrictions may include the achievement of certain performance goals and/or continued employment with us through a specified vesting period. Our compensation committee may also grant shares of common stock that are free from any restrictions under the 2013 Plan. Unrestricted stock may be granted to participants in recognition of past services or for other valid consideration and may be issued in lieu of cash compensation due to such participant.

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Our compensation committee may grant performance share awards to participants that entitle the recipient to receive share awards of common stock upon the achievement of certain performance goals and such other conditions as our compensation committee shall determine. Our compensation committee may grant dividend equivalent rights to participants that entitle the recipient to receive credits for dividends that would be paid if the recipient had held a specified number of shares of common stock.

Our compensation committee may grant cash bonuses under the 2013 Plan to participants, subject to the achievement of certain performance goals.

Our compensation committee may grant awards of restricted stock, restricted stock units, performance shares or cash-based awards under the 2013 Plan that are intended to qualify as “performance-based compensation” under Section 162(m) of the Code. Those awards would only vest or become payable upon the attainment of performance goals that are established by our compensation committee and related to one or more performance criteria. The performance criteria that could be used with respect to any such awards include: total shareholder return, earnings before interest, taxes, depreciation and amortization, net income (loss) (either before or after interest, taxes, depreciation and/or amortization), changes in the market price of our common stock, economic value-added, funds from operations or similar measure, sales or revenue, development, clinical or regulatory milestones, acquisitions or strategic transactions, operating income (loss), cash flow (including, but not limited to, operating cash flow and free cash flow), return on capital, assets, equity, or investment, return on sales, gross or net profit levels, productivity, expense, margins, operating efficiency, customer satisfaction, working capital, earnings (loss) per share of stock, sales or market shares and number of customers, any of which may be measured either in absolute terms or as compared to any incremental increase or as compared to results of a peer group. From and after the time that we become subject to Section 162(m) of the Code, the maximum award that is intended to qualify as “performance-based compensation” under Section 162(m) of the Code that may be made to any one employee during any one calendar year period is \_\_\_\_\_ shares of common stock with respect to a stock-based award and \$ \_\_\_\_\_ with respect to a cash-based award.

The 2013 Plan provides that upon the effectiveness of a “sale event,” as defined in the 2013 Plan, in the event that all awards are not assumed or continued or substituted by the successor entity, all options and stock appreciation rights that are not exercisable immediately prior to the effective time of the sale event shall become fully exercisable as of the effective time of the sale event, all other awards with time-based vesting, conditions or restrictions, shall become fully vested and nonforfeitable as of the effective time of the sale event and all awards with conditions and restrictions relating to the attainment of performance goals may become vested and nonforfeitable in the discretion of the compensation committee and all awards granted under the 2013 Plan shall terminate. In addition, in connection with the termination of the 2013 Plan upon a sale event, we may make or provide for a cash payment to participants holding options and stock appreciation rights equal to the difference between the per share cash consideration payable to stockholders in the sale event and the exercise price of the options or stock appreciation rights.

Our board of directors may amend or discontinue the 2013 Plan and our compensation committee may amend or cancel outstanding awards for purposes of satisfying changes in law or any other lawful purpose, but no such action may adversely affect rights under an award without the holder’s consent. Certain amendments to the 2013 Plan require the approval of our stockholders.

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No awards may be granted under the 2013 Plan after the date that is ten years from the date of stockholder approval of the 2013 Plan. No awards under the 2013 Plan have been made prior to the date hereof.

**2013 Employee Stock Purchase Plan**

Our 2013 Employee Stock Purchase Plan was adopted by our board of directors and approved by our stockholders in 2013 and will become effective upon closing of this offering. Our 2013 Employee Stock Purchase Plan authorizes the initial issuance of up to a total of \_\_\_\_\_ shares of our common stock to participating employees.

All employees who have been employed by us or our designated subsidiaries for at least \_\_\_\_\_ and whose customary employment is for \_\_\_\_\_ are eligible to participate in our 2013 Employee Stock Purchase Plan. Any employee who owns, or would own upon such purchase under our 2013 Employee Stock Purchase Plan, 5% or more of the voting power or value of our stock is not eligible to purchase shares under our 2013 Employee Stock Purchase Plan.

We may make one or more offerings to our employees to purchase stock under our 2013 Employee Stock Purchase Plan. Unless otherwise determined by the administrator of our 2013 Employee Stock Purchase Plan, the first offering will begin on \_\_\_\_\_ of the year designated by the administrator and end on the following \_\_\_\_\_. Subsequent offerings will begin on the first business day occurring on or after each \_\_\_\_\_ and \_\_\_\_\_ and will continue for \_\_\_\_\_ periods, referred to as offering periods. The administrator may designate different offering periods in its discretion but no offering shall exceed six months in duration or overlap with another offering.

Each employee who is a participant in our 2013 Employee Stock Purchase Plan may purchase shares by authorizing payroll deductions of up to \_\_\_\_\_ % of his or her eligible compensation during an offering period. Unless the participating employee has previously withdrawn from the offering, his or her accumulated payroll deductions will be used to purchase common stock on the last business day of the offering period at a price equal to \_\_\_\_\_ % of the fair market value of the common stock on or the last business day of the offering period, whichever is lower, provided that no more than \_\_\_\_\_ shares of common stock or such other maximum number established by the compensation committee may be purchased by any one employee during each offering period. Under applicable tax rules, an employee may purchase no more than \$25,000 worth of common stock, valued at the start of the purchase period, under our 2013 Employee Stock Purchase Plan in any calendar year.

The accumulated payroll deductions of any employee who is not a participant on the last day of an offering period will be refunded. An employee's rights under our 2013 Employee Stock Purchase Plan terminate upon voluntary withdrawal from the plan or when the employee ceases employment for any reason.

Our 2013 Employee Stock Purchase Plan may be terminated or amended by our board of directors at any time. Amendments that increase the number of shares of our common stock authorized under our 2013 Employee Stock Purchase Plan and certain other amendments require the approval of our stockholders.

**2010 Stock Option and Grant Plan**

Our 2010 Stock Option and Grant Plan, or the 2010 Plan, was approved by our board of directors on September 15, 2010 and was subsequently approved by our stockholders on October 4, 2010. The

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2010 Plan was most recently amended in January 2013. Under the 2010 Plan, as of January 16, 2013, we have reserved for issuance an aggregate of (i) 81,953,382 shares of our common stock plus (ii) the number of shares of common stock returned to the 2002 Plan, as defined below, after January 16, 2013. The number of shares of common stock reserved for issuance is subject to adjustment in the event of a stock split, stock dividend or other change in our capitalization.

The shares we issue under the 2010 Plan will be authorized but unissued shares or shares we reacquire. The shares of common stock underlying any awards that are forfeited, canceled, repurchased, expire or are otherwise terminated (other than by exercise) under the 2010 Plan are added to the shares of common stock available for issuance under the 2010 Plan. Upon this offering, such shares will be added to the shares of common stock available for issuance under the 2013 Plan.

Our board of directors has acted as administrator of the 2010 Plan. The administrator has full power to select, from among the individuals eligible for awards, the individuals to whom awards will be granted, and to determine the specific terms and conditions of each award, subject to the provisions of the 2010 Plan. Persons eligible to participate in the 2010 Plan are those full or part-time officers, employees, directors, consultants and other key persons (including prospective employees, but conditioned upon their employment) of us and our subsidiaries as selected from time to time by the administrator in its discretion.

The 2010 Plan permits the granting of (1) options to purchase common stock intended to qualify as incentive stock options under Section 422 of the Code and (2) options that do not so qualify. The option exercise price of each option will be determined by the administrator but may not be less than 100% of the fair market value of the common stock on the date of grant. The term of each option will be fixed by the administrator and may not exceed ten years from the date of grant. The administrator will determine at what time or times each option may be exercised. In addition, the 2010 Plan permits the granting of restricted shares of common stock, restricted stock units and unrestricted stock.

The 2010 Plan provides that upon the occurrence of a "sale event" as defined in the 2010 Plan, all outstanding stock options will terminate at the effective time of such sale event, unless the parties to the sale event agree that such awards will be assumed or continued by the successor entity. In the event of a termination of the 2010 Plan and all options issued thereunder in connection with a sale event, the optionees will be provided an opportunity to exercise their options prior to the completion of the sale event. In the case of a sale event in which our stockholders will receive cash consideration, the administrator has the right to provide for cash payment to holders of vested options in an amount equal to the difference between the per share cash consideration and the exercise price of such options. Restricted stock and restricted stock units will be forfeited immediately prior to the effective time of a sale event unless such awards are assumed or continued by the successor entity. In the event that the shares of restricted stock are forfeited in connection with a sale event, such shares of restricted stock shall be repurchased at a price per share equal to the lower of the original per share purchase price and the fair market value of such shares. The administrator has the right to provide for cash payment to holders of restricted stock or restricted stock units in an amount equal to the per share cash consideration in the sale event.

No awards may be granted under the 2010 Plan after the date that is ten years from the date the 2010 Plan was adopted by the board of directors. Our board of directors has determined not to make any further awards under the 2010 Plan following the closing of this offering.

### **2002 Employee, Director and Consultant Plan**

Our Second Amended and Restated 2002 Employee, Director and Consultant Stock Plan, or the 2002 Plan, was approved by our board of directors and our stockholders on June 2, 2004. Our board of directors has not granted any awards under our 2002 Plan since it terminated on October 4, 2010 and does not plan to grant any further awards under our 2002 Plan. As of December 31, 2012, there were options to purchase 5,420,099 shares of common stock outstanding under the 2002 Plan.

The shares of common stock underlying any awards that are forfeited, canceled, repurchased, expire or are otherwise terminated (other than by exercise) under the 2002 Plan are added to the shares of common stock available for issuance under the 2010 Plan. Upon this offering, such shares will be added to the shares of common stock available for issuance under the 2013 Plan.

The 2002 Plan provides that upon the occurrence of a "corporate transaction" as defined in the 2002 Plan, the parties to such corporate transaction may provide that (i) the options will be assumed or continued by the successor entity, (ii) optionees will be provided an opportunity to exercise their options prior to the completion of the corporate transaction, or (iii) vested options will be terminated in exchange for a cash payment equal to the difference between the fair market value of the shares subject to the options and the exercise price.

### **Executive Cash Incentive Bonus Plan**

Our board of directors has adopted the Executive Cash Incentive Bonus Plan, or the Bonus Plan, which is effective as of the closing of this offering. The Bonus Plan provides for cash bonus payments based upon the attainment of performance targets established by our compensation committee. The payment targets will be related to corporate, financial and operational measures or objectives, or Corporate Performance Goals, as well as individual performance objectives.

Our compensation committee may select Corporate Performance Goals from among the following: achievement of specified research and development, publication, clinical and/or regulatory milestones; cash flow (including, but not limited to, operating cash flow and free cash flow); sales or revenue; corporate revenue; earnings before interest, taxes, depreciation and amortization; net income (loss) (either before or after interest, taxes, depreciation and/or amortization); changes in the market price of our common stock; economic value-added; funds from operations or similar measure; acquisitions or strategic transactions; operating income (loss); return on capital, assets, equity, or investment; stockholder returns; return on sales; gross or net profit levels; productivity; expense efficiency; margins; operating efficiency; customer satisfaction; working capital; earnings (loss) per share of our common stock; bookings, new bookings or renewals; sales or market shares; number of customers; number of new customers or customer references; operating income and/or net annual recurring revenue, any of which may be measured in absolute terms, as compared to any incremental increase, in terms of growth, as compared to results of a peer group, against the market as a whole, compared to applicable market indices and/or measured on a pre-tax or post-tax basis.

Each executive officer who is selected to participate in the Bonus Plan will have a target bonus opportunity set for each performance period. The bonus formulas will be adopted in each performance period by the compensation committee and communicated to each executive. The Corporate Performance Goals will be measured at the end of each performance period after our financial reports have been published or such other appropriate time as the compensation

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committee determines. If the Corporate Performance Goals and individual performance objectives are met, payments will be made as soon as practicable following the end of each performance period. Subject to the rights contained in any agreement between the executive officer and the company, an executive officer must be employed by the company on the bonus payment date to be eligible to receive a bonus payment. The Bonus Plan also permits the compensation committee to approve additional bonuses to executive officers in its sole discretion.

### **401(k) plan**

We maintain a 401(k) plan for employees. The 401(k) plan is intended to qualify under Section 401(k) of the Code, so that contributions to the 401(k) plan by employees or by us, and the investment earnings thereon, are not taxable to the employees until withdrawn from the 401(k) plan, and so that contributions by us, if any, will be deductible by us when made. Under the 401(k) plan, employees may elect to reduce their current compensation by up to the statutorily prescribed annual limit and to have the amount of such reduction contributed to the 401(k) plan. The 401(k) plan permits us to make contributions up to the limits allowed by law on behalf of all eligible employees. Historically, we have not made any matching contributions to the 401(k) plan.

### **Rule 10b5-1 sales plans**

Our directors and executive officers may adopt written plans, known as Rule 10b5-1 plans, in which they will contract with a broker to buy or sell shares of our common stock on a periodic basis. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the director or officer when entering into the plan, without further direction from the director or officer. The director or officer may amend or terminate the plan in limited circumstances. Our directors and executive officers may also buy or sell additional shares of our common stock outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information.



## Certain relationships and related party transactions

The following is a description of transactions since January 1, 2010 to which we have been a party, in which the amount involved exceeds \$120,000, and in which any of our directors, executive officers or holders of more than 5% of our capital stock, or an affiliate or immediate family member thereof, had or will have a direct or indirect material interest. We believe the terms obtained or consideration that we paid or received, as applicable, in connection with the transactions described below were comparable to terms available or the amounts that would be paid or received, as applicable, from unaffiliated third parties.

### Sales and purchases of securities

#### Series B financing

In March 2010, we issued an aggregate of 61,555,660 shares of our Series B Preferred Stock for aggregate consideration of \$16.8 million in cash and \$3.3 million in converted bridge notes to five investors. In April 2011, we issued, pursuant to a second tranche closing, an aggregate of 53,648,066 shares of our Series B Preferred Stock for aggregate consideration of \$17.5 million to the same five investors. The table below sets forth the aggregate number of shares of Series B Preferred Stock sold to our directors, executive officers or holders of more than 5% of our capital stock, or an affiliate or immediate family member thereof:

Name	Shares	Aggregate purchase price
Third Rock Ventures, L.P.	64,377,682	\$21,000,000
TVM V Life Science Ventures GmbH & Co. KG	17,749,014	\$ 5,789,728
Cooperative AAC LS U.A.	10,649,408	\$3,473,837

In May 2007, December 2007, May 2008, August 2008, December 2008, April 2009, July 2009, October 2009 and December 2009, we issued warrants to purchase 1,133,100, 472,124, 472,124, 472,124, 472,124, 321,044, 321,044, 283,274, and 574,800 shares, respectively, of either (i) our Series A-1 Preferred Stock or (ii) such preferred stock that we may issue in a subsequent qualified financing. In March 2010, in connection with the Series B Preferred Stock financing, the 2007, 2008 and the April, July and October 2009 warrants were amended to provide that such warrants would be exercisable only for shares of our Series A-1 Preferred Stock at a per share price of \$0.6619 and the December 2009 warrants were amended to provide that such warrants would be exercisable only for shares of our Series B Preferred Stock at a per share price of \$0.3262. The table below set forth the number and class of shares issuable pursuant to warrants amended in March 2010 held by our directors, executive officers or holders of more than 5% of our capital stock, or an affiliate or immediate family member thereof:

Name	Warrants to purchase shares of Series A-1 preferred stock	Warrants to purchase shares of Series B preferred stock
TVM V Life Science Ventures GmbH & Co. KG	3,075,111	287,400
Cooperative AAC LS U.A.	1,656,206	172,440

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**Series C financing**

In April 2011, we issued an aggregate of 39,942,483 shares of our Series C Preferred Stock for aggregate consideration of \$15.0 million to five investors. The table below sets forth the number of shares of Series C Preferred Stock sold to our directors, executive officers or holders of more than 5% of our capital stock, or an affiliate or immediate family member thereof:

<b>Name</b>	<b>Shares</b>	<b>Aggregate purchase price</b>
ARCH Venture Fund VII, L.P.	19,971,242	\$ 7,500,000
Third Rock Ventures, L.P.	14,379,294	\$ 5,400,000
TVM V Life Science Ventures GmbH & Co. KG	3,994,248	\$ 1,500,000
Cooperative AAC LS U.A.	1,331,416	\$ 500,000

**Series D financing**

In July 2012, we issued an aggregate of 120,409,385 shares of our Series D Preferred Stock for aggregate consideration of \$60.0 million to 17 investors. The table below sets forth the number of shares of Series D Preferred Stock sold to our directors, executive officers or holders of more than 5% of our capital stock, or an affiliate or immediate family member thereof:

<b>Name</b>	<b>Shares</b>	<b>Aggregate purchase price</b>
Entities Affiliated with Fidelity Investors	37,728,275	\$ 18,800,000
Entities Affiliated with Capital Research and Management Company	29,500,300	\$ 14,700,000
ARCH Venture Fund VII, L.P.	14,047,762	\$ 7,000,000
Third Rock Ventures, L.P.	11,037,527	\$ 5,500,000
TVM V Life Science Ventures GmbH & Co. KG	3,010,234	\$ 1,500,000
Cooperative AAC LS U.A.	1,003,411	\$ 500,000

**Consulting services provided by Third Rock Ventures, LLC**

During the fiscal years ended December 31, 2010, 2011 and 2012, we incurred consulting fees to Third Rock Ventures, LLC in the amount of \$0.8, \$0.4 and \$0.1 million, respectively. Third Rock Ventures, LLC is a management company that is party to a services agreement with Third Rock Ventures, L.P., the beneficial owner of more than five percent of our voting securities. Robert I. Tepper, M.D., one of our directors, is a managing member of TRV GP, LLC, which is the general partner of Third Rock Ventures GP, L.P., the general partner of Third Rock Ventures, L.P. and a managing member of Third Rock Ventures, LLC. These consulting fees were paid to Third Rock Ventures, LLC in consideration of certain strategic and business operations consulting services provided to us during this period by Third Rock Ventures, LLC by individuals other than Dr. Tepper. None of these consulting fees were paid directly or indirectly to Dr. Tepper. The consulting fees paid to Third Rock Ventures, LLC did not exceed five percent of the consolidated gross revenues of Third Rock Ventures, LLC during any of these fiscal years. We are not currently party to a consulting agreement with Third Rock Ventures, LLC and we do not expect to engage Third Rock Ventures, LLC for consulting services on a going forward basis.

## **Director and executive officer compensation**

Please see “Executive and director compensation—Director compensation” for a discussion of options granted to our non-employee directors. Please see “Executive and director compensation—Equity compensation” for additional information regarding compensation of executive officers.

## **Employment agreements**

We have entered into employment agreements with our executive officers. For more information regarding these agreements, see “Executive and director compensation—Employment agreements with our named executive officers.”

## **Indemnification agreements and directors’ and officers’ liability insurance**

We have entered into indemnification agreements with each of our executive officers and directors.

## **Registration rights agreements**

We and certain holders of our preferred stock have entered into an investor rights agreement pursuant to which these stockholders will have, among other things, registration rights under the Securities Act of 1933, as amended, with respect to common stock that they will hold following this offering. Upon the closing of this offering, all outstanding shares of our preferred stock will be converted into common stock. See “Description of capital stock—Registration rights” for a further description of the terms of these agreements.

## **Procedures for related party transactions**

We have adopted a related person transaction approval policy that will govern the review of related person transactions following the closing of this offering. Pursuant to this policy, if we want to enter into a transaction with a related person or an affiliate of a related person, our chief operating officer will review the proposed transaction to determine, based on applicable Nasdaq and Securities and Exchange Commission rules, if such transaction requires pre-approval by the audit committee and/or board of directors. If pre-approval is required, such matters will be reviewed at the next regular or special audit committee and/or board of directors meeting. We may not enter into a related person transaction unless our chief operating officer has either specifically confirmed in writing that no further reviews are necessary or has confirmed that all requisite corporate reviews have been obtained.

## Principal stockholders

The following table sets forth information relating to the beneficial ownership of our common stock as of March 31, 2013, by:

- each person, or group of affiliated persons, known by us to beneficially own more than 5% of our outstanding shares of common stock;
- each of our directors;
- each of our named executive officers; and
- all directors and executive officers as a group.

The number of shares beneficially owned by each entity, person, director or executive officer is determined in accordance with the rules of the Securities and Exchange Commission, and the information is not necessarily indicative of beneficial ownership for any other purpose. Under such rules, beneficial ownership includes any shares over which the individual has sole or shared voting power or investment power as well as any shares that the individual has the right to acquire within 60 days of March 31, 2013 through the exercise of any stock option, warrants or other rights. Except as otherwise indicated, and subject to applicable community property laws, the persons named in the table have sole voting and investment power with respect to all shares of common stock held by that person.

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The percentage of shares beneficially owned is computed on the basis of 319,946,737 shares of our common stock outstanding as of March 31, 2013, which reflects the assumed conversion of all of our outstanding shares of preferred stock into an aggregate of 310,841,204 shares of common stock. Shares of our common stock that a person has the right to acquire within 60 days of March 31, 2013 are deemed outstanding for purposes of computing the percentage ownership of the person holding such rights, but are not deemed outstanding for purposes of computing the percentage ownership of any other person, except with respect to the percentage ownership of all directors and executive officers as a group. Unless otherwise indicated below, the address for each beneficial owner listed is c/o bluebird bio, Inc., 840 Memorial Drive, 4<sup>th</sup> Floor, Cambridge, MA 02139.

Name and address of beneficial owner	Number of shares beneficially owned	Percentage of shares beneficially owned	
		Before offering	After offering
<b>5% or greater stockholders:</b>			
Third Rock Ventures, L.P.(1) 29 Newbury Street Boston, MA 02116	89,794,503	28.1%	
TVM V Life Science Ventures GmbH & Co. KG(2) Maximilianstrasse 35 Entrance C 80539 Munich, Germany	46,120,958	14.3%	
Entities affiliated with Fidelity Investments(3) 82 Devonshire St. Boston, MA 02109	37,728,275	11.8%	
ARCH Venture Fund VII, L.P.(4) 8725 West Higgins Road Suite 290 Chicago, IL 60631	34,019,004	10.6%	
Entities affiliated with Capital Research and Management Company(5) 333 S. Hope Street, 55 <sup>th</sup> Floor Los Angeles, CA 90071	29,500,300	9.2%	
Coöperative AAC LS U.A. (Forbion)(6) PO Box 5187 1410 AD Naarden The Netherlands	23,737,882	7.4%	
<b>Directors and named executive officers:</b>			
Nick Leschly(7)	7,962,439	2.5%	
Robert I. Tepper, M.D.(8)	89,794,503	28.1%	
Dr. Axel Polack(9)	46,120,958	14.3%	
Steven Gillis, Ph.D.(10)	34,019,004	10.6%	
Geert-Jan Mulder, M.D.(11)	23,737,882	7.4%	
Daniel S. Lynch(12)	647,135	*	
John M. Maraganore, Ph.D.(13)	224,289	*	
Wendy L. Dixon, Ph.D.	—	—	
David P. Schenkein, M.D.	—	—	
Jeffrey T. Walsh(14)	2,174,637	*	
David Davidson, M.D.(15)	931,990	*	
All executive officers and directors as a group (11 persons)(16)	208,553,356	62.5%	

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\* Represents beneficial ownership of less than one percent of our outstanding common stock.

- (1) Consists of (i) 64,377,682 shares of common stock underlying shares of Series B Convertible Preferred Stock, or Series B Stock, (ii) 14,379,294 shares of common stock underlying shares of Series C Convertible Preferred Stock, or Series C Stock, and (iii) 11,037,527 shares of common stock underlying shares of Series D Convertible Preferred Stock, or Series D Stock. All shares are held directly by Third Rock Ventures, L.P. ("TRV LP"). Each of Third Rock Ventures GP, LP ("TRV GP"), the general partner of TRV LP, and Third Rock Ventures GP, LLC ("TRV LLC"), the general partner of TRV GP, may be deemed to have voting and dispositive power over the shares held by TRV LP. Investment decisions with respect to the shares held by TRV LP are made by an investment committee at TRV GP comprised of Mark Levin, Kevin Starr, Bob Tepper, Neil Exter, Kevin Gillis, Lou Tartaglia, Craig Muir, Cary Pfeffer, Alexis Borisy and Craig Greaves. No stockholder, director, officer, manager, member or employee of TRV GP or TRV LLC has beneficial ownership (within the meaning of Rule 13d-3 promulgated under the Exchange Act) of any shares held by TRV LP.
- (2) Consists of (i) 6,169,117 shares of common stock underlying shares of Series A-1 Convertible Preferred Stock, or Series A-1 Stock, and 3,075,111 shares of common stock underlying warrants to purchase Series A-1 Stock that are exercisable as of March 31, 2013 or will become exercisable within 60 days after such date, (ii) 11,835,834 shares of common stock underlying shares of Series A-2 Convertible Preferred Stock, or Series A-2 Stock, (iii) 17,749,014 shares of common stock underlying shares of Series B Stock and 287,400 shares of common stock underlying warrants to purchase Series B Stock that are exercisable as of March 31, 2013 or will become exercisable within 60 days after such date, (iv) 3,994,248 shares of common stock underlying shares of Series C Stock and (iv) 3,010,234 shares of common stock underlying shares of Series D Stock. All shares are held directly by TVM V Life Science Ventures GmbH & Co. KG ("TVM LSV V"). Its general partner TVM Capital, or TVM, and its authorized officers Axel Polack, Helmut Schuehler, Alexandra Goll, Hubert Birner and Stefan Fischer may be deemed to share voting and dispositive power over the shares held by TVM LSV V. No stockholder, director, officer, manager, member or employee of TVM and no director, officer, manager, member or employee of TVM LSV V has beneficial ownership (within the meaning of Rule 13d-3 promulgated under the Exchange Act) of any shares held by TVM LSV V.
- (3) Consists of (i) 19,295,922 shares of common stock underlying shares of Series D Stock held by Mag & Co. f/b/o Fidelity Contrafund: Fidelity Contrafund, (ii) 4,658,909 shares of common stock underlying shares of Series D Stock held by Mag & Co. f/b/o Fidelity Contrafund: Fidelity Advisor New Insights Fund, (iii) 9,767,944 shares of common stock underlying shares of Series D Stock held by Ball & Co. f/b/o Fidelity Mt. Vernon Street Trust: Fidelity Growth Company Fund, (iv) 535,716 shares of common stock underlying shares of Series D Stock held by Mag & Co. f/b/o Fidelity Select Portfolios: Biotechnology Portfolio, (v) 35,360 shares of common stock underlying shares of Series D Stock held by Bangle & Co f/b/o Fidelity Advisor Series VII: Fidelity Advisor Biotechnology Fund and (vi) 3,434,424 shares of common stock underlying shares of Series D Stock held by Sailboat & Co. f/b/o Fidelity Magellan Fund: Fidelity Magellan Fund. Each of these entities is a registered investment fund (each, a "Fund") advised by Fidelity Management & Research Company ("FMR Co."), a registered investment adviser under the Investment Advisers Act of 1940, as amended. The address of FMR Co., a wholly-owned subsidiary of FMR LLC and an investment adviser registered under Section 203 of the Investment Advisers Act of 1940 is 82 Devonshire Street, Boston, Massachusetts 02109. FMR LLC, through its control of FMR Co., Edward C. Johnson 3d, as Chairman of FMR LLC, and each Fund has power to dispose of the securities owned by such Fund. Neither FMR LLC nor Edward C. Johnson 3d has sole power to vote or direct the voting of the shares owned directly by each Fund, which power resides with each Fund's Board of Trustees.
- (4) Consists of (i) 19,971,242 shares of common stock underlying shares of Series C Stock and (ii) 14,047,762 shares of common stock underlying shares of Series D Stock. All shares are held directly by ARCH Venture Fund VII, L.P. ("ARCH VII"). ARCH Venture Partners VII, L.P. (the "GPLP"), as the sole general partner of ARCH VII, may be deemed to beneficially own certain of the shares held of record by ARCH VII. The GPLP disclaims beneficial ownership of all shares held of record by ARCH VII in which the GPLP does not have an actual pecuniary interest. ARCH Venture Partners VII, LLC (the "GPLLC"), as the sole general partner of the GPLP, may be deemed to beneficially own certain of the shares held of record by ARCH VII. The GPLLC disclaims beneficial ownership of all shares held of record by ARCH VII in which it does not have an actual pecuniary interest. Keith Crandell, Clinton Bybee and Robert Nelsen are the managing directors of the GPLLC, and may be deemed to share voting and dispositive power over the shares held of record by ARCH VII. The managing directors disclaim beneficial ownership of all shares held of record by ARCH VII in which they do not have an actual pecuniary interest. Steven Gillis, one of our directors, owns an interest in GPLP. Mr. Gillis does not have voting or disposition authority of the shares held by ARCH VII.
- (5) Consists of (i) 22,374,386 shares of common stock underlying shares of Series D Stock held by Clipperbay & Co. HG22 as nominee for SMALLCAP World Fund, Inc. and (ii) 7,125,914 shares of common stock underlying shares of Series D Stock held by Piping & Co. HG19 as nominee for American Funds Insurance Series —Global Small Capitalization Fund. Capital Research and Management Company serves as the investment adviser for SMALLCAP World Fund, Inc. and American Funds Insurance Series – Global Small Capitalization Fund. Capital Research and Management Company or its affiliates has voting and dispositive power of all of the shares held by these funds and may be deemed to be the beneficial owner for purposes of reporting requirements of the Exchange Act. Capital Research and Management Company, however, expressly disclaims that it is, in fact, the beneficial owner of such securities. Capital Research and Management Company is an investment adviser registered under the Investment Advisers Act of 1940.

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- (6) Consists of (i) 2,643,906 shares of common stock underlying shares of Series A-1 Stock and 1,656,206 shares of common stock underlying warrants to purchase Series A-1 Stock that are exercisable as of March 31, 2013 or will become exercisable within 60 days after such date, (ii) 6,281,095 shares of common stock underlying shares of Series A-2 Stock, (iii) 10,649,408 shares of common stock underlying shares of Series B Stock and 172,440 shares of common stock underlying warrants to purchase Series B Stock that are exercisable as of March 31, 2013 or will become exercisable within 60 days after such date, (iv) 1,003,411 shares of common stock underlying shares of Series C Stock and (v) 3,010,234 shares of common stock underlying shares of Series D Stock. All shares are held by Coöperative AAC LS U.A., or Coöperative. Forbion 1 Management B.V., or Forbion, the director of Coöperative, may be deemed to have voting and dispositive power over the shares held by Coöperative. Investment decisions with respect to the shares held by Coöperative can be made by any two of the six duly authorized representatives of Coöperative, which comprise directors L.P.A. Bergstein, M.A. van Osch, H.A. Slootweg and proxy holders S.J.H. van Deventer, G.J. Mulder and C. Takke. No stockholder, director, officer, manager, member or employee of Coöperative or Forbion has beneficial ownership (within the meaning of Rule 13d-3 promulgated under the Exchange Act) of any shares held by Coöperative.
- (7) Includes 1,717,426 shares of common stock underlying options that are exercisable as of March 31, 2013 or will become exercisable within 60 days after such date.
- (8) Consists of 89,794,503 shares of common stock into which the shares of preferred stock beneficially owned by Third Rock Ventures, L.P. are convertible. Dr. Tepper is a partner of Third Rock Ventures, L.P. and may be deemed to have voting and investment power over the shares held by Third Rock Ventures, L.P. Dr. Tepper disclaims beneficial ownership with respect to any such shares, except to the extent of his pecuniary interest therein, if any.
- (9) Consists of (i) 42,758,447 shares of common stock into which the shares of preferred stock beneficially owned by TVM V Life Science Ventures GmbH & Co. KG are convertible and (ii) 3,362,511 shares of common stock underlying preferred stock warrants that are exercisable as of March 31, 2013 or will become exercisable within 60 days after such date. Dr. Polack is a managing limited partner of TVM V Life Science Ventures GmbH & Co. KG and may be deemed to have voting and investment power, jointly not solely, over the shares held by TVM V Life Science Ventures GmbH & Co. KG. Dr. Polack disclaims beneficial ownership with respect to any such shares, except to the extent of his pecuniary interest therein, if any.
- (10) Consists of 34,019,004 shares of common stock into which the shares of preferred stock beneficially owned by ARCH Venture Fund II, L.P. are convertible. ARCH Venture Fund II, L.P. is an affiliated fund of ARCH Venture Partners. Dr. Gillis is a managing director with ARCH Venture Partners and may be deemed to have voting and investment power over the shares held by ARCH Venture Fund II, L.P. Dr. Gillis disclaims beneficial ownership with respect to any such shares, except to the extent of his pecuniary interest therein, if any.
- (11) Consists of (i) 23,737,882 shares of common stock into which the shares of preferred stock beneficially owned by Coöperative AAC LS U.A. are convertible and (ii) 1,828,646 shares of common stock underlying preferred stock warrants that are exercisable as of March 31, 2013 or will become exercisable within 60 days after such date. Dr. Mulder is a general partner of Coöperative AAC LS U.A. and may be deemed to have voting and investment power over the shares held by Forbion Capital Partners. Dr. Mulder disclaims beneficial ownership with respect to any such shares, except to the extent of his pecuniary interest therein, if any.
- (12) Consists of 647,135 shares of common stock underlying options that are exercisable as of March 31, 2013 or will become exercisable within 60 days after such date.
- (13) Consists of 224,289 shares of common stock underlying options that are exercisable as of March 31, 2013 or will become exercisable within 60 days after such date.
- (14) Consists of 2,174,637 shares of common stock underlying options that are exercisable as of March 31, 2013 or will become exercisable within 60 days after such date.
- (15) Consists of 931,990 shares of common stock underlying options that are exercisable as of March 31, 2013 or will become exercisable within 60 days after such date.
- (16) Includes (i) 5,191,157 shares of common stock underlying preferred stock warrants that are exercisable as of March 31, 2013 or will become exercisable within 60 days after such date and (ii) 8,635,997 shares of common stock underlying options that are exercisable as of March 31, 2013 or will become exercisable within 60 days after such date.

## Description of capital stock

### General

Upon completion of this offering, our authorized capital stock will consist of \_\_\_\_\_ shares of common stock, par value \$0.01 per share, and \_\_\_\_\_ shares of preferred stock, par value \$0.01 per share. The following description of our capital stock is intended as a summary only and is qualified in its entirety by reference to our amended and restated certificate of incorporation, or our certificate of incorporation, and amended and restated bylaws, or our by-laws, to be in effect at the closing of this offering, which are filed as exhibits to the registration statement, of which this prospectus forms a part, and to the applicable provisions of the Delaware General Corporation Law. We refer in this section to our amended and restated certificate of incorporation as our certificate of incorporation, and we refer to our amended and restated by-laws as our by-laws.

### Common stock

As of March 31, 2013, there were 319,946,737 shares of our common stock outstanding, including 2,506,114 shares of unvested restricted stock subject to repurchase by us, held of record by 50 stockholders, and assuming the conversion of all outstanding shares of preferred stock for shares of our common stock. Upon completion of this offering, there will be \_\_\_\_\_ shares of our common stock outstanding.

Holders of our common stock are entitled to one vote for each share of common stock held of record for the election of directors and on all matters submitted to a vote of stockholders. Holders of our common stock are entitled to receive dividends ratably, if any, as may be declared by our board of directors out of legally available funds, subject to any preferential dividend rights of any preferred stock then outstanding. Upon our dissolution, liquidation or winding up, holders of our common stock are entitled to share ratably in our net assets legally available after the payment of all our debts and other liabilities, subject to the preferential rights of any preferred stock then outstanding. Holders of our common stock have no preemptive, subscription, redemption or conversion rights. The rights, preferences and privileges of holders of common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of preferred stock that we may designate and issue in the future. Except as described below in "Anti-takeover effects of Delaware law, our certificate of incorporation and our by-laws," a majority vote of common stockholders is generally required to take action under our certificate of incorporation and by-laws.

### Preferred stock

Upon completion of this offering, our board of directors will be authorized, without action by the stockholders, to designate and issue up to an aggregate of \_\_\_\_\_ shares of preferred stock in one or more series. The board of directors can fix the rights, preferences and privileges of the shares of each series and any of its qualifications, limitations or restrictions. Our board of directors may authorize the issuance of preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of common stock. The issuance of preferred stock, while providing flexibility in connection with possible future financings and acquisitions and other corporate purposes could, under certain circumstances, have the effect of delaying or preventing a change in control of our company and might harm the market price of our common stock.



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Our board of directors will make any determination to issue such shares based on its judgment as to our best interests and the best interests of our stockholders. We have no current plans to issue any shares of preferred stock.

Certain of our stockholders hold, as of the date of this prospectus, 12,981,286 shares of our Series A-1 preferred stock, 22,304,324 shares of our Series A-2 preferred stock, 115,203,726 shares of our Series B preferred stock, 39,942,483 shares of our Series C preferred stock and 120,409,385 shares of our Series D preferred stock. Upon completion of this offering, each share of Series A-1, Series A-2, Series B, Series C and Series D preferred stock outstanding will be converted into our common stock on a -for-1 basis. Holders of substantially all of the shares of our preferred stock are subject to lock-up agreements with the underwriters that restrict the sale of our securities for 180 days following the date of this prospectus. See "Underwriting" for a description of these lock-up agreements

## Warrants

As of March 31, 2013, warrants to purchase a total of 1,942,131 shares of our common stock were outstanding with a weighted average exercise price of \$0.01 per share. These warrants expire beginning in March 2020.

As of March 31, 2013, warrants to purchase a total of 5,835,456 shares of our Series A-1 preferred stock were outstanding with an exercise price of \$0.6619 per share. These warrants to purchase 5,835,456 Series A-1 preferred shares, which will be converted into warrants to purchase shares of common stock upon completion of this offering, are exercisable immediately and expire beginning in November 2015 through April 2019.

As of March 31, 2013, warrants to purchase a total of 574,800 shares of our Series B preferred stock were outstanding with an exercise price of \$0.3262 per share. These warrants to purchase 574,800 Series B preferred shares, which will be converted into warrants to purchase shares of common stock upon completion of this offering, are exercisable immediately and expire in April 2019.

## Registration rights

We entered into an amended and restated investors' rights agreement, dated as of July 23, 2012, with the holders of shares of our common stock issuable upon conversion of the shares of preferred stock. These shares will represent approximately % of our outstanding common stock after this offering, or % if the underwriters exercise their option to purchase additional shares in full. These shares also may be sold under Rule 144 under the Securities Act of 1933, as amended, depending on their holding period and subject to restrictions in the case of shares held by persons deemed to be our affiliates.

Under the amended and restated investors' rights agreement, holders of registrable shares can demand that we file a registration statement or request that their shares be included on a registration statement that we are otherwise filing, in either case, registering the resale of their shares of common stock. These registration rights are subject to conditions and limitations, including the right, in certain circumstances, of the underwriters of an offering to limit the number of shares included in such registration and our right, in certain circumstances, not to effect a requested S-1 registration within 60 days before or 180 days following any offering of our securities, including this offering or a requested S-3 registration within 30 days before or 90 days following any offering of our securities, including this offering.

### ***Demand registration rights***

Following the six-month anniversary of the date of this prospectus, the holders of at least a majority of the registrable shares may require us to file a registration statement under the Securities Act on a Form S-1 or S-3, if available, at our expense with respect to the resale of their registrable shares, and we are required to use our best efforts to effect the registration.

### ***Piggyback registration rights***

If we propose to register any of our securities under the Securities Act for our own account or the account of any other holder, the holders of registrable shares are entitled to notice of such registration and to request that we include registrable shares for resale on such registration statement, subject to the right of any underwriter to limit the number of shares included in such registration.

We will pay all registration expenses, other than underwriting discounts and commissions, related to any demand or piggyback registration. The amended and restated investors' rights agreement contains customary cross-indemnification provisions, pursuant to which we are obligated to indemnify the selling stockholders, in the event of misstatements or omissions in the registration statement attributable to us except in the event of fraud and they are obligated to indemnify us for misstatements or omissions attributable to them.

The registration rights will terminate upon the later of the date on which all registrable shares have been sold and the fifth anniversary of the closing date of this offering.

### ***Voting agreement and right of first refusal and co-sale agreement***

We entered into an amended and restated voting agreement and an amended and restated right of first refusal and co-sale agreement, each dated as of July 23, 2012, with all holders of our preferred stock and certain holders of our common stock. These agreements provide for certain rights and obligations, such as board composition requirements and stock transfer restrictions. These agreements will terminate upon the completion of this offering; however, the lock-up provision under the amended and restated right of first refusal and co-sale agreement will survive termination pursuant to the terms of the agreement. The lock-up provision under the investors' rights agreement shall also survive the completion of this offering. See "Shares eligible for future sales—Lock-up agreements."

### ***Anti-takeover effects of Delaware law, our certificate of incorporation and our by-laws***

Our certificate of incorporation and by-laws include a number of provisions that may have the effect of encouraging persons considering unsolicited tender offers or other unilateral takeover proposals to negotiate with our board of directors rather than pursue non-negotiated takeover attempts. These provisions include the items described below.

### ***Board composition and filling vacancies***

In accordance with our certificate of incorporation, our board is divided into three classes serving three-year terms, with one class being elected each year. Our certificate of incorporation also provides that directors may be removed only for cause and then only by the affirmative vote

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of the holders of 75% or more of the shares then entitled to vote at an election of directors. Furthermore, any vacancy on our board of directors, however occurring, including a vacancy resulting from an increase in the size of our board, may only be filled by the affirmative vote of a majority of our directors then in office, even if less than a quorum.

***No written consent of stockholders***

Our certificate of incorporation provides that all stockholder actions are required to be taken by a vote of the stockholders at an annual or special meeting, and that stockholders may not take any action by written consent in lieu of a meeting.

***Meetings of stockholders***

Our by-laws provide that only a majority of the members of our board of directors then in office may call special meetings of stockholders and only those matters set forth in the notice of the special meeting may be considered or acted upon at a special meeting of stockholders. Our by-laws limit the business that may be conducted at an annual meeting of stockholders to those matters properly brought before the meeting.

***Advance notice requirements***

Our by-laws establish advance notice procedures with regard to stockholder proposals relating to the nomination of candidates for election as directors or new business to be brought before meetings of our stockholders. These procedures provide that notice of stockholder proposals must be timely given in writing to our corporate secretary prior to the meeting at which the action is to be taken. Generally, to be timely, notice must be received at our principal executive offices not less than 90 days or more than 120 days prior to the first anniversary date of the annual meeting for the preceding year. The notice must contain certain information specified in the by-laws. These provisions may have the effect of precluding the conduct of certain business at a meeting if the proper procedures are not followed. These provisions may also discourage or deter a potential acquirer from conducting a solicitation of proxies to elect the acquirer's own slate of directors or otherwise attempting to obtain control of our company.

***Amendment to by-laws and certificate of Incorporation***

As required by the Delaware General Corporation Law, any amendment of our certificate of incorporation must first be approved by a majority of our board of directors and, if required by law or our certificate of incorporation, thereafter be approved by a majority of the outstanding shares entitled to vote on the amendment, and a majority of the outstanding shares of each class entitled to vote thereon as a class, except that the amendment of the provisions relating to stockholder action, directors, limitation of liability, exclusive jurisdiction of Delaware Courts and the amendment of our by-laws and certificate of incorporation must be approved by not less than 75% of the outstanding shares entitled to vote on the amendment, and not less than 75% of the outstanding shares of each class entitled to vote thereon as a class. Our by-laws may be amended by the affirmative vote of a majority of the directors then in office, subject to any limitations set forth in the by-laws; and may also be amended by the affirmative vote of at least 75% of the outstanding shares entitled to vote on the amendment, or, if the board of directors recommends that the stockholders approve the amendment, by the affirmative vote of the majority of the outstanding shares entitled to vote on the amendment, in each case voting together as a single class.

### **Blank check preferred stock**

Our certificate of incorporation provides for \_\_\_\_\_ authorized shares of preferred stock. The existence of authorized but unissued shares of preferred stock may enable our board of directors to render more difficult or to discourage an attempt to obtain control of us by means of a merger, tender offer, proxy contest or otherwise. For example, if in the due exercise of its fiduciary obligations, our board of directors were to determine that a takeover proposal is not in the best interests of us or our stockholders, our board of directors could cause shares of preferred stock to be issued without stockholder approval in one or more private offerings or other transactions that might dilute the voting or other rights of the proposed acquirer or insurgent stockholder or stockholder group. In this regard, our certificate of incorporation grants our board of directors broad power to establish the rights and preferences of authorized and unissued shares of preferred stock. The issuance of shares of preferred stock could decrease the amount of earnings and assets available for distribution to holders of shares of common stock. The issuance may also adversely affect the rights and powers, including voting rights, of these holders and may have the effect of delaying, deterring or preventing a change in control of us.

### **Section 203 of the Delaware General Corporation Law**

Upon completion of this offering, we will be subject to the provisions of Section 203 of the Delaware General Corporation Law. In general, Section 203 prohibits a publicly-held Delaware corporation from engaging in a “business combination” with an “interested stockholder” for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in a prescribed manner. A “business combination” includes, among other things, a merger, asset or stock sale or other transaction resulting in a financial benefit to the interested stockholder. An “interested stockholder” is a person who, together with affiliates and associates, owns, or did own within three years prior to the determination of interested stockholder status, 15% or more of the corporation’s voting stock.

Under Section 203, a business combination between a corporation and an interested stockholder is prohibited unless it satisfies one of the following conditions:

- before the stockholder became interested, the board of directors approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- upon consummation of the transaction which resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, shares owned by persons who are directors and also officers, and employee stock plans, in some instances; or
- at or after the time the stockholder became interested, the business combination was approved by the board of directors of the corporation and authorized at an annual or special meeting of the stockholders by the affirmative vote of at least two-thirds of the outstanding voting stock which is not owned by the interested stockholder.

A Delaware corporation may “opt out” of these provisions with an express provision in its original certificate of incorporation or an express provision in its certificate of incorporation or by-laws resulting from a stockholders’ amendment approved by at least a majority of the outstanding voting shares. We have not opted out of these provisions. As a result, mergers or other takeover or change in control attempts of us may be discouraged or prevented.

***Exclusive jurisdiction of certain actions***

Our certificate of incorporation requires, to the fullest extent permitted by law, that derivative actions brought in our name, actions against our directors, officers and employees for breach of fiduciary duty and other similar actions may be brought only in the Court of Chancery in the State of Delaware, unless we otherwise consent. Although we believe this provision benefits us by providing increased consistency in the application of Delaware law in the types of lawsuits to which it applies, the provision may have the effect of discouraging lawsuits against our directors and officers.

**Nasdaq Global Market listing**

We have applied for listing of our common stock on The Nasdaq Global Market under the trading symbol "BLUE."

**Transfer agent and registrar**

The transfer agent and registrar for our common stock is American Stock Transfer & Trust Company, LLC.

## Shares eligible for future sale

Prior to this offering, there has been no public market for our common stock. Future sales of our common stock, including shares issued upon the exercise of outstanding options or warrants, in the public market after this offering, or the perception that those sales may occur, could cause the prevailing market price for our common stock to fall or impair our ability to raise equity capital in the future. As described below, only a limited number of shares of our common stock will be available for sale in the public market for a period of several months after completion of this offering due to contractual and legal restrictions on resale described below. Future sales of our common stock in the public market either before (to the extent permitted) or after restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price of our common stock at such time and our ability to raise equity capital at a time and price we deem appropriate.

### Sale of restricted shares

As of March 31, 2013, based on the number of shares of our common stock then outstanding, upon the closing of this offering and assuming (1) the conversion of our outstanding preferred stock into common stock, (2) no exercise of the underwriters' option to purchase additional shares of common stock and (3) no exercise of outstanding options or warrants, we would have had outstanding an aggregate of approximately \_\_\_\_\_ shares of common stock. Of these shares, all of the \_\_\_\_\_ shares of common stock to be sold in this offering, and any shares sold upon exercise of the underwriters' option to purchase additional shares will be freely tradable in the public market without restriction or further registration under the Securities Act of 1933, as amended, or the Securities Act, unless the shares are held by any of our "affiliates" as such term is defined in Rule 144 of the Securities Act. All remaining shares of common stock held by existing stockholders immediately prior to the completion of this offering will be "restricted securities" as such term is defined in Rule 144. These restricted securities were issued and sold by us, or will be issued and sold by us, in private transactions and are eligible for public sale only if registered under the Securities Act or if they qualify for an exemption from registration under the Securities Act, including the exemptions provided by Rule 144 or Rule 701, which rules are summarized below.

As a result of the lock-up agreements referred to below and the provisions of Rule 144 and Rule 701 under the Securities Act, the shares of our common stock (excluding the shares sold in this offering) that will be available for sale in the public market are as follows:

Approximate Number of Shares	First Date Available for Sale into Public Market
	180 days after the date of this prospectus upon expiration of the lock-up agreements referred to below, subject in some cases to applicable volume limitations under Rule 144

### Lock-up agreements

In connection with this offering, we, our directors, our executive officers and stockholders holding approximately \_\_\_\_\_ % of our shares of common stock outstanding as of March 31, 2013 (assuming conversion of all of our outstanding shares of preferred stock), and substantially all of our option holders who are not also stockholders have agreed, subject to certain exceptions, with the underwriters not to dispose of or hedge any shares of our common stock or securities convertible into or exchangeable for shares of common stock during the period from the date of

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the lock-up agreement continuing through the date 180 days after the date of this prospectus, except with the prior written consent of J.P. Morgan Securities LLC and Merrill Lynch, Pierce, Fenner & Smith Incorporated, together the representatives of the underwriters. The representatives of the underwriters have advised us that they have no current intent or arrangement to release any of the shares subject to the lock-up agreements prior to the expiration of the lock-up period.

Following the lock-up periods set forth in the agreements described above, and assuming that the representatives of the underwriters do not release any parties from these agreements, all of the shares of our common stock that are restricted securities or are held by our affiliates as of the date of this prospectus will be eligible for sale in the public market in compliance with Rule 144 under the Securities Act.

In addition, pursuant to each of our amended and restated investors' rights agreement and amended and restated right of first refusal and co-sale agreement, the parties thereto have agreed that, if requested in writing by the representatives of the underwriters of the initial public offering of our securities, they will not sell, make any short sale of, grant any option for the purchase of, or otherwise dispose of any shares of our stock during the same 180-day restricted period referred to above. We expect the representatives of the underwriters to invoke this written request prior to the completion of this offering and, accordingly, that the parties to these agreements will be subject to the related transaction restrictions.

Holders of approximately \_\_\_\_\_ shares of common stock (including shares of our preferred stock that will be converted into shares of our common stock upon completion of this offering), or \_\_\_\_\_ % of our outstanding shares of common stock on an as converted basis, are, collectively subject to lock-up restrictions as parties to these agreements or lock-up agreements with the underwriters.

## Rule 144

In general, under Rule 144, as currently in effect, once we have been subject to the public company reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, for at least 90 days, a person (or persons whose shares are required to be aggregated) who is not deemed to have been one of our "affiliates" for purposes of Rule 144 at any time during the three months preceding a sale, and who has beneficially owned restricted securities within the meaning of Rule 144 for at least six months, including the holding period of any prior owner other than one of our "affiliates," is entitled to sell those shares in the public market (subject to the lock-up agreement referred to above, if applicable) without complying with the manner of sale, volume limitations or notice provisions of Rule 144, but subject to compliance with the public information requirements of Rule 144. If such a person has beneficially owned the sales proposed to be sold for at least one year, including the holding period of any prior owner other than "affiliates," then such person is entitled to sell such shares in the public market without complying with any of the requirements of Rule 144 (subject to the lock-up agreement referred to above, if applicable). In general, under Rule 144, as currently in effect, once we have been subject to the public company reporting requirements of the Exchange Act for at least 90 days, our "affiliates," as defined in Rule 144, who have beneficially owned the shares proposed to be sold for at least six months are entitled to sell in the public market, upon expiration of any applicable lock-up agreements and within any three-month period, a number of those shares of our common stock that does not exceed the greater of:

- one percent of the number of common shares then outstanding, which will equal approximately \_\_\_\_\_ shares of common stock immediately after this offering (calculated

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on the basis of the number of shares of our common stock outstanding as of \_\_\_\_\_, the assumptions described above and assuming no exercise of the underwriter's option to purchase additional shares and no exercise of outstanding options or warrants); or

- the average weekly trading volume of our common stock on The Nasdaq Global Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to such sale.

Such sales under Rule 144 by our "affiliates" or persons selling shares on behalf of our "affiliates" are also subject to certain manner of sale provisions, notice requirements and to the availability of current public information about us. Notwithstanding the availability of Rule 144, the holders of substantially all of our restricted securities have entered into lock-up agreements as referenced above and their restricted securities will become eligible for sale (subject to the above limitations under Rule 144) upon the expiration of the restrictions set forth in those agreements.

## **Rule 701**

In general, under Rule 701 as currently in effect, any of our employees, directors, officers, consultants or advisors who acquired common stock from us in connection with a written compensatory stock or option plan or other written agreement in compliance with Rule 701 under the Securities Act before the effective date of the registration statement of which this prospectus is a part (to the extent such common stock is not subject to a lock-up agreement) is entitled to rely on Rule 701 to resell such shares beginning 90 days after we become subject to the public company reporting requirements of the Exchange Act in reliance on Rule 144, but without compliance with the holding period requirements contained in Rule 144. Accordingly, subject to any applicable lock-up agreements, beginning 90 days after we become subject to the public company reporting requirements of the Exchange Act, under Rule 701 persons who are not our "affiliates," as defined in Rule 144, may resell those shares without complying with the minimum holding period or public information requirements of Rule 144, and persons who are our "affiliates" may resell those shares without compliance with Rule 144's minimum holding period requirements (subject to the terms of the lock-up agreement referred to below, if applicable).

## **Equity incentive plans**

We intend to file with the Securities and Exchange Commission a registration statement under the Securities Act covering the shares of common stock that we may issue upon exercise of outstanding options reserved for issuance under the 2002 Employee, Director and Consultant Plan, the 2010 Stock Option and Grant Plan and the 2013 Stock Option and Incentive Plan. Such registration statement is expected to be filed and become effective as soon as practicable after the completion of this offering. Accordingly, shares registered under such registration statement will be available for sale in the open market following its effective date, subject to Rule 144 volume limitations and the lock-up agreements described above, if applicable.



## Material U.S. federal income tax considerations for non-U.S. holders

The following is a summary of the material U.S. federal income tax considerations of the ownership and disposition of our common stock to non-U.S. holders. It is not intended to be a complete analysis of all the U.S. federal income tax considerations that may be relevant to non-U.S. holders. This summary is based upon the provisions of the Internal Revenue Code of 1986, as amended, or the Code, Treasury regulations promulgated thereunder, administrative rulings and judicial decisions, all as of the date hereof. These authorities may be changed, possibly with retroactive effect, which may result in U.S. federal income tax consequences different from those set forth below. We have not sought any ruling from the Internal Revenue Service, or the IRS, with respect to the statements made and the conclusions reached in the following summary. There can be no assurance that the IRS will agree with such statements and conclusions or that any contrary position taken by the IRS would not be sustained by a court.

This summary also does not address the tax considerations arising under the laws of any foreign, state or local jurisdiction. In addition, this discussion does not address tax considerations applicable to an investor's particular circumstances or to investors that may be subject to special tax rules, including, without limitation:

- banks, insurance companies or other financial institutions;
- persons subject to the alternative minimum tax;
- tax-exempt organizations;
- an integral part or controlled entity of a foreign sovereign;
- dealers in securities or currencies;
- traders in securities that elect to use a mark-to-market method of accounting for their securities holdings;
- persons that own, or are deemed to own, more than five percent of our capital stock (except to the extent specifically set forth below);
- controlled foreign corporations or passive foreign investment companies
- certain former citizens or long-term residents of the United States;
- persons who hold our common stock as a position in a hedging transaction, "straddle," "conversion transaction" or other risk reduction transaction;
- persons deemed to sell our common stock under the constructive sale provisions of the Code; or
- persons who hold our common stock other than as a capital asset (generally, an asset held for investment purposes).

If an entity that is classified as a partnership for U.S. federal income tax purposes holds our common stock, the tax treatment of persons treated as its partners for U.S. federal income tax purposes will generally depend upon the status of the partner and the activities of the partnership. Prospective investors that are classified as partnerships for U.S. federal income tax purposes and prospective investors that may hold our common stock through an entity classified as a partnership for U.S. federal income tax purposes, should consult their own tax advisors.

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YOU ARE URGED TO CONSULT YOUR TAX ADVISOR WITH RESPECT TO THE APPLICATION OF THE UNITED STATES FEDERAL INCOME TAX LAWS TO YOUR PARTICULAR SITUATION, AS WELL AS ANY TAX CONSEQUENCES OF THE PURCHASE, OWNERSHIP AND DISPOSITION OF OUR COMMON STOCK ARISING UNDER THE UNITED STATES FEDERAL ESTATE OR GIFT TAX RULES OR UNDER THE LAWS OF ANY STATE, LOCAL, FOREIGN OR OTHER TAXING JURISDICTION OR UNDER ANY APPLICABLE TAX TREATY.

### **Non-U.S. holder defined**

For purposes of this discussion, you are a non-U.S. holder if you are a holder that, for U.S. federal income tax purposes, is not a U.S. person or a partnership. For purposes of this discussion, you are a U.S. person if you are:

- an individual citizen or resident of the United States;
- a corporation or other entity taxable as a corporation created or organized in the United States or under the laws the United States or any political subdivision thereof;
- an estate whose income is subject to U.S. federal income tax regardless of its source; or
- a trust (a) if a court within the United States is able to exercise primary jurisdiction over the administration of the trust and one or more U.S. persons have the authority to control all substantial decisions of the trust or (b) that has made an election to be treated as a U.S. person.

### **Distributions**

We have not made any distributions on our common stock and do not plan to make any distributions for the foreseeable future. However, if we do make distributions on our common stock, those payments will constitute dividends for U.S. tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. To the extent those distributions exceed both our current and our accumulated earnings and profits, they will constitute a return of capital and will first reduce your basis in our common stock, but not below zero, and then will be treated as gain from the sale of stock, which will be subject to tax as described in "Gain on Disposition of Common Stock," below.

Any dividend paid to you generally will be subject to U.S. withholding tax either at a rate of 30% of the gross amount of the dividend or such lower rate as may be specified by an applicable income tax treaty. In order to receive a reduced treaty rate, you must provide us with an IRS Form W-8BEN or other appropriate version of IRS Form W-8 certifying qualification for the reduced rate.

Dividends received by you that are effectively connected with your conduct of a U.S. trade or business are exempt from such withholding tax. In order to obtain this exemption, you must provide us with an IRS Form W-8ECI properly certifying such exemption. Such effectively connected dividends, although not subject to withholding tax, are taxed at the same graduated rates applicable to U.S. persons, net of certain deductions and credits. In addition, if you are a corporate non-U.S. holder, dividends you receive that are effectively connected with your conduct of a U.S. trade or business may also be subject to a "branch profits tax" at a rate of 30% or such lower rate as may be specified by an applicable income tax treaty.

If you are eligible for a reduced rate of withholding tax pursuant to a tax treaty, you may obtain a refund of any excess amounts withheld if you file an appropriate claim for refund with the IRS in a timely manner.

### **Gain on disposition of common stock**

You generally will not be required to pay U.S. federal income tax on any gain realized upon the sale or other disposition of our common stock unless:

- the gain is effectively connected with your conduct of a U.S. trade or business;
- you are an individual non-U.S. holder who holds our common stock as a capital asset, who is present in the United States for a period or periods aggregating 183 days or more during the calendar year in which the sale or disposition occurs and certain other conditions are met; or
- our common stock constitutes a “U.S. real property interest” by reason of our status as a “United States real property holding corporation” for U.S. federal income tax purposes, or a USRPHC, at any time within the shorter of the five-year period preceding the disposition or your holding period for our common stock.

If you are a non-U.S. holder described in the first bullet above, you will be required to pay tax on the net gain derived from the sale under regular graduated U.S. federal income tax rates. Corporate non-U.S. holders described in the first bullet above may be subject to the “branch profits tax” at a 30% rate or such lower rate as may be specified by an applicable income tax treaty. If you are an individual non-U.S. holder described in the second bullet above, you will be required to pay a flat 30% tax on the gain derived from the sale, which may be offset by U.S.-source capital losses (even though you are not considered a resident of the United States). You should consult any applicable income tax or other treaties, which may provide different rules.

We believe that we are not currently and do not anticipate becoming a USRPHC. However, because the determination of whether we are a USRPHC depends on the fair market value of our U.S. real property relative to the fair market value of our other business assets, there can be no assurance that we will not become a USRPHC in the future. Even if we become a USRPHC, however, as long as our common stock is treated for federal income tax purposes as regularly traded on an established securities market during the applicable calendar year, such common stock will not be treated as “U.S. real property interests” unless you actually or constructively hold more than five percent of such regularly traded common stock at any time during the shorter of the five-year period preceding the disposition or your holding period for our common stock. However, no assurance can be provided that our common stock will be treated as regularly traded on an established securities market for purposes of the rules described above. If we were treated as a USRPHC during the applicable period and the exception described above did not apply, gain on the sale or other taxable disposition of our stock will be subject to tax in the same manner as gain that is effectively connected with the conduct of a U.S. trade or business, except that the “branch profits tax” will not apply.

### **Backup withholding and information reporting**

Generally, we must report annually to the IRS the amount of dividends paid to you, your name and address, and the amount of tax withheld, if any. A similar report will be sent to you. Pursuant to applicable income tax treaties or other agreements, the IRS may make these reports available to tax authorities in your country of residence.

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Payments of dividends or of proceeds on the disposition of stock made to you may be subject to information reporting and backup withholding (currently at a rate of 28%) unless you establish an exemption, for example by properly certifying your non-U.S. status on a Form W-8BEN or another appropriate version of IRS Form W-8. Notwithstanding the foregoing, backup withholding and information reporting may apply if either we or our paying agent has actual knowledge, or reason to know, that you are a U.S. person.

Backup withholding is not an additional tax; rather, the U.S. income tax liability of persons subject to backup withholding will be reduced by the amount of tax withheld. If withholding results in an overpayment of taxes, a refund or credit may be obtained, provided that the required information is furnished to the IRS in a timely manner.

### **FATCA withholding and information reporting**

Legislation enacted in March 2010, commonly referred to as FATCA, will impose United States federal withholding at a rate of 30% on payments to certain non-U.S. entities (including financial intermediaries), including dividends on and the gross proceeds from dispositions of our common stock, unless various information reporting and due diligence requirements, which are different from and in addition to the certification requirements described elsewhere in this discussion, have been satisfied (generally relating to ownership by U.S. persons of interests in or accounts with those entities). The withholding rules applicable to payments of dividends on our common stock will be phased in beginning January 1, 2014. The withholding rules will apply to gross proceeds from dispositions of U.S. common stock beginning January 1, 2017. Although Treasury regulations implementing FATCA were recently finalized, these rules remain unclear in several respects and are subject to material changes. Prospective investors should consult their tax advisors regarding the possible implications of FATCA on their investment in our common stock.

## Underwriting

We are offering the shares of common stock described in this prospectus through a number of underwriters. J.P. Morgan Securities LLC and Merrill Lynch, Pierce, Fenner & Smith Incorporated are acting as joint book-running managers of the offering and as representatives of the underwriters. We have entered into an underwriting agreement with the underwriters. Subject to the terms and conditions of the underwriting agreement, we have agreed to sell to the underwriters, and each underwriter has severally agreed to purchase, at the public offering price less the underwriting discounts and commissions set forth on the cover of this prospectus, the number of shares of common stock listed next to its name in the following table:

<b>Name</b>	<b>Number of shares</b>
J.P. Morgan Securities LLC	
Merrill Lynch, Pierce, Fenner & Smith Incorporated	
Cowen and Company, LLC	
Canaccord Genuity Inc.	
Wedbush Securities Inc.	
<b>Total</b>	

The underwriters are committed to purchase all the shares of common stock offered by us if they purchase any shares. The underwriting agreement also provides that if an underwriter defaults, the purchase commitments of non-defaulting underwriters may also be increased or the offering may be terminated.

The underwriters propose to offer the shares of common stock directly to the public at the initial public offering price set forth on the cover of this prospectus and to certain dealers at that price less a concession not in excess of \$      per share. Any such dealers may resell shares to certain other brokers or dealers at a discount of up to \$      per share from the initial public offering price. After the initial public offering of the shares, the offering price and other selling terms may be changed by the underwriters. Sales of shares made outside of the United States may be made by affiliates of the underwriters.

The underwriters have an option to buy up to      additional shares of common stock from us to cover sales of shares by the underwriters which exceed the number of shares specified in the table above. The underwriters have 30 days from the date of this prospectus to exercise this option to purchase additional shares. If any shares are purchased with this option to purchase additional shares, the underwriters will purchase shares in approximately the same proportion as shown in the table above. If any additional shares of common stock are purchased, the underwriters will offer the additional shares on the same terms as those on which the shares are being offered.

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The underwriting fee is equal to the public offering price per share of common stock less the amount paid by the underwriters to us per share of common stock. The underwriting fee is \$            per share. The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

	<b>Without exercise of option to purchase additional shares</b>	<b>With full exercise of option to purchase additional shares</b>
Per share	\$	\$
<b>Total</b>	<b>\$</b>	<b>\$</b>

We estimate that the total expenses of this offering, including registration, filing and listing fees, printing fees and legal and accounting expenses, but excluding the underwriting discounts and commissions, will be approximately \$            .

A prospectus in electronic format may be made available on the web sites maintained by one or more underwriters, or selling group members, if any, participating in the offering. The underwriters may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters and selling group members that may make Internet distributions on the same basis as other allocations.

We have agreed that we will not (i) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, or file with the Securities and Exchange Commission a registration statement under the Securities Act of 1933, as amended (the "Securities Act"), relating to, any shares of our common stock or any securities convertible into or exchangeable or exercisable for any shares of our common stock, or publicly disclose the intention to make any offer, sale, pledge, disposition or filing, or (ii) enter into any swap or other arrangement that transfers all or a portion of the economic consequences associated with the ownership of any shares of our common stock or any such other securities (regardless of whether any of these transactions are to be settled by the delivery of shares of our common stock or such other securities, in cash or otherwise), in each case without the prior written consent of J.P. Morgan Securities LLC and Merrill Lynch, Pierce, Fenner & Smith Incorporated for a period of 180 days after the date of this prospectus, other than the shares of our common stock to be sold hereunder and any shares of our common stock issued upon the exercise of options granted under our existing management incentive plans.

Our directors and executive officers, and certain of our significant shareholders have entered into lock-up agreements with the underwriters prior to the commencement of this offering pursuant to which each of these persons or entities, with limited exceptions, for a period of 180 days after the date of this prospectus, may not, without the prior written consent of J.P. Morgan Securities LLC and Merrill Lynch, Pierce, Fenner & Smith Incorporated, (1) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for our common stock (including without limitation, common stock or such other

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securities which may be deemed to be beneficially owned by such directors, executive officers and shareholders in accordance with the rules and regulations of the Securities and Exchange Commission and securities which may be issued upon exercise of a stock option or warrant), or publicly disclose the intention to make any offer, sale, pledge or disposition, (2) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of our common stock or such other securities, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of common stock or such other securities, in cash or otherwise or (3) make any demand for or exercise any right with respect to the registration of any shares of our common stock or any security convertible into or exercisable or exchangeable for our common stock.

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act of 1933, as amended.

We have applied to have our common stock approved for listing/quotation on The Nasdaq Global Market under the symbol "BLUE."

In connection with this offering, the underwriters may engage in stabilizing transactions, which involves making bids for, purchasing and selling shares of common stock in the open market for the purpose of preventing or retarding a decline in the market price of the common stock while this offering is in progress. These stabilizing transactions may include making short sales of the common stock, which involves the sale by the underwriters of a greater number of shares of common stock than they are required to purchase in this offering, and purchasing shares of common stock on the open market to cover positions created by short sales. Short sales may be "covered" shorts, which are short positions in an amount not greater than the underwriters' option to purchase additional shares referred to above, or may be "naked" shorts, which are short positions in excess of that amount. The underwriters may close out any covered short position either by exercising their option to purchase additional shares, in whole or in part, or by purchasing shares in the open market. In making this determination, the underwriters will consider, among other things, the price of shares available for purchase in the open market compared to the price at which the underwriters may purchase shares through the option to purchase additional shares. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market that could adversely affect investors who purchase in this offering. To the extent that the underwriters create a naked short position, they will purchase shares in the open market to cover the position.

The underwriters have advised us that, pursuant to Regulation M of the Securities Act, they may also engage in other activities that stabilize, maintain or otherwise affect the price of the common stock, including the imposition of penalty bids. This means that if the representatives of the underwriters purchase common stock in the open market in stabilizing transactions or to cover short sales, the representatives can require the underwriters that sold those shares as part of this offering to repay the underwriting discount received by them.

These activities may have the effect of raising or maintaining the market price of the common stock or preventing or retarding a decline in the market price of the common stock, and, as a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. If the underwriters commence these activities, they may discontinue them at any time. The underwriters may carry out these transactions on The Nasdaq Global Market, in the over-the-counter market or otherwise.

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Prior to this offering, there has been no public market for our common stock. The initial public offering price will be determined by negotiations between us and the representatives of the underwriters. In determining the initial public offering price, we and the representatives of the underwriters expect to consider a number of factors including:

- the information set forth in this prospectus and otherwise available to the representatives;
- our prospects and the history and prospects for the industry in which we compete;
- an assessment of our management;
- our prospects for future earnings;
- the general condition of the securities markets at the time of this offering;
- the recent market prices of, and demand for, publicly traded common stock of generally comparable companies; and
- other factors deemed relevant by the underwriters and us.

Neither we nor the underwriters can assure investors that an active trading market will develop for our common shares, or that the shares will trade in the public market at or above the initial public offering price.

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

This document is only being distributed to and is only directed at (i) persons who are outside the United Kingdom or (ii) to investment professionals falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, or the Order, or (iii) high net worth entities, and other persons to whom it may lawfully be communicated, falling with Article 49(2)(a) to (d) of the Order (all such persons together being referred to as "relevant persons"). The securities are only available to, and any invitation, offer or agreement to subscribe, purchase or otherwise acquire such securities will be engaged in only with, relevant persons. Any person who is not a relevant person should not act or rely on this document or any of its contents.

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive, each, a Relevant Member State, from and including the date on which the European Union Prospectus Directive, or the EU Prospectus Directive, was implemented in that Relevant Member State, or the Relevant Implementation Date, an offer of securities described in this prospectus may not be made to the public in that Relevant Member State prior to the publication of a prospectus in relation to the shares which has been approved by the competent authority in that Relevant Member State or, where appropriate, approved in another Relevant Member State and notified to the competent authority in that Relevant Member State,



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all in accordance with the EU Prospectus Directive, except that, with effect from and including the Relevant Implementation Date, an offer of securities described in this prospectus may be made to the public in that Relevant Member State at any time:

- to any legal entity which is a qualified investor as defined under the EU Prospectus Directive;
- to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150 natural or legal persons (other than qualified investors as defined in the EU Prospectus Directive); or
- in any other circumstances falling within Article 3(2) of the EU Prospectus Directive, provided that no such offer of securities described in this prospectus shall result in a requirement for the publication by us of a prospectus pursuant to Article 3 of the EU Prospectus Directive.

For the purposes of this provision, the expression an “offer of securities to the public” in relation to any securities in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and the securities to be offered so as to enable an investor to decide to purchase or subscribe for the securities, as the same may be varied in that Member State by any measure implementing the EU Prospectus Directive in that Member State. The expression “EU Prospectus Directive” means Directive 2003/71/EC (and any amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State) and includes any relevant implementing measure in each Relevant Member State, and the expression “2010 PD Amending Directive” means Directive 2010/73/EU.

Certain of the underwriters and their affiliates have provided in the past to us and our affiliates and may provide from time to time in the future certain commercial banking, financial advisory, investment banking and other services for us and such affiliates in the ordinary course of their business, for which they have received and may continue to receive customary fees and commissions. In addition, from time to time, certain of the underwriters and their affiliates may effect transactions for their own account or the account of customers, and hold on behalf of themselves or their customers, long or short positions in our debt or equity securities or loans, and may do so in the future.

### **Notice to prospective investors in Switzerland**

The shares may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or the SIX, or on any other stock exchange or regulated trading facility in Switzerland. This document has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this prospectus nor any other offering or marketing material relating to the shares or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this prospectus nor any other offering or marketing material relating to the offering, the Company, the shares have been or will be filed with or approved by any Swiss regulatory authority. In particular, this document will not be filed with, and the offer of shares will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA, and the offer of shares

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has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or the CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of shares.

**Notice to prospective investors in the Dubai International Financial Centre**

This prospectus relates to an Exempt Offer in accordance with the Offered Securities Rules of the Dubai Financial Services Authority, or the DFSA. This prospectus is intended for distribution only to persons of a type specified in the Offered Securities Rules of the DFSA. It must not be delivered to, or relied on by, any other person. The DFSA has no responsibility for reviewing or verifying any documents in connection with Exempt Offers. The DFSA has not approved this prospectus nor taken steps to verify the information set forth herein and has no responsibility for the prospectus. The shares to which this prospectus relates may be illiquid and/or subject to restrictions on their resale. Prospective purchasers of the shares offered should conduct their own due diligence on the shares. If you do not understand the contents of this prospectus you should consult an authorized financial advisor.

## Legal matters

The validity of the issuance of our common stock offered in this prospectus will be passed upon for us by Goodwin Procter LLP, Boston, Massachusetts. Certain legal matters in connection with this offering will be passed upon for the underwriters by Ropes & Gray LLP, Boston, Massachusetts.

## Experts

The consolidated financial statements as of December 31, 2011, and for the year then ended, appearing in this Prospectus and Registration Statement have been audited by McGladrey LLP, an independent registered public accounting firm, as stated in their report appearing elsewhere herein, and are included in reliance upon such report and upon the authority of such firm as experts in accounting and auditing.

The consolidated financial statements of bluebird bio, Inc. at December 31, 2012, and for the year then ended, appearing in this Prospectus and Registration Statement have been audited by Ernst & Young LLP, independent registered public accounting firm, as set forth in their report thereon appearing elsewhere herein, and are included in reliance upon such report given on the authority of such firm as experts in accounting and auditing.

## Where you can find more information

We have filed with the Securities and Exchange Commission, or SEC, a registration statement on Form S-1, or the registration statement, under the Securities Act of 1933, as amended, with respect to the shares of common stock offered hereby. This prospectus, which constitutes a part of the registration statement, does not contain all of the information set forth in the registration statement or the exhibits and schedules filed therewith. For further information with respect to bluebird bio, Inc. and the common stock offered hereby, reference is made to the registration statement and the exhibits and schedules filed therewith. Statements contained in this prospectus regarding the contents of any contract or any other document that is filed as an exhibit to the registration statement are not necessarily complete, and each such statement is qualified in all respects by reference to the full text of such contract or other document filed as an exhibit to the registration statement. A copy of the registration statement and the exhibits and schedules filed therewith may be inspected without charge at the public reference room maintained by the SEC, located at 100 F Street N.E., Washington, D.C. 20549, and copies of all or any part of the registration statement may be obtained from such offices upon the payment of the fees prescribed by the SEC. Please call the SEC at 1-800-SEC-0330 for further information about the public reference room. The SEC also maintains a website that contains reports, proxy and information statements and other information regarding registrants that file electronically with the SEC. The address is [www.sec.gov](http://www.sec.gov).

Upon completion of this offering, we will become subject to the information and periodic reporting requirements of the Securities Exchange Act of 1934, as amended, and, in accordance therewith, will file periodic reports, proxy statements and other information with the SEC. Such periodic reports, proxy statements and other information will be available for inspection and copying at the public reference room and website of the SEC referred to above. We maintain a website at [www.bluebirdbio.com](http://www.bluebirdbio.com). The reference to our website address does not constitute incorporation by reference of the information contained on our website, and you should not consider the contents of our website in making an investment decision with respect to our common stock.

## Glossary

AAV	adeno-associated virus
ACALD	adult cerebral adrenoleukodystrophy, a type of ALD that develops in males 15 years or older
ALD	adrenoleukodystrophy, a rare X-linked, inherited, neurological disorder caused by mutations in the ABCD1 gene
ALDP	ALD protein, a protein that plays a critical role in the breakdown and metabolism of VLCFA
AMN	adrenomyeloneuropathy, the most common form of ALD, typically developing in adults 21 years or older
ANSM	l'agence nationale de sécurité du médicament et des produits de santé (France)
BLA	Biologics License Application
CAR	chimeric antigen receptor
CBER	FDA Center for Biologics Evaluation and Research
CCALD	childhood cerebral adrenoleukodystrophy, the most severe form of ALD, typically developing in boys between ages of 3 and 15
CIRM	California Institute for Regenerative Medicine
CMC	chemical, manufacturing and control
CMS	Centers for Medicare & Medicaid Services, an agency within the U.S. Department of Health and Human Services
CRO	contract research organization
CTA	clinical trial application
CTGTAC	Cellular, Tissue and Gene Therapies Advisory Committee
DNA	deoxyribonucleic acid,
EMA	European Medicines Agency
FDA	U.S. Food and Drug Administration
GCP	good clinical practices
GLP	good laboratory practices
GMP	good manufacturing practices
GTP	good tissue practices
GVHD	graft-versus-host disease
HCT/P	human cells, tissues, and cellular and tissue based product
HIV-1	Human Immunodeficiency Virus Type 1
HLA	Human-Leukocyte-Antigen
HSC	hematopoietic stem cell
HSCT	hematopoietic stem cell transplant, an approach of treating a patient with HSCs contributed by a donor that contain a functioning copy of the gene underlying the disease.
IBC	institutional biosafety committee
IND	Investigational New Drug application
Inserm	institut national de la santé et de la recherché médicale (France), or the French Institute of Health and Medical Research
IRB	institutional review board
IVIM	in vitro immortalization
MAA	Marketing Authorization Application
MFDs	major functional disabilities
MHRA	Medicines and Healthcare Products Regulatory Agency (United Kingdom)

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MRI	magnetic resonance imaging
NDA	new drug application
NFS	Neurological Function Score
NIH	U.S. National Institutes of Health
OBA	NIH Office of Biotechnology Activities
OCTGT	FDA Office of Cellular, Tissue and Gene Therapies
PDCO	EMA Pediatric Committee
PIP	Pediatric Investigation Plan
RAC	NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee
RBC	red blood cell
REMS	Risk Evaluation and Mitigation Strategy
SCD	sickle cell disease
TTCF	ten tray cell factories
VLCFA	very long-chain fatty acids

## bluebird bio, Inc.

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## Report of independent registered public accounting firm

The Board of Directors and Stockholders

bluebird bio, Inc.

We have audited the accompanying consolidated balance sheet of bluebird bio, Inc. as of December 31, 2012 and the related consolidated statement of operations and comprehensive loss, convertible preferred stock and stockholders' (deficit) equity, and cash flows for the year then ended. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audit.

We conducted our audit in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company's internal control over financial reporting. Our audit included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audit provides a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the consolidated financial position of bluebird bio, Inc. at December 31, 2012, and the consolidated results of its operations and its cash flows for the year then ended, in conformity with U.S. generally accepted accounting principles .

/s/ Ernst & Young LLP

Boston, Massachusetts  
March 21, 2013

## Report of independent registered public accounting firm

The Board of Directors and Stockholders of  
bluebird bio, Inc.  
Cambridge, Massachusetts

We have audited the accompanying consolidated balance sheet of bluebird bio, Inc. as of December 31, 2011, and the related consolidated statements of operations and comprehensive loss, convertible preferred stock and stockholders' deficit, and cash flows for the year then ended. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audit.

We conducted our audit in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. Our audit included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audit provides a reasonable basis for our opinion.

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the financial position of bluebird bio, Inc. as of December 31, 2011, and the results of its operations and its cash flows for the year then ended, in conformity with U.S. generally accepted accounting principles.

/s/ McGladrey LLP

Boston, Massachusetts  
March 21, 2013



# bluebird bio, Inc.

## Consolidated balance sheets

(In thousands, except per share data)

	December 31,		Pro forma March 31,	
	2011	2012	2013	2013
	Actual		(unaudited)	
<b>Assets</b>				
Current assets:				
Cash and cash equivalents	\$ 25,604	\$ 67,011	\$131,836	\$131,836
Marketable securities	3,507	—	—	—
Prepaid expenses and other current assets	869	773	3,253	3,253
Total current assets	29,980	67,784	135,089	135,089
Property and equipment, net	728	1,288	2,120	2,120
Restricted cash	210	250	250	250
Total assets	\$ 30,918	\$ 69,322	\$137,459	\$137,459
<b>Liabilities, convertible preferred stock, and stockholders' (deficit) equity</b>				
Current liabilities:				
Accounts payable	\$ 1,793	\$ 2,173	\$ 2,227	\$ 2,227
Accrued expenses and other current liabilities	760	2,115	2,132	2,132
Deferred revenue, current portion	340	340	25,340	25,340
Total current liabilities	2,893	4,628	29,699	29,699
Warrant liability	637	215	256	—
Deferred rent, net of current portion	13	46	46	46
Deferred revenue, net of current portion	679	340	49,213	49,213
Total liabilities	4,222	5,229	79,214	78,958
Commitments and contingencies (Note 8)				
Series A-1 convertible preferred stock, \$0.01 par value, 18,817 shares authorized; 12,981 shares issued and outstanding at December 31, 2011, and no shares issued and outstanding pro forma (unaudited)	9,217	—	—	—
Series A-2 convertible preferred stock, \$0.01 par value, 22,304 shares authorized; 22,304 shares issued and outstanding at December 31, 2011 and 2012 and March 31, 2013 (unaudited), and no shares issued and outstanding pro forma (unaudited) (aggregate liquidation preference of \$12,843)	15,837	7,137	7,137	—
Series B convertible preferred stock, \$0.01 par value, 115,779 shares authorized; 115,204 shares issued and outstanding at December 31, 2011 and 2012 and March 31, 2013 (unaudited), and no shares issued and outstanding pro forma (unaudited) (aggregate liquidation preference of \$56,369)	41,495	40,321	40,321	—
Series C convertible preferred stock, \$0.01 par value, 39,943 shares authorized; 39,943 shares issued and outstanding at December 31, 2011 and 2012 and March 31, 2013 (unaudited), and no shares issued and outstanding pro forma (unaudited) (aggregate liquidation preference of \$15,000)	15,854	12,382	12,382	—
Series D convertible preferred stock, \$0.01 par value, 120,409 shares authorized; no shares, 120,409 and 120,409 shares issued and outstanding at December 31, 2011 and 2012 and March 31, 2013 (unaudited), respectively, and no shares issued and outstanding pro forma (unaudited) (aggregate liquidation preference of \$60,000)	—	60,000	60,000	—
Stockholders' (deficit) equity:				
Series A-1 convertible preferred stock, \$0.01 par value, 18,817 shares authorized; 12,981 shares issued and outstanding at December 31, 2012 and March 31, 2013 (unaudited), and no shares issued and outstanding pro forma (unaudited) (no liquidation preference)	—	2,337	2,337	—
Common stock, \$0.01 par value, 408,000 shares authorized; 3,895, 5,864, and 6,599 shares issued and outstanding at December 31, 2011 and 2012 and March 31, 2013 (unaudited), respectively, and 317,440 shares issued and outstanding pro forma (unaudited)	39	59	66	3,174
Additional paid-in capital	7,695	15,211	15,900	135,225
Accumulated other comprehensive income	1	—	—	—
Accumulated deficit	(63,442)	(73,354)	(79,898)	(79,898)
Total stockholders' (deficit) equity	(55,707)	(55,747)	(61,595)	58,501
Total liabilities, convertible preferred stock and stockholders' (deficit) equity	\$ 30,918	\$ 69,322	\$137,459	\$137,459

See accompanying notes to consolidated financial statements.

**bluebird bio, Inc.****Consolidated statements of operations and comprehensive loss**

(In thousands, except per share data)

	Year ended December 31,		Three months ended March 31,	
	2011	2012	2012	2013
			(unaudited)	
<b>Revenue:</b>				
Collaboration revenue	\$ —	\$ —	\$ —	\$ 1,042
Research and license fees	640	340	85	85
Grant revenue	242	—	—	—
Total revenue	882	340	85	1,127
<b>Expenses:</b>				
Research and development	11,409	17,210	3,858	5,284
General and administrative	4,615	6,846	1,363	2,324
Total expenses	16,024	24,056	5,221	7,608
Loss from operations	(15,142)	(23,716)	(5,136)	(6,481)
<b>Other (expense) income, net:</b>				
Interest income	5	5	1	3
Foreign currency (losses) gains	(100)	13	8	(25)
Re-measurement of warrants	(361)	28	59	(41)
Other (expense) income, net	(456)	46	68	(63)
Net loss	<u>\$(15,598)</u>	<u>\$(23,670)</u>	<u>\$(5,068)</u>	<u>\$(6,544)</u>
<b>Other comprehensive income (loss):</b>				
Foreign currency translation adjustment	72	—	—	—
Unrealized gains (losses) on marketable securities	1	(1)	—	—
Total other comprehensive income (loss)	73	(1)	—	—
Comprehensive loss	<u>\$(15,525)</u>	<u>\$(23,671)</u>	<u>\$(5,068)</u>	<u>\$(6,544)</u>
<b>Reconciliation of net loss to net loss applicable to common stockholders:</b>				
Net loss	<u>\$(15,598)</u>	<u>\$(23,670)</u>	<u>\$(5,068)</u>	<u>\$(6,544)</u>
Accretion and dividends on convertible preferred stock	(4,993)	(3,057)	(1,285)	—
Gain on extinguishment of convertible preferred stock	—	23,114	—	—
Net loss applicable to common stockholders	<u>\$(20,591)</u>	<u>\$(3,613)</u>	<u>\$(6,353)</u>	<u>\$(6,544)</u>
Net loss per share applicable to common stockholders—basic and diluted	<u>\$ (9.01)</u>	<u>\$ (0.73)</u>	<u>\$ (1.50)</u>	<u>\$ (1.05)</u>
Weighted-average number of common shares used in net loss per share applicable to common stockholders—basic and diluted	2,285	4,972	4,236	6,226
Pro forma net loss per share applicable to common stockholders—basic and diluted (unaudited)		<u>\$ (0.10)</u>		<u>\$ (0.02)</u>
Pro forma weighted-average number of common shares used in net loss per share applicable to common stockholders—basic and diluted (unaudited)		<u>248,700</u>		<u>317,067</u>

See accompanying notes to consolidated financial statements.

# bluebird bio, Inc.

## Consolidated statements of convertible preferred stock and stockholders' (deficit) equity

(In thousands)

	Series A-1 convertible preferred stock		Series A-2 convertible preferred stock		Series B convertible preferred stock		Series C convertible preferred stock		Series D convertible preferred stock		Series A-1 convertible preferred stock		Common stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' (deficit) equity
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at December 31, 2010	12,981	\$ 8,760	22,304	\$15,246	61,556	\$ 21,000	—	\$ —	—	\$ —	—	\$ —	1,576	\$ 16	\$ 11,817	\$ (72)	\$(47,844)	\$ (36,083)
Issuance of Series C Preferred Stock, net of issuance costs	—	—	—	—	—	—	39,943	14,904	—	—	—	—	—	—	—	—	—	—
Issuance of Series B Preferred Stock	—	—	—	—	53,648	17,500	—	—	—	—	—	—	—	—	—	—	—	—
Accretion and dividends on convertible preferred stock	—	457	—	591	—	2,995	—	950	—	—	—	—	—	—	(4,993)	—	—	(4,993)
Issuance of restricted stock	—	—	—	—	—	—	—	—	—	—	—	—	273	3	27	—	—	30
Issuance of restricted stock in exchange for consulting services	—	—	—	—	—	—	—	—	—	—	—	—	50	1	2	—	—	3
Vesting of restricted stock issued in exchange for nonrecourse note	—	—	—	—	—	—	—	—	—	—	—	—	1,822	18	(18)	—	—	—
Vesting of restricted stock	—	—	—	—	—	—	—	—	—	—	—	—	142	1	(1)	—	—	—
Issuance of warrants for Common Stock in exchange for consulting services	—	—	—	—	—	—	—	—	—	—	—	—	—	—	102	—	—	102
Exercise of stock options	—	—	—	—	—	—	—	—	—	—	—	—	32	—	1	—	—	1
Foreign currency translation adjustment	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	72	—	72
Unrealized gain on marketable securities	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	1	—	1
Stock-based compensation	—	—	—	—	—	—	—	—	—	—	—	—	—	—	758	—	—	758
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(15,598)	(15,598)
Balance at December 31, 2011	12,981	\$ 9,217	22,304	\$15,837	115,204	\$41,495	39,943	\$15,854	—	\$ —	—	\$ —	3,895	\$ 39	\$ 7,695	\$ 1	\$(63,442)	\$(55,707)

# bluebird bio, Inc.

## Consolidated statements of convertible preferred stock and stockholders' (deficit) equity

(In thousands)

	Series A-1 convertible preferred stock		Series A-2 convertible preferred stock		Series B convertible preferred stock		Series C convertible preferred stock		Series D convertible preferred stock		Series A-1 convertible preferred stock		Common stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' (deficit) equity
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at December 31, 2011	12,981	\$ 9,217	22,304	\$15,837	115,204	\$41,495	39,943	\$15,854	—	\$ —	—	\$ —	3,895	\$ 39	\$ 7,695	\$ 1	\$(63,442)	\$(55,707)
Issuance of Series D Preferred Stock, net of issuance costs	—	—	—	—	—	—	—	—	120,409	59,831	—	—	—	—	—	—	—	—
Accretion and dividends on convertible preferred stock	—	194	—	332	—	1,689	—	673	—	169	—	—	—	—	(3,057)	—	—	(3,057)
Gain on extinguishment of convertible preferred stock	—	(7,074)	—	(9,032)	—	(2,863)	—	(4,145)	—	—	—	—	—	—	9,356	—	13,758	23,114
Reclassification of Series A-1 Preferred Stock	(12,981)	(2,337)	—	—	—	—	—	—	—	—	12,981	2,337	—	—	—	—	—	2,337
Reclassification of Series A-1 Preferred Stock warrants	—	—	—	—	—	—	—	—	—	—	—	—	—	394	—	—	—	394
Vesting of restricted stock issued in exchange for nonrecourse note	—	—	—	—	—	—	—	—	—	—	—	—	1,561	16	(16)	—	—	—
Vesting of restricted stock	—	—	—	—	—	—	—	—	—	—	—	—	223	2	(2)	—	—	—
Exercise of stock options	—	—	—	—	—	—	—	—	—	—	—	—	185	2	19	—	—	21
Realized gain on marketable securities	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(1)	—	(1)
Stock-based compensation	—	—	—	—	—	—	—	—	—	—	—	—	—	—	822	—	—	822
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(23,670)	(23,670)
Balance at December 31, 2012	—	\$ —	22,304	\$ 7,137	115,204	\$ 40,321	39,943	\$ 12,382	120,409	\$ 60,000	12,981	\$ 2,337	5,864	\$ 59	\$ 15,211	\$ —	\$(73,354)	\$(55,747)

# bluebird bio, Inc.

## Consolidated statements of convertible preferred stock and stockholders' (deficit) equity

(In thousands)

	Series A-1 convertible preferred stock		Series A-2 convertible preferred stock		Series B convertible preferred stock		Series C convertible preferred stock		Series D convertible preferred stock		Series A-1 convertible preferred stock		Common stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' (deficit) equity
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at December 31, 2012	—	\$ —	22,304	\$ 7,137	115,204	\$ 40,321	39,943	\$ 12,382	120,409	\$ 60,000	12,981	\$ 2,337	5,864	\$ 59	\$ 15,211	\$ —	\$ (73,354)	\$ (55,747)
Vesting of restricted stock issued in exchange for nonrecourse note (unaudited)	—	—	—	—	—	—	—	—	—	—	—	—	390	4	(4)	—	—	—
Vesting of restricted stock (unaudited)	—	—	—	—	—	—	—	—	—	—	—	—	35	—	—	—	—	—
Exercise of stock options (unaudited)	—	—	—	—	—	—	—	—	—	—	—	—	310	3	32	—	—	35
Stock-based compensation (unaudited)	—	—	—	—	—	—	—	—	—	—	—	—	—	—	661	—	—	661
Net loss (unaudited)	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(6,544)	(6,544)
Balance at March 31, 2013 (unaudited)	—	\$ —	22,304	\$ 7,137	115,204	\$ 40,321	39,943	\$ 12,382	120,409	\$ 60,000	12,981	\$ 2,337	6,599	\$ 66	\$ 15,900	\$ —	\$ (79,898)	\$ (61,595)
Conversion of convertible preferred stock into common stock (unaudited)	—	—	(22,304)	(7,137)	(115,204)	(40,321)	(39,943)	(12,382)	(120,409)	(60,000)	(12,981)	(2,337)	310,841	3,108	119,069	—	—	119,840
Reclassification of warrants to purchase preferred stock to stockholders' equity (unaudited)	—	—	—	—	—	—	—	—	—	—	—	—	—	—	256	—	—	256
Pro forma balance at March 31, 2013 (unaudited)	—	\$ —	—	\$ —	—	\$ —	—	\$ —	—	\$ —	—	\$ —	317,440	\$ 3,174	\$ 135,225	\$ —	\$ (79,898)	\$ 58,501

See accompanying notes to consolidated financial statements.

# bluebird bio, Inc.

## Consolidated statements of cash flows

(In thousands)

	Year ended December 31,		Three months ended March 31,	
	2011	2012	2012 (unaudited)	2013
<b>Operating activities</b>				
Net loss	\$(15,598)	\$(23,670)	\$(5,068)	\$(6,544)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation	205	301	56	139
Stock-based compensation expense	758	822	233	661
Issuance of common stock warrants in exchange for consulting services	102	—	—	—
Issuance of restricted common stock in exchange for consulting services	3	—	—	—
Re-measurement of warrants	361	(28)	(59)	41
Amortization of premium on marketable securities	20	—	—	—
Loss on disposal of equipment	—	10	—	2
Changes in operating assets and liabilities:				
Prepaid expenses and other current assets	(337)	93	(522)	(1,073)
Accounts payable	870	380	(1,133)	(593)
Accrued expenses and other liabilities	380	1,388	378	(488)
Deferred revenue	1,019	(340)	(85)	73,873
Net cash (used in) provided by operating activities	<u>(12,217)</u>	<u>(21,044)</u>	<u>(6,200)</u>	<u>66,018</u>
<b>Investing activities</b>				
Restricted cash	(35)	(40)	(40)	—
Purchase of property and equipment	(403)	(867)	(291)	(812)
Purchase of marketable securities	(5,276)	—	—	—
Proceeds from sales or maturities of marketable securities	1,750	3,506	3,506	—
Net cash (used in) provided by investing activities	<u>(3,964)</u>	<u>2,599</u>	<u>3,175</u>	<u>(812)</u>
<b>Financing activities</b>				
Accumulated issuance costs of planned initial public offering	—	—	—	(415)
Proceeds from sale of convertible preferred stock, net of issuance costs	32,404	59,831	—	—
Proceeds from sale of restricted stock, net of issuance costs	30	—	—	—
Proceeds from issuance of common stock	1	21	—	34
Net cash provided by (used in) financing activities	<u>32,435</u>	<u>59,852</u>	<u>—</u>	<u>(381)</u>
Increase (decrease) in cash and cash equivalents	16,254	41,407	(3,025)	64,825
Cash and cash equivalents at beginning of period	9,350	25,604	25,604	67,011
Cash and cash equivalents at end of period	<u>\$ 25,604</u>	<u>\$ 67,011</u>	<u>\$ 22,579</u>	<u>\$ 131,836</u>
<b>Non-cash investing and financing activities:</b>				
Fixed asset additions included in accounts payable and accrued expenses	\$ —	\$ —	\$ —	\$ 160
Deferred issuance costs included in accounts payable and accrued expenses	\$ —	\$ —	\$ —	\$ 992
Accretion and dividends on convertible preferred stock	\$ 4,993	\$ 3,057	\$ 1,285	\$ —
Gain on extinguishment of convertible preferred stock	\$ —	\$ 23,114	\$ —	\$ —
Reclassification of warrants to purchase Series A-1 Preferred Stock to common stock	\$ —	\$ 394	\$ —	\$ —
Reclassification of Series A-1 Preferred Stock to common stock	\$ —	\$ 2,337	\$ —	\$ —

See accompanying notes to consolidated financial statements.

# bluebird bio, Inc.

## Notes to consolidated financial statements

Amounts as of March 31, 2013 and for the three months ended March 31, 2013 and 2012 are unaudited  
(In thousands, except per share data)

### 1. Nature of business

bluebird bio, Inc. (the “Company”) was incorporated in Delaware on April 16, 1992, and is headquartered in Cambridge, Massachusetts. The Company was formed to develop, manufacture and market therapies to safely and effectively deliver genes useful in the treatment of serious human diseases.

The Company has generated an accumulated deficit of \$79,898 (unaudited) since inception and will require substantial additional capital to fund its research and development. It is subject to risks common to companies in the biotechnology industry, including, but not limited to, development by the Company or its competitors of technological innovations, risks of failure of clinical studies, dependence on key personnel, protection of proprietary technology, compliance with government regulations and ability to transition from pilot-scale manufacturing to large-scale production of products.

#### *Liquidity*

The Company believes that its cash resources of \$131,836 at March 31, 2013 (unaudited), will be sufficient to allow the Company to fund its current operating plan for at least the next 12 months. As the Company continues to incur losses, transition to profitability is dependent upon the successful development, approval, and commercialization of its product candidates and achieving a level of revenues adequate to support the Company’s cost structure. The Company may never achieve profitability, and unless and until it does, the Company will continue to need to raise additional capital. Management intends to fund future operations through additional private or public debt or equity offerings, and may seek additional capital through arrangements with strategic partners or from other sources. There can be no assurances, however, that additional funding will be available on terms acceptable to the Company, or at all.

### 2. Summary of significant accounting policies

#### *Basis of presentation and principles of consolidation*

The accompanying consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries, bluebird bio France, SARL, and bluebird bio Securities Corporation. All intercompany balances and transactions have been eliminated in consolidation. These consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America (“GAAP”). Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification (“ASC”) and Accounting Standards Update (“ASU”) of the Financial Accounting Standards Board (“FASB”).

# bluebird bio, Inc.

## Notes to consolidated financial statements

(In thousands, except per share data)

### ***Reclassifications***

The Company has reclassified certain prior period amounts to conform to the current period presentation. The amounts reclassified impact prepaid expenses and other current assets, accounts payable, and accrued expenses for the year ended December 31, 2011.

### ***Foreign currency translation***

The Company's consolidated financial statements are prepared in U.S. dollars. Its foreign subsidiary uses the U.S. dollar as its functional currency and maintains its records in the local currency. Nonmonetary assets and liabilities are re-measured at historical rates and monetary assets and liabilities are re-measured at exchange rates in effect at the end of the reporting period. Income statement accounts are re-measured at average exchange rates for the reporting period. The resulting gains or losses are included in foreign currency (losses) gains in the consolidated statements of operations and comprehensive loss.

### ***Unaudited interim financial information***

The accompanying consolidated balance sheet as of March 31, 2013, the consolidated statements of operations and comprehensive loss and statements of cash flows for the three months ended March 31, 2012 and 2013, and the statement of convertible preferred stock and stockholders' deficit for the three months ended March 31, 2013, are unaudited. The interim unaudited financial statements have been prepared on the same basis as the annual audited financial statements; and in the opinion of management, reflect all adjustments, which include only normal recurring adjustments necessary for the fair statement of the Company's financial position as of March 31, 2013, and the results of its operations and comprehensive loss and its cash flows for the three months ended March 31, 2012 and 2013. The financial data and other information disclosed in these notes related to the three months ended March 31, 2012 and 2013, are unaudited. The results for the three months ended March 31, 2013, are not necessarily indicative of results to be expected for the year ending December 31, 2013, any other interim periods, or any future year or period.

### ***Unaudited pro forma information***

On February 6, 2013, the Company's board of directors authorized the management of the Company to file a registration statement with the Securities and Exchange Commission ("SEC") for the Company to sell shares of its common stock (the "Common Stock") to the public. The unaudited pro forma balance sheet as of December 31, 2012, assumes the automatic conversion of all the outstanding convertible preferred stock into shares of Common Stock upon the completion of this proposed offering and the reclassification of the Company's outstanding warrants to purchase shares of Series B convertible preferred stock ("Series B Preferred Stock") from a liability to equity, occurring upon the closing of the Company's proposed initial public offering.

Unaudited pro forma net loss per share applicable to common stockholders is computed using the weighted-average number of common shares outstanding after giving effect to the



# bluebird bio, Inc.

## Notes to consolidated financial statements

(In thousands, except per share data)

conversion of all the outstanding convertible preferred stock into shares of Common Stock as if such conversion had occurred at the beginning of the period presented, or the date of original issuance, if later, and excludes the gain on extinguishment of preferred stock and the accretion of dividends.

### ***Use of estimates***

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts in the financial statements and accompanying notes. Actual results could materially differ from those estimates. Management considers many factors in selecting appropriate financial accounting policies and controls, and in developing the estimates and assumptions that are used in the preparation of these financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including: expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates, and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes and management must select an amount that falls within that range of reasonable estimates. This process may result in actual results differing materially from those estimated amounts used in the preparation of the financial statements. Estimates are used in the following areas, among others: stock-based compensation expense, fair value of Common Stock and convertible preferred stock, liability-classified warrants, accrued expenses, and income taxes.

The Company utilized various valuation methodologies in accordance with the framework of the American Institute of Certified Public Accountants' Technical Practice Aid, *Valuation of Privately-Held Company Equity Securities Issued as Compensation*, to estimate the fair value of its Common Stock. Each valuation methodology includes estimates and assumptions that require the Company's judgment. These estimates and assumptions include a number of objective and subjective factors, including external market conditions affecting the biotechnology industry sector, the prices at which the Company sold shares of preferred stock, the superior rights and preferences of securities senior to the Common Stock at the time and the likelihood of achieving a liquidity event, such as an initial public offering or sale. Significant changes to the key assumptions used in the valuations could result in different fair values of Common Stock at each valuation date.

### ***Segment information***

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision maker, or decision making group, in making decisions on how to allocate resources and assess performance. The Company views its operations and manages its business in one operating segment. All material long-lived assets of the Company reside in the United States.

# bluebird bio, Inc.

## Notes to consolidated financial statements

(In thousands, except per share data)

### ***Cash and cash equivalents***

The Company considers all highly liquid investments purchased with original maturities of 90 days or less at acquisition to be cash equivalents. Cash and cash equivalents include cash held in banks and money market accounts. Cash equivalents are reported at fair value.

### ***Concentrations of credit risk and off-balance sheet risk***

Financial instruments that potentially subject the Company to concentrations of credit risk are primarily cash, cash equivalents and restricted cash. The Company maintains its cash and cash equivalent balances in the form of money market accounts with financial institutions that management believes are creditworthy. The Company's investment policy includes guidelines on the quality of the institutions and financial instruments and defines allowable investments that the Company believes minimizes the exposure to concentration of credit risk. The Company has no financial instruments with off-balance sheet risk of loss.

### ***Marketable securities***

The Company maintains funds in an account that invests in marketable securities. Management determines the appropriate classification of the securities at the time they are acquired and evaluates the appropriateness of such classification at each balance sheet date. Available-for-sale securities consist of debt securities and are stated at fair value. Unrealized holding gains and losses are reported in the consolidated statements of operations and comprehensive loss. Premiums and discounts on investments in debt securities are amortized over the contractual lives of those securities. The method of amortization results in a constant effective yield on those securities (the effective interest method). Interest on debt securities is recognized in income as earned. Realized gains and losses, including losses from declines in value of specific securities determined by management to be other-than-temporary, are included in income. Realized gains and losses are determined on the basis of the specific cost of the securities sold. As of December 31, 2011, all marketable securities were classified as available-for-sale securities due to their short-term nature (maturity dates within one year of the balance sheet date). The Company did not have any marketable securities as of December 31, 2012 and March 31, 2013 (unaudited).

### ***Deferred issuance costs***

Deferred issuance costs, which primarily consist of direct incremental legal and accounting fees relating to the initial public offering ("IPO"), are capitalized. The deferred issuance costs will be offset against IPO proceeds upon the consummation of the offering. In the event the offering is terminated, deferred offering costs will be expensed. As of March 31, 2013 (unaudited), the Company capitalized \$1,407 of deferred offering costs, which are included in prepaid expenses and other current assets on the consolidated balance sheet. No amounts were deferred as of December 31, 2012 or 2011.

# bluebird bio, Inc.

## Notes to consolidated financial statements

(In thousands, except per share data)

### ***Fair value of financial instruments***

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. FASB ASC Topic 820, *Fair Value Measurements and Disclosures* ("ASC 820"), establishes a hierarchy of inputs used in measuring fair value that maximizes the use of observable inputs and minimizes the use of unobservable inputs by requiring that the observable inputs be used when available. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability, and are developed based on the best information available in the circumstances. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments and is not a measure of the investment credit quality. The three levels of the fair value hierarchy are described below:

Level 1—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

Level 2—Valuations based on quoted prices for similar assets or liabilities in markets that are not active or for which all significant inputs are observable, either directly or indirectly.

Level 3—Valuations that require inputs that reflect the Company's own assumptions that are both significant to the fair value measurement and unobservable.

To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

Items measured at fair value on a recurring basis include marketable securities (Note 3) and warrant liability (Note 7). The carrying amounts of accounts payable and accrued expenses approximate their fair values due to their short-term maturities.

### ***Property and equipment***

Property and equipment is stated at cost. Maintenance and repairs that do not improve or extend the lives of the respective assets are expensed to operations as incurred. Upon disposal, the related cost and accumulated depreciation is removed from the accounts and any resulting gain or loss is included in the results of operations. Upon retirement or sale, the cost of the disposed asset and the related accumulated depreciation are removed from the accounts and any

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## Notes to consolidated financial statements

(In thousands, except per share data)

resulting gain or loss is recognized. Depreciation and amortization is calculated using the straight-line method over the estimated useful lives of the assets, which are as follows:

Asset	Estimated useful life
Computer equipment and software	3 years
Office and laboratory equipment	3 to 5 years
Leasehold improvements	Shorter of the useful life or remaining lease term

### ***Impairment of long-lived assets***

The Company reviews long-lived assets when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. Recoverability is measured by comparison of the book values of the assets to future net undiscounted cash flows that the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the book value of the assets exceed their fair value, which is measured based on the projected discounted future net cash flows arising from the assets. No impairment losses have been recorded during the years ended December 31, 2011 and 2012 and three months ended March 31, 2013 (unaudited).

### ***Warrants to purchase convertible preferred stock***

In conjunction with various financing transactions, the Company issued warrants to purchase shares of the Company's Series A-1 convertible preferred stock ("Series A-1 Preferred Stock") and Series B Preferred Stock. Prior to July 23, 2012, the Company's Series A-1 Preferred Stock and Series B Preferred Stock were subject to a redemption provision that was outside of the Company's control. Therefore, the associated shares were presented as temporary equity. Consequently, the warrants to purchase shares of Series A-1 Preferred Stock and Series B Preferred Stock were accounted for as liabilities through July 23, 2012, and adjusted to fair value at the end of each reporting period. The fair value of the warrants classified as liabilities is estimated using the Black-Scholes option pricing model. The estimates in the Black-Scholes option pricing model are based, in part, on subjective assumptions, including, stock price volatility, term of the warrants, risk free interest rate, dividend yield, and fair value of the preferred stock underlying the warrants. Such assumptions could differ materially in the future. The re-measurement gain or loss associated with the change in the fair value of the preferred stock warrant liability from the prior period is recognized as a component of other (expense) income, net.

On July 23, 2012, in connection with the sale of the Company's Series D convertible preferred stock ("Series D Preferred Stock") and the associated modifications to the rights, preferences and privileges of the then-existing series of preferred stock, the Series A-1 Preferred Stock was reclassified to permanent equity because the redemption rights were relinquished and no liquidation preferences were obtained. Additionally, the fair value of the warrants to purchase shares of Series A-1 Preferred Stock as of July 23, 2012 were correspondingly reclassified to additional paid-in capital consistent with the treatment of the associated shares of preferred stock. All other classes of preferred stock remain classified within temporary equity as of

# bluebird bio, Inc.

## Notes to consolidated financial statements

(In thousands, except per share data)

December 31, 2012, due to their associated liquidation preferences. Due to these remaining liquidating preferences, the warrants to purchase shares of Series B Preferred Stock remain classified within liabilities as of December 31, 2012 and March 31, 2013 (unaudited).

The Company will continue to re-measure the fair value of the liability associated with the warrants to purchase shares of Series B Preferred Stock at the end of each reporting period until the earlier of the exercise or expiration of the applicable warrants or until such time that the underlying preferred stock is reclassified to permanent equity.

### **Revenue recognition**

The Company has primarily generated revenue through collaboration arrangements, research arrangements and license arrangements with strategic partners and nonprofit organizations for the development and commercialization of product candidates. Additionally, the Company has generated revenue from research and development grant programs.

The Company recognizes revenue in accordance with FASB ASC Topic 605, *Revenue Recognition* ("ASC 605"). Accordingly, revenue is recognized for each unit of accounting when all of the following criteria are met:

- Persuasive evidence of an arrangement exists
- Delivery has occurred or services have been rendered
- The seller's price to the buyer is fixed or determinable
- Collectability is reasonably assured

Amounts received prior to satisfying the revenue recognition criteria are recorded as deferred revenue in the Company's consolidated balance sheets. Amounts expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, current portion. Amounts not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, net of current portion.

### *Collaboration revenue*

As of March 31, 2013, the Company's collaboration revenue is generated exclusively from its collaboration arrangement with Celgene Corporation ("Celgene"). The terms of this arrangement contain multiple deliverables, which include at inception: (i) discovery, research and development services, (ii) participation on the joint steering committee and (iii) participation on the patent committee. The collaboration arrangement also provides Celgene with the option to obtain a license to any product candidates resulting from the collaboration. Moreover, Celgene has the option to extend the term of the collaboration arrangement, first for a period of two years and then for an additional period of one year. Additionally, the Company has the sole right to manufacture or have manufactured supplies of vectors and associated payloads manufactured for incorporation into the associated product candidate in the event a product candidate is licensed. Non-refundable payments to the Company under this arrangement may include: (i) up-front research fees, (ii) product candidate license fees, (iii) extension term research fees, (iv) payments for the manufacture and supply of vectors and payloads, (v) payments based on the

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## Notes to consolidated financial statements

(In thousands, except per share data)

achievement of certain milestones and (vi) royalties on product sales. Additionally, the Company may elect to share in the costs incurred from the development, commercialization and manufacture of product candidates licensed by its collaborators and earn its share of the net profits or bear its share of the net losses generated from the sale of product candidates licensed by its collaborators.

The Company analyzes multiple-element arrangements based on the guidance in FASB ASC Topic 605-25, *Revenue Recognition-Multiple-Element Arrangements* ("ASC 605-25"). Pursuant to the guidance in ASC 605-25, the Company evaluates multiple-element arrangements to determine (1) the deliverables included in the arrangement and (2) whether the individual deliverables represent separate units of accounting or whether they must be accounted for as a combined unit of accounting. This evaluation involves subjective determinations and requires management to make judgments about the individual deliverables and whether such deliverables are separable from the other aspects of the contractual relationship. Deliverables are considered separate units of accounting provided that: (i) the delivered item(s) has value to the customer on a standalone basis and (ii) if the arrangement includes a general right of return relative to the delivered item(s), delivery or performance of the undelivered item(s) is considered probable and substantially in the control of the Company. In assessing whether an item has standalone value, the Company considers factors such as the research, manufacturing and commercialization capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. In addition, the Company considers whether the collaboration partner can use the other deliverable(s) for their intended purpose without the receipt of the remaining element(s), whether the value of the deliverable is dependent on the undelivered item(s) and whether there are other vendors that can provide the undelivered element(s). The Company's collaboration arrangement does not contain a general right of return relative to the delivered item(s).

Arrangement consideration that is fixed or determinable is allocated among the separate units of accounting using the relative selling price method. Then, the applicable revenue recognition criteria in ASC 605 are applied to each of the separate units of accounting in determining the appropriate period and pattern of recognition. The Company determines the selling price of a unit of accounting following the hierarchy of evidence prescribed by ASC 605-25. Accordingly, the Company determines the estimated selling price for units of accounting within each arrangement using vendor-specific objective evidence ("VSOE") of selling price, if available, third-party evidence ("TPE") of selling price if VSOE is not available, or best estimate of selling price ("BESP") if neither VSOE nor TPE is available. The Company typically uses BESP to estimate the selling price, since it generally does not have VSOE or TPE of selling price for its units of accounting. Determining the BESP for a unit of accounting requires significant judgment. In developing the BESP for a unit of accounting, the Company considers applicable market conditions and relevant entity-specific factors, including factors that were contemplated in negotiating the agreement with the customer and estimated costs. The Company validates the BESP for units of accounting by evaluating whether changes in the key assumptions used to determine the BESP will have a significant effect on the allocation of arrangement consideration between multiple units of accounting.

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(In thousands, except per share data)

Options are considered substantive if, at the inception of the arrangement, the Company is at risk as to whether the collaboration partner will choose to exercise the option. Factors that the Company considers in evaluating whether an option is substantive include the overall objective of the arrangement, the benefit the collaborator might obtain from the arrangement without exercising the option, the cost to exercise the option and the likelihood that the option will be exercised. For arrangements under which an option is considered substantive, the Company does not consider the item underlying the option to be a deliverable at the inception of the arrangement and the associated option fees are not included in allocable arrangement consideration, assuming the option is not priced at a significant and incremental discount. Conversely, for arrangements under which an option is not considered substantive or if an option is priced at a significant and incremental discount, the Company would consider the item underlying the option to be a deliverable at the inception of the arrangement and a corresponding amount would be included in allocable arrangement consideration. All of the options included in the Company's collaboration arrangement have been determined to be substantive, and none of the options are priced at a significant and incremental discount.

The Company recognizes arrangement consideration allocated to each unit of accounting when all of the revenue recognition criteria in ASC 605 are satisfied for that particular unit of accounting. The Company will recognize as revenue arrangement consideration attributed to licenses that have standalone value from the other deliverables to be provided in an arrangement upon delivery. The Company will recognize as revenue arrangement consideration attributed to licenses that do not have standalone value from the other deliverables to be provided in an arrangement over the Company's estimated performance period as the arrangement would be accounted for as a single unit of accounting.

The Company recognizes revenue from the Celgene arrangement associated with discovery, research and development services, joint steering committee services and patent committee services ratably over the associated period of performance. If there is no discernible pattern of performance and/or objectively measurable performance measures do not exist, then the Company recognizes revenue under the arrangement on a straight-line basis over the period the Company is expected to complete its performance obligations. Conversely, if the pattern of performance in which the service is provided to the customer can be determined and objectively measurable performance measures exist, then the Company recognizes revenue under the arrangement using the proportional performance method. Revenue recognized is limited to the lesser of the cumulative amount of payments received or the cumulative amount of revenue earned, as determined using the straight-line method or proportional performance method, as applicable, as of the period ending date.

At the inception of an arrangement that includes milestone payments, the Company evaluates whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This evaluation includes an assessment of whether: (i) the consideration is commensurate with either the Company's performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the Company's performance to achieve the milestone, (ii) the consideration relates solely to past performance and (iii) the consideration is reasonable relative to all of the

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## Notes to consolidated financial statements

(In thousands, except per share data)

deliverables and payment terms within the arrangement. The Company evaluates factors such as the scientific, clinical, regulatory, commercial and other risks that must be overcome to achieve the respective milestone and the level of effort and investment required to achieve the respective milestone in making this assessment. There is considerable judgment involved in determining whether a milestone satisfies all of the criteria required to conclude that a milestone is substantive. The Company has concluded that all of the clinical and regulatory milestones pursuant to its collaboration arrangement are substantive. Accordingly, in accordance with FASB ASC Topic 605-28, *Revenue Recognition-Milestone Method* ("ASC 605-28"), revenue from clinical and regulatory milestone payments will be recognized in its entirety upon successful accomplishment of the milestone, assuming all other revenue recognition criteria are met. Milestones that are not considered substantive would be recognized as revenue over the remaining period of performance, assuming all other revenue recognition criteria are met. Revenue from commercial milestone payments will be accounted for as royalties and recorded as revenue upon achievement of the milestone, assuming all other revenue recognition criteria are met.

The Company will recognize royalty revenue in the period of sale of the related product(s), based on the underlying contract terms, provided that the reported sales are reliably measurable and the Company has no remaining performance obligations, assuming all other revenue recognition criteria are met.

### *Research fees and license fees*

The terms of the Company's research agreements and license agreements include delivery of an intellectual property license or the performance of research and development activities. The Company does not have any material research arrangements or license arrangements that contain multiple deliverables. The Company is compensated under research arrangements and license arrangements through nonrefundable up-front payments and future royalties on net product sales. Research fees are recognized as revenue on a straight-line basis over the period that the research services are expected to be performed unless the Company's pattern of performance can be determined to be other than straight-line, in which case, the Company uses the proportional performance method. Nonrefundable license fees are recognized as revenue upon delivery provided there are no undelivered elements in the arrangement.

### *Grant revenue*

Grant revenue is primarily generated through research and development grant programs offered by federal, state, and local governments. The Company evaluates the terms of the grant to assess the Company's obligations and if the Company's obligations are satisfied over time, revenue is recognized on a straight-line basis. In situations where the performance of the Company's obligations has been satisfied when the grant is received, revenue is recognized upon receipt of the grant. Certain grants contain refund provisions. The Company reviews those refund provisions to determine the likelihood of repayment. If the likelihood of repayment of the grant is determined to be remote, the grant is recognized as revenue. If the probability of repayment is determined to be more than remote, the Company records the grant as a liability, until such time that the grant requirements have been satisfied.



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(In thousands, except per share data)

### **Research and development expenses**

Research and development costs are charged to expense as costs are incurred in performing research and development activities, including salaries and benefits, facilities costs, overhead costs, clinical study and related clinical manufacturing costs, contract services and other related costs. Research and development costs, including up-front fees and milestones paid to collaborators, are also expensed as incurred.

### **Stock-based compensation**

The Company accounts for its stock-based compensation awards in accordance with FASB ASC Topic 718, *Compensation—Stock Compensation* (“ASC 718”). ASC 718 requires all stock-based payments to employees, including grants of employee stock options and restricted stock and modifications to existing stock options, to be recognized in the consolidated statements of operations and comprehensive loss based on their fair values. The Company uses the Black-Scholes option pricing model to determine the fair value of options granted.

Compensation expense related to awards to employees is recognized on a straight-line basis based on the grant date fair value over the associated service period of the award, which is generally the vesting term. Awards to non-employees are adjusted through share-based compensation expense as the award vests to reflect the current fair value of such awards, and expensed using an accelerated attribution model.

The Company expenses restricted stock awards based on the fair value of the award on a straight-line basis over the associated service period of the award. Awards of restricted stock to non-employees are adjusted through share-based compensation expense at each reporting period end to reflect the current fair value of such awards and expensed using an accelerated attribution model.

The Company estimates the fair value of its stock-based awards to employees and directors using the Black-Scholes option pricing model, which requires the input of and subjective assumptions, including (a) the expected stock price volatility, (b) the calculation of expected term of the award, (c) the risk-free interest rate, and (d) expected dividends. Due to the lack of a public market for the trading of its Common Stock and a lack of company specific historical and implied volatility data, the Company has based its estimate of expected volatility on the historical volatility of a group of similar companies that are publicly traded. Due to the lack of a public market for the trading of the Company's common stock and a lack of company-specific historical and implied volatility data, the Company has based its estimate of expected volatility on the historical volatility of a group of similar companies that are publicly traded. When selecting these public companies on which it has based its expected stock price volatility, the Company selected companies with comparable characteristics to it, including enterprise value, risk profiles, position within the industry, and with historical share price information sufficient to meet the expected term of the stock-based awards. The Company computes historical volatility data using the daily closing prices for the selected companies' shares during the equivalent period of the calculated expected term of the stock-based awards. The Company will continue to apply this process until a sufficient amount of historical information regarding the volatility of its own stock price becomes

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(In thousands, except per share data)

available. The Company has estimated the expected term of its employee stock options using the “simplified” method, whereby, the expected term equals the arithmetic average of the vesting term and the original contractual term of the option due to its lack of sufficient historical data. The risk-free interest rates for periods within the expected term of the option are based on the U.S. Treasury securities with a maturity date commensurate with the expected term of the associated award. The Company has never paid, and does not expect to pay dividends in the foreseeable future.

The Company is also required to estimate forfeitures at the time of grant, and revise those estimates in subsequent periods if actual forfeitures differ from its estimates. The Company uses historical data to estimate pre-vesting option forfeitures and records stock-based compensation expense only for those awards that are expected to vest. To the extent that actual forfeitures differ from the Company’s estimates, the differences are recorded as a cumulative adjustment in the period the estimates were revised. Stock-based compensation expense recognized in the financial statements is based on awards that are ultimately expected to vest.

Consistent with the guidance in FASB ASC Topic 505-50, *Equity-Based Payments to Non-Employees*, the fair value of each non-employee stock option and warrant award is estimated at the date of grant using the Black-Scholes option pricing model with assumptions generally consistent with those used for employee stock options, with the exception of expected term, which is over the contractual life.

### **Income taxes**

Income taxes are recorded in accordance with FASB ASC Topic 740, *Income Taxes* (“ASC 740”), which provides for deferred taxes using an asset and liability approach. The Company recognizes deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Valuation allowances are provided, if based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. As of December 31, 2011 and 2012, and March 31, 2013 (unaudited), the Company does not have any significant uncertain tax positions.

### **Net loss per share and unaudited pro forma net loss per share**

Basic net loss per share is calculated by dividing net loss attributable to common stockholders by the weighted average shares outstanding during the period, without consideration for

# bluebird bio, Inc.

## Notes to consolidated financial statements

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common stock equivalents. Diluted net loss per share is calculated by adjusting weighted average shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock method. For purposes of the diluted net loss per share calculation, preferred stock, stock options, unvested restricted stock, and warrants are considered to be common stock equivalents but have been excluded from the calculation of diluted net loss per share, as their effect would be anti-dilutive for all periods presented. Therefore, basic and diluted net loss per share were the same for all periods presented.

The calculations for the unaudited pro forma basic and diluted net loss applicable to common stockholders per share assume the conversion of all outstanding shares of preferred stock into shares of common stock as if the conversions had occurred at the beginning of the period or the date of issuance, if later (and excludes the gain on extinguishment of preferred stock and the accretion of dividends).

### ***Comprehensive loss***

Comprehensive loss is comprised of net loss and other comprehensive income or loss. Other comprehensive income or loss consists of unrealized gains and losses on marketable securities and foreign currency translation adjustments.

### ***Subsequent events***

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the financial statements to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure.

### ***Recently adopted accounting pronouncements***

In February 2013, the FASB issued guidance to provide information about the amounts reclassified out of accumulated other comprehensive income ("AOCI") by component. An entity is required to present, either on the face of the financial statements or in the notes, significant amounts reclassified out of AOCI by the respective line items of net income, but only if the amount reclassified is required to be reclassified in its entirety in the same reporting period. For amounts that are not required to be reclassified in their entirety to net income, an entity is required to cross-reference to other disclosures that provide additional details about those amounts. On January 1, 2013 the Company adopted this standard, which had no impact on its financial position or results of operations.

In June 2011, the FASB issued an amendment to the accounting guidance for presentation of comprehensive income. Under the amended guidance, a company may present the total of comprehensive income, the components of net income, and the components of other comprehensive income either in a single continuous statement of comprehensive income or in two separate but consecutive statements. In either case, a company is required to present each component of net income along with total net income, each component of other comprehensive income along with a total for other comprehensive income, and a total amount for comprehensive income. The amendment is effective for fiscal years ending, and interim periods

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(In thousands, except per share data)

within those years, beginning after December 15, 2011, and is applied retrospectively. The Company adopted this standard in the accompanying financial statements by presenting comprehensive loss in one consecutive statement along with net loss.

In May 2011, the FASB issued amended guidance on fair value measurements. This newly issued accounting standard clarifies the application of certain existing fair value measurement guidance and expands the disclosures for fair value measurements that are estimated using significant unobservable (Level 3) inputs. This accounting standard was effective on a prospective basis for annual and interim reporting periods beginning on or after December 15, 2011. The adoption of this standard has not had a material impact on the Company's financial position or results of operations.

### 3. Cash, cash equivalents, and marketable securities

The Company considers all highly liquid securities with original final maturities of three months or less from the date of purchase to be cash equivalents. As of December 31, 2011 and 2012 and March 31, 2013 (unaudited), cash and cash equivalents are comprised of funds in cash and money market accounts.

From time to time, the Company invests in marketable securities, which are classified as available-for-sale securities and are stated at fair value as determined by quoted market prices. As of December 31, 2011, the marketable securities held by the Company consisted of debt instruments with an amortized cost basis of \$3,506 and a fair value of \$3,507, resulting in cumulative unrealized gains of \$1, which were included in other comprehensive loss in the consolidated statements of operations and comprehensive loss for the year ended December 31, 2011. As of December 31, 2012 and March 31, 2013 (unaudited), the Company did not hold any marketable securities.

The following table presents the cash and cash equivalents and available-for-sale marketable securities carried at fair value in accordance with the hierarchy defined in Note 2:

Description	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
<b>December 31, 2011</b>				
Cash and cash equivalents	\$25,604	\$ 25,604	\$ —	\$ —
U.S. Government Treasury Bonds	\$ 3,507	\$ 3,507	\$ —	\$ —
	\$ 29,111	\$ 29,111	\$ —	\$ —

# bluebird bio, Inc.

## Notes to consolidated financial statements

(In thousands, except per share data)

Description	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
<b>December 31, 2012</b>				
Cash and cash equivalents	\$67,011	\$ 67,011	\$ —	\$ —
	\$67,011	\$ 67,011	\$ —	\$ —

Description	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
<b>March 31, 2013 (unaudited)</b>				
Cash and cash equivalents	\$131,836	\$ 131,836	\$ —	\$ —
	\$131,836	\$ 131,836	\$ —	\$ —

### 4. Property and equipment, net

Property and equipment, net, consists of the following:

	December 31,		March 31,
	2011	2012	2013
			(unaudited)
Computer equipment and software	\$ 173	\$ 199	\$ 235
Office equipment	149	148	160
Laboratory equipment	856	1,111	1,955
Leasehold improvements	206	357	434
Total property and equipment	1,384	1,815	2,784
Accumulated depreciation and amortization	(656)	(527)	(664)
Property and equipment, net	\$ 728	\$ 1,288	\$ 2,120

Depreciation expense was \$205, \$301, \$56 and \$139 for the years ending December 31, 2011 and 2012 and the three months ended March 31, 2012 and 2013 (unaudited), respectively.

### 5. Restricted cash

As of December 31, 2011 and 2012 and March 31, 2013 (unaudited), the Company maintains a letter of credit of \$150 that is required to be held in the form of a money market account in accordance with a building lease agreement. In addition, under the Company's corporate credit card agreement, the Company granted a security interest in a money market account of \$60, \$100 and \$100 as of December 31, 2011 and 2012 and March 31, 2013 (unaudited), respectively, to the financial institution issuing the credit cards.

# bluebird bio, Inc.

## Notes to consolidated financial statements

(In thousands, except per share data)

### 6. Accrued expenses and other current liabilities

Accrued expenses and other current liabilities consist of the following:

	<u>December 31,</u>		<u>March 31,</u>
	<u>2011</u>	<u>2012</u>	<u>2013</u>
			<b>(unaudited)</b>
Employee compensation	\$549	\$ 911	\$ 618
Accrued professional fees	104	688	930
Other	107	516	584
	<u>\$760</u>	<u>\$ 2,115</u>	<u>\$ 2,132</u>

### 7. Warrant liability

Below is a summary of the warrants outstanding:

	<u>December 31,</u>		<u>March 31,</u>
	<u>2011</u>	<u>2012</u>	<u>2013</u>
			<b>(unaudited)</b>
Warrants to purchase Series A-1 Preferred Stock	5,835	5,835	5,835
Warrants to purchase Series B Preferred Stock	575	575	575
Warrants to purchase Common Stock	1,942	1,942	1,942
	<u>8,352</u>	<u>8,352</u>	<u>8,352</u>

Below is a summary of the terms and accounting treatment for the warrants outstanding:

	<u>Shares</u>	<u>Weighted- average exercise price per share</u>	<u>Expiration</u>	<u>Balance sheet classification</u>		
				<u>December 31,</u>	<u>2012</u>	<u>March 31,</u>
				<u>2011</u>	<u>2012</u>	<u>2013</u>
						<b>(unaudited)</b>
Warrants to purchase Series A-1 Preferred Stock	5,835	\$ 0.66	November 16, 2015 - April 15, 2019	Liability	Equity	Equity
Warrants to purchase Series B Preferred Stock	575	0.33	April 15, 2019	Liability	Liability	Liability
Warrants to purchase Common Stock	1,942	0.01	March 15, 2020 - April 15, 2021	Equity	Equity	Equity
	<u>8,352</u>	<u>\$ 0.49</u>				

In connection with various financing transactions that were consummated in periods prior to December 31, 2011, the Company issued warrants for the purchase of up to 5,835 shares of the Company's Series A-1 Preferred Stock and up to 575 shares of the Company's Series B Preferred Stock to certain investors. Each warrant was immediately exercisable and generally expires

# bluebird bio, Inc.

## Notes to consolidated financial statements

(In thousands, except per share data)

approximately ten years from the original date of issuance. The warrants to purchase shares of the Company's preferred stock have an exercise price equal to the estimated fair value of the underlying instrument as of the initial date such shares were issued. Each warrant is exercisable on either a physical settlement or net share settlement basis. Upon the conversion of Series A-1 Preferred Stock and/or Series B Preferred Stock into shares of Common Stock, the associated warrants to purchase shares of the Company's preferred stock are exercisable for shares of Common Stock.

In addition, the Company issued warrants to purchase up to 962 and up to 980 shares of Common Stock in exchange for consulting services provided by non-employees during the year ended December 31, 2011, and in periods prior to the year ended December 31, 2011, respectively. The awards of warrants to purchase shares of Common Stock are accounted for as equity instruments (Note 10).

There were no exercises, cancellations, or expirations of warrants during the years ended December 31, 2011 and 2012 and during the three months ended March 31, 2013 (unaudited). All warrants were fully vested and exercisable as of December 31, 2011 and 2012 and March 31, 2013 (unaudited).

### Fair value

The fair value of the warrants on the date of issuance and on each re-measurement date for those warrants classified as liabilities is estimated using the Black-Scholes option pricing model. This method of valuation involves using inputs such as the fair value of the Company's various classes of preferred stock, stock price volatility, contractual term of the warrants, risk free interest rates, and dividend yields. Due to the nature of these inputs, the valuation of the warrants is considered a Level 3 measurement.

Liabilities measured at fair value on a recurring basis are as follows:

Description	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
<b>December 31, 2011</b>				
Warrant liability	\$637	\$ —	\$ —	\$ 637
	\$637	\$ —	\$ —	\$ 637
<b>December 31, 2012</b>				
Warrant liability	\$ 215	\$ —	\$ —	\$ 215
	\$ 215	\$ —	\$ —	\$ 215
<b>March 31, 2013 (unaudited)</b>				
Warrant liability	\$256	\$ —	\$ —	\$ 256
	\$256	\$ —	\$ —	\$ 256

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## Notes to consolidated financial statements

(In thousands, except per share data)

The following table sets forth a summary of changes in the fair value of the Company's preferred stock warrant liability which represents a recurring measurement that is classified within Level 3 of the fair value hierarchy wherein fair value is estimated using significant unobservable inputs:

	Year ended December 31,		Three months ended March 31,	
	2011	2012	2012	2013
			(unaudited)	
Beginning balance	\$ 276	\$ 637	\$ 637	\$ 215
Change in fair value	361	(28)	(59)	41
Reclassification	—	(394)	—	—
Ending balance	\$ 637	\$ 215	\$ 578	\$ 256

The fair value of each warrant to purchase shares of the Company's Series A-1 Preferred Stock was estimated using the Black-Scholes option pricing model with the following weighted-average assumptions:

	Year ended December 31,		Three months ended March 31,
	2011	2012*	2012
			(unaudited)
Fair value of underlying instrument	\$ 0.16	\$ 0.18	\$ 0.15
Expected volatility	82.7%	78.9%	79.6%
Expected term (in years)	5.70	4.98	5.29
Risk-free interest rate	1.0%	0.6%	1.1%
Expected dividend yield	0.0%	0.0%	0.0%

\* These warrants were re-measured to fair value and then reclassified to stockholders' deficit on July 23, 2012.

The fair value of each warrant to purchase shares of the Company's Series B Preferred Stock was estimated using the Black-Scholes option pricing model with the following weighted-average assumptions:

	Year ended December 31,		Three months ended March 31,	
	2011	2012	2012	2013
			(unaudited)	
Fair value of underlying instrument	\$ 0.54	\$ 0.48	\$ 0.55	\$ 0.57
Expected volatility	81.0%	80.4%	76.1%	82.4%
Expected term (in years)	7.29	6.29	7.04	6.04
Risk-free interest rate	1.6%	1.2%	1.6%	1.0%
Expected dividend yield	0.0%	0.0%	0.0%	0.0%

### Fair value

The Company estimated the fair value of its shares of Series A-1 Preferred Stock and Series B Preferred stock as of December 31, 2011, based on the option-pricing method value and the guideline public company method under the market approach value. The Company estimated the



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## Notes to consolidated financial statements

(In thousands, except per share data)

fair value of its shares of Series B Preferred Stock as of December 31, 2012 and March 31, 2013 (unaudited), and estimated the fair value of its shares of Series A-1 Preferred Stock as of July 23, 2012 and March 31, 2012 (unaudited), using a hybrid approach based on a probability-weighted average of the expected return method and the option pricing method.

### **Expected volatility**

The Company estimated the expected volatility based on actual historical volatility of the stock price of similar companies with publicly-traded equity securities. The Company calculated the historical volatility of the selected companies by using daily closing prices over a period of the expected term of the associated award. The companies were selected based on their enterprise value, risk profiles, position within the industry, and with historical share price information sufficient to meet the expected term of the associated award.

### **Expected term**

The Company based expected term on the actual remaining contractual term of each respective warrant.

### **Risk-free interest rate**

The Company estimated the risk-free interest rate in reference to yield on U.S. Treasury securities with a maturity date commensurate with the expected term of the associated award.

### **Expected dividend yield**

The Company estimated the expected dividend yield based on consideration of its historical dividend experience and future dividend expectations. The Company has not historically declared or paid dividends to stockholders. Moreover, it does not intend to pay dividends in the future, but instead expects to retain any earnings to invest in the continued growth of the business. Accordingly, the Company assumed an expected dividend yield of 0.0%.

## **8. Commitments and contingencies**

The Company leases office space under non-cancelable operating leases. Future minimum lease payments as of December 31, 2012, under the non-cancelable operating leases through the end of the lease term are as follows:

	<b>Operating leases</b>
2013	\$ 831
2014	841
2015	213
Total minimum lease payments	\$ 1,885

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## Notes to consolidated financial statements

(In thousands, except per share data)

Rent expense is calculated on a straight-line basis over the term of the lease. Rent expense recognized under all operating leases, including additional rent charges for utilities, parking, maintenance, and real estate taxes was \$638, \$815, \$178 and \$319 for the years ended December 31, 2011 and 2012 and for the three months ended March 31, 2012 and 2013 (unaudited), respectively.

The Company is also party to various agreements, principally relating to licensed technology, that require future payments relating to milestones not met at December 31, 2012 and March 31, 2013 (unaudited), or royalties on future sales of specified products. No milestone or royalty payments under these agreements are expected to be payable in the immediate future.

The Company enters into standard indemnification agreements in the ordinary course of business. Pursuant to the agreements, the Company indemnifies, holds harmless, and agrees to reimburse the indemnified party for losses suffered or incurred by the indemnified party, generally the Company's business partners or customers, in connection with any U.S. patent or any copyright or other intellectual property infringement claim by any third party with respect to the Company's products. The term of these indemnification agreements is generally perpetual any time after execution of the agreement. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is unlimited. The Company has never incurred costs to defend lawsuits or settle claims related to these indemnification agreements.

### 9. Convertible Preferred Stock

As of December 31, 2012, the authorized capital stock of the Company included 317,252 shares of preferred stock, par value \$0.01 per share, of which: (i) 18,817 shares have been designated as Series A-1 Preferred Stock, (ii) 22,304 shares have been designated as Series A-2 convertible preferred stock ("Series A-2 Preferred Stock"), (iii) 115,779 shares have been designated as Series B Preferred Stock, (iv) 39,943 shares have been designated as Series C convertible preferred stock ("Series C Preferred Stock"), and (v) 120,409 shares have been designated as Series D Preferred Stock and all collectively "Preferred Stock."

On April 15, 2011, the Company issued 53,648 shares of Series B Preferred Stock in a second closing of the financing round at a purchase price per share of \$0.3262 for aggregate proceeds totaling \$17,500. Additionally, on April 15, 2011, the Company entered into a Series C Preferred Stock Purchase Agreement (the "Series C Agreement") to issue a total of 79,885 shares of Series C Preferred Stock at a purchase price per share of \$0.37554, with substantially the same rights, preferences and privileges as the previous classes of preferred stock. On April 15, 2011, the Company issued the initial tranche of 39,943 shares of Series C Preferred Stock at a purchase price per share of \$0.37554 for aggregate proceeds totaling \$14,904, net of issuance costs of \$96. The remaining 39,942 shares of Series C Preferred Stock were to be issued at any time subsequent to the initial issuance through April 15, 2014, upon agreement among the holders of Series A-1 Preferred Stock, Series A-2 Preferred Stock, Series B Preferred Stock, and Series C Preferred Stock, as well as the Company's Board of Directors. In connection with the Series C Agreement, the redemption dates of Series A-1 Preferred Stock, Series A-2 Preferred Stock, and Series B Preferred

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## Notes to consolidated financial statements

(In thousands, except per share data)

Stock were amended to commence at any time on or after April 15, 2016; and the shares were to be redeemable in three annual installments after such date. As a result of the modification, the accretion associated with the Series A-1 Preferred Stock, Series A-2 Preferred Stock, and Series B Preferred Stock was adjusted prospectively beginning on April 15, 2011, to reflect the change in terms. The Company has evaluated the future tranche right included in the terms of the Series B Preferred Stock and Series C Preferred Stock offerings and determined that the investors' right to acquire additional shares of preferred stock is contractually embedded and not legally detachable. Such feature is not required to be bifurcated from either the Series B Preferred Stock or Series C Preferred Stock as it does not meet the definition of a derivative.

On July 23, 2012, the Company issued 120,409 shares of Series D Preferred Stock at a purchase price per share of \$0.4983 for aggregate proceeds totaling \$59,831, net of issuance costs of \$169. Subsequent to the issuance of Series D Preferred Stock, there no longer remain shares of Series C Preferred Stock available for issuance. In connection with the issuance of Series D Preferred Stock, the rights, preferences, and privileges for all classes of preferred stock then-existing were modified, as specified below.

### **General**

The rights, preferences and privileges of the preferred stock are as follows:

#### *Voting*

The holders of shares of preferred stock are entitled to the number of votes equal to the number of whole shares of common stock into which the shares of the applicable series of preferred stock held by such holder are convertible or any matter presented to the stockholders of the Company for their action or consideration at any meeting of stockholders of the Company or by written consent of stockholders in lieu of meetings. Except as provided by law or otherwise, the holders of shares of preferred stock vote together with the holders of shares of Common Stock as a single class.

#### *Dividends*

The holders of shares of preferred stock are entitled to receive dividends, if and when declared by the board of directors on a pari passu basis. Dividends payable on each share of preferred stock is determined as if such share had been converted into shares of Common Stock. As of December 31, 2012, no dividends have been declared or paid since the Company's inception.

All accrued, but unpaid dividends on the Company's outstanding shares of Series A-1 Preferred Stock, Series A-2 Preferred Stock, Series B Preferred Stock, and Series C Preferred Stock were forfeited as a condition to the issuance of the Series D Preferred Stock. The forfeiture of the dividends that had accumulated as of July 23, 2012, for each respective series of preferred stock was considered in combination with the other modifications to the preferred stock that occurred upon the consummation of the sale of Series D Preferred Stock, as discussed below.

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(In thousands, except per share data)

### *Liquidation*

In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company or upon the occurrence of a Deemed Liquidation Event, as defined, the holders of shares of Series D Preferred Stock then outstanding are entitled to be paid out of the assets of the Company available for distribution to stockholders an amount per share equal to \$0.4983, subject to appropriate adjustment, plus any dividends declared but unpaid thereon, before any payment is made to holders of shares of Series A-1 Preferred Stock, holders of shares of Series A-2 Preferred Stock, holders of shares of Series B Preferred Stock, holders of shares of Series C Preferred Stock, or holders of shares of Common Stock. The holders of shares of Series C Preferred Stock then outstanding and the holders of shares of Series B Preferred Stock then outstanding are entitled to be paid out of the assets of the Company available for distribution to stockholders, on a pari passu basis, an amount per share equal to \$0.37554, subject to appropriate adjustment, and \$0.4893, subject to appropriate adjustment, respectively, plus any dividends declared but unpaid thereon, before any payment is made to holders of shares of Series A-1 Preferred Stock, holders of shares of Series A-2 Preferred Stock, or holders of shares of Common Stock. The holders of shares of Series A-2 Preferred Stock then outstanding are entitled to be paid out of the assets of the Company available for distribution to stockholders an amount per share equal to \$0.5758, subject to appropriate adjustment, plus any dividends declared but unpaid thereon, before any payment is made to holders of shares of Series A-1 Preferred Stock or holders of shares of Common Stock.

After the payment of all preferential amounts required to be paid to the holders of shares of Series A-2 Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and Series D Preferred Stock, the remaining assets of the Company available for distribution to stockholders will be distributed among the holders of shares of preferred stock and Common Stock, pro rata based on the number of shares held by each such holder, treating such securities as if they had been converted to common stock immediately prior to such dissolution, liquidation, or winding up of the Company. In the event the assets of the Company available for distribution to stockholders are insufficient to permit payment of the full amount to which each shareholder is entitled, holders of shares of capital stock will share ratably in any distribution of the remaining assets of the Company in proportion to the respective amounts which would otherwise be payable under the circumstances in the order of liquidation preference.

### *Conversion*

Each share of Series A-1 Preferred Stock, Series A-2 Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and Series D Preferred Stock is convertible at the option of the holder, at any time and from time to time, into fully paid and non-assessable shares of common stock. Each share of Series A-1 Preferred Stock, Series A-2 Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and Series D Preferred Stock is convertible into that number of common shares as is determined by dividing the original purchase price of such share by the applicable conversion price (\$0.6619, \$0.6619, \$0.3262, \$0.37554, and \$0.4983 for the Series A-1 Preferred Stock, Series A-2 Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and Series D

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Preferred Stock, respectively). As of December 31, 2012, the conversion rate is 1:1, but is subject to adjustment in the future upon the occurrence of certain events.

Each share of Series A-1 Preferred Stock, Series A-2 Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and Series D Preferred Stock is automatically convertible into fully paid and non-assessable shares of common stock upon either: (i) the closing of the sale of shares of the Company's common stock to the public in an underwritten public offering resulting in at least \$30,000 of gross proceeds to the Company and a listing of the Company's common stock on a nationally recognized securities exchange or trading system or (ii) the date and time, or the occurrence of an event, specified by vote or written consent of the holders of shares constituting a majority of the then outstanding shares of preferred stock and the holders of shares constituting a majority of the then outstanding shares of Series D Preferred Stock.

The Company evaluated each series of its preferred stock and determined that each individual series is considered an equity host under FASB ASC Topic 815, *Derivatives and Hedging*. In making this determination, the Company's analysis followed the whole instrument approach which compares an individual feature against the entire preferred stock instrument which includes that feature. The Company's analysis was based on a consideration of the economic characteristics and risks of each series of preferred stock. More specifically, the Company evaluated all of the stated and implied substantive terms and features, including: (i) whether the preferred stock included redemption features, (ii) how and when any redemption features could be exercised, (iii) whether the holders of preferred stock were entitled to dividends, (iv) the voting rights of the preferred stock and (v) the existence and nature of any conversion rights. As a result of the Company's conclusion that the preferred stock represents an equity host, the conversion feature of all series of preferred stock is considered to be clearly and closely related to the associated preferred stock host instrument. Accordingly, the conversion feature of all series of preferred stock is not considered an embedded derivative that requires bifurcation.

The Company accounts for potentially beneficial conversion features under FASB ASC Topic 470-20, *Debt with Conversion and Other Options*. At the time of each of the issuances of convertible preferred stock, the Company's common stock into which each series of the Company's preferred stock is convertible had an estimated fair value less than the effective conversion prices of the convertible preferred stock. Therefore, there was no intrinsic value on the respective commitment dates.

### *Redemption*

Prior to July 23, 2012, the Series A-1 Preferred Stock, Series A-2 Preferred Stock, Series B Preferred Stock and Series C Preferred Stock included rights of redemption. Accordingly, each series of preferred stock was being accreted to redemption value through the respective redemption dates, including appropriate accruals for the then-existing cumulative dividend rights. Upon the issuance of Series D Preferred Stock on July 23, 2012, the redemption rights of the Series A-1 Preferred Stock, Series A-2 Preferred Stock, Series B Preferred Stock, and Series C Preferred Stock were removed in their entirety. Accordingly, upon the issuance of Series D Preferred Stock, accretion ceased for the Series A-1 Preferred Stock, Series A-2 Preferred Stock,

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(In thousands, except per share data)

Series B Preferred Stock, and Series C Preferred Stock and cumulative dividends that had been accrued through July 23, 2012, were considered in the calculation of the gain on extinguishment, as discussed below. The shares of Series D Preferred Stock do not include redemption rights.

In accordance with the guidance in FASB ASC Topic 480, *Distinguishing Liabilities from Equity*, shares of Series A-1 Preferred Stock, Series A-2 Preferred Stock, Series B Preferred Stock, and Series C Preferred Stock were classified outside of permanent equity through July 23, 2012. As the result of the elimination of the redemption rights for all classes of preferred stock that occurred in connection with the issuance of Series D Preferred Stock, the outstanding shares of Series A-1 Preferred Stock were reclassified from temporary equity to permanent equity during the year ended December 31, 2012, because these shares have no liquidation preference and therefore no possibility of being redeemed. All other classes of preferred stock remain classified within temporary equity as of December 31, 2012 and March 31, 2013 (unaudited), due to their associated liquidation preferences.

### ***Extinguishment of preferred stock***

In connection with the issuance of the Series D Preferred Stock, the rights, preferences, and privileges for all classes of preferred stock then outstanding were modified. More specifically, the redemption privileges were eliminated in their entirety for Series A-1 Preferred Stock, Series A-2 Preferred Stock, Series B Preferred Stock, and Series C Preferred Stock. Additionally, the dividend rights changed from cumulative dividend rights to non-cumulative dividend rights and all accrued, but unpaid dividends on the Company's Series A-1 Preferred Stock, Series A-2 Preferred Stock, Series B Preferred Stock, and Series C Preferred Stock as of July 23, 2012, were forfeited. Lastly, the liquidation preference for the Series B Preferred Stock was reduced from \$0.6524 per share to \$0.4893 per share.

The Company has accounted for the amendment to the rights, preferences, and privileges of the preferred stock as an extinguishment of the old preferred stock and issuance of new preferred stock due to the significance of the modifications to the substantive contractual terms of the preferred stock and the associated fundamental changes to the nature of the preferred stock. Accordingly, the Company recorded an aggregate gain of \$23,114 within stockholders' deficit equal to the difference between the fair value of the new shares of preferred stock issued and the carrying amount of the old shares of preferred stock extinguished. The Company allocated \$9,356 of the gain to additional paid-in capital to recover the amount of additional paid-in capital that had previously been reduced by accreted dividends that were forfeited as part of the extinguishment, while the remaining \$13,758 was recorded to accumulated deficit. The gain on extinguishment is reflected in the calculation of net loss available to common stockholders in accordance with FASB ASC Topic 260, *Earnings per Share*. The fair value of the Series A-1 Preferred Stock, Series A-2 Preferred Stock, Series B Preferred Stock, and Series C Preferred Stock was determined using a hybrid approach based on a probability-weighted average of the expected return method and the option pricing method.

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(In thousands, except per share data)

### 10. Common stock

As of December 31, 2012, the authorized capital stock of the Company included 400,000 shares of Common Stock, par value \$0.01 per share. On January 16, 2013, the Company increased the authorized capital stock of the Company to 408,000 shares.

#### *General*

The voting, dividend and liquidation rights of the holders of shares of Common Stock are subject to and qualified by the rights, powers and preferences of the holders of shares of preferred stock. The Common Stock has the following characteristics:

#### *Voting*

The holders of shares of Common Stock are entitled to one vote for each share of Common Stock held at all meetings of stockholders and written actions in lieu of meetings.

#### *Dividends*

The holders of shares of Common Stock are entitled to receive dividends, if and when declared by the board of directors. Cash dividends may not be declared or paid to holders of shares of common stock until paid on each series of outstanding preferred stock in accordance with their respective terms. As of December 31, 2012, no dividends have been declared or paid since the Company's inception.

#### *Liquidation*

After payment to the holders of shares of preferred stock of their liquidation preferences, the holders of shares of Common Stock are entitled to share ratably in the Company's assets available for distribution to stockholders, in the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company or upon the occurrence of a deemed liquidation event.

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### Reserved for future issuance

The Company has reserved for future issuance the following number of shares of Common Stock:

	December 31,		March 31,
	2011	2012	2013
			(unaudited)
Conversion of Series A-1 Preferred Stock	12,981	12,981	12,981
Conversion of Series A-2 Preferred Stock	22,304	22,304	22,304
Conversion of Series B Preferred Stock	115,204	115,204	115,204
Conversion of Series C Preferred Stock	79,885	39,943	39,943
Conversion of Series D Preferred Stock	—	120,409	120,409
Vesting of Restricted Stock	4,715	2,931	2,506
Options to purchase Common Stock	48,720	71,089	79,639
Warrants to purchase Series A-1 Preferred Stock	5,835	5,835	5,835
Warrants to purchase Series B Preferred Stock	575	575	575
Warrants to purchase Common Stock	1,942	1,942	1,942
	<b>292,161</b>	<b>393,213</b>	<b>401,338</b>

## 11. Significant agreements

### Celgene Corporation

#### Summary of the Collaboration Agreement

On March 19, 2013, the Company entered into a Master Collaboration Agreement (the "Collaboration Agreement") with Celgene to discover, develop and commercialize disease-altering gene therapies in oncology. The collaboration is focused on applying gene therapy technology to genetically modify a patient's own T cells, known as chimeric antigen receptor, or CAR, T cells, to target and destroy cancer cells. Additionally, on March 19, 2013, the Company entered into a Platform Technology Sublicense Agreement (the "Sublicense Agreement") with Celgene pursuant to which the Company sublicensed certain intellectual property, originating under Celgene's license from Baylor College of Medicine, for use in the collaboration.

Under the terms of the Collaboration Agreement, the Company received a \$75,000 up-front, non-refundable cash payment. The Company will be responsible for conducting discovery, research and development activities through completion of Phase I clinical studies, if any, during the initial term of the agreement, or three years. The collaboration will be governed by a joint steering committee ("JSC") formed by an equal number of representatives from the Company and Celgene. The JSC will, among other activities, review the collaboration program, review and evaluate product candidates and approve regulatory plans. In addition to the JSC, the Collaboration Agreement provides that the Company and Celgene will each appoint representatives to establish a patent committee, which will be responsible for managing the intellectual property developed and used during the collaboration.



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## Notes to consolidated financial statements

(In thousands, except per share data)

Prior to expiration of the initial term of the Collaboration Agreement, Celgene has two options to extend the term, through March 19, 2019, with the payment of significant extension fees. Separately, Celgene has an option to license an unlimited number of product candidates resulting from the collaboration during a period commencing upon execution of the Collaboration Agreement and continuing through a specified period following the completion of Phase I clinical study for each individual product candidate. In the event such option is exercised, the Company would grant Celgene an exclusive worldwide license to develop and commercialize such product candidate. Upon exercise of the option to license a product candidate, Celgene is required to pay an option fee, which is subject to reduction if the Company elects to co-develop and co-promote such product candidate in the United States. For any product candidates licensed by Celgene, the Company may be responsible, at Celgene's election, to continue performing certain development activities contemplated as part of the collaboration plan. If Celgene does not exercise its option with respect to a product candidate prior to the expiration of the applicable option period, then the Company has the right to develop the product candidate outside the scope of the collaboration, subject to a Celgene opt-in right to obtain a license to that product candidate for additional cash consideration. The opt-in right exists through completion of a pivotal study for the specific product candidate.

In addition, Celgene would be required to make certain milestone payments upon the achievement of specified clinical, regulatory and commercial events. For each product candidate that is licensed by Celgene, the Company would be eligible to receive per product up to \$30,000 in aggregate potential option fees and clinical milestone payments, up to \$117,000 in regulatory milestone payments and up to \$78,000 in commercial milestone payments. In addition, to the extent any of the product candidates licensed by Celgene are commercialized, the Company would be entitled to receive tiered royalty payments ranging from the mid-single digits to mid-teens based on a percentage of net sales. Royalty payments are subject to certain reductions, including for any royalty payments required to be made by Celgene to acquire patent rights, with an aggregate minimum floor.

Additionally, the Company may elect to co-develop and co-promote product candidates licensed by Celgene. If the Company elects to co-develop and co-promote a product candidate, then the parties would share equally in all costs incurred relating to the development, commercialization and manufacture of the product candidate within the United States and share equally in the profits generated by such product candidate in the United States. Additionally, if the Company elects to co-develop and co-promote a product candidate, then the option fees, milestones and royalties would change compared to those described above. Under this scenario, the Company would receive per product up to \$20,000 in aggregate potential option fees and clinical milestone payments and outside the United States, up to \$54,000 in regulatory milestone payments and up to \$36,000 in commercial milestone payments. In addition, to the extent any of the product candidates licensed by Celgene and co-developed and co-promoted by the Company are commercialized, the Company would be entitled to receive tiered royalty payments ranging from the mid-single digits to mid-teens based on a percentage of net sales from sales generated outside of the United States. Royalty payments are subject to certain reductions, including for any royalty payments required to be made by Celgene to acquire patent rights, with an aggregate minimum floor.

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In the event Celgene elects to license a product candidate discovered and developed as part of the Collaboration Agreement, Celgene would be solely responsible for all costs and expenses of manufacturing and supplying any product candidates. Subject to customary back-up supply rights granted to Celgene, the Company has the sole right to manufacture or have manufactured supplies of vectors and associated payloads manufactured for incorporation into the associated product candidate. Celgene would reimburse the Company for the costs incurred to manufacture and supply such vectors and associated payloads, plus a modest mark-up.

The Collaboration Agreement may be terminated by either the Company or Celgene, upon written notice, in the event of the other party's uncured material breach. Celgene may terminate the Collaboration Agreement for any reason upon written notice to the Company. If the Collaboration Agreement is terminated, rights to product candidates in development at the time of such termination will be allocated to the parties through a mechanism included in the Collaboration Agreement. In addition, if Celgene terminated the Collaboration Agreement as a result of a breach by the Company, then any then-existing co-development and co-promotion agreement will be automatically terminated and replaced with a license agreement for such product candidate and any amounts payable by Celgene under any then-existing product license agreements will be reduced.

### *Call Option*

Effective upon completion of the Company's initial public offering, during the initial three-year term of the collaboration and, if extended, during the first two-year extension term of the collaboration, in the event that the Company engages in a change in control transaction, including for such purposes a merger or consolidation of the Company or the sale of all or substantially all of the Company's assets, or if another person or entity or group of persons or entities acquires at least 50% of the Company's voting capital stock, then Celgene has the right, but not the obligation, to terminate the Collaboration Agreement and obtain perpetual, non-terminable, worldwide, exclusive, fully paid-up licenses to all, but not less than all, of the product candidates previously identified under the Collaboration Agreement (the "Call Option"). Under the Call Option, the product candidates to which Celgene would have the right to acquire licenses include any product candidate previously licensed out of the collaboration during the term of the collaboration, any product candidate for which the Company has exercised the right to co-develop and co-promote within the United States, any product candidate for which Celgene previously declined its option to obtain a license and any product candidate for which at least in vivo efficacy studies have been initiated or authorized by the JSC. The purchase price for such licenses would be based on the fair value of these rights received and obligations assumed determined pursuant to a binding arbitration process.

In addition, during the initial three-year term of the collaboration, but not during any extension term, in the event that Celgene exercises the Call Option, in addition to the right to acquire the fully paid-up licenses describe above, Celgene would obtain a perpetual, non-terminable, worldwide, exclusive license to the Company's intellectual property to develop one or more CAR T cell products targeting one or more oncology associated target antigens for the remainder of the initial collaboration term. Following the initial collaboration term, the license

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to the Company's intellectual property is limited to target antigens identified by Celgene promptly following the initial collaboration term for which Celgene reasonably intends to develop CAR T cell products. There is no limit to the number of oncology-related target antigens Celgene may select under this license. Upon commercialization of any such product candidate so licensed by Celgene, Celgene would be obligated to pay the Company a specified milestone payment upon regulatory approval and a percentage of net sales as a royalty.

The Company has concluded that the value of the Call Option is immaterial based primarily on the probability that the Call Option would become exercisable.

### **Accounting Analysis**

The Company's arrangement with Celgene contains the following deliverables: (i) discovery, research and development services, (ii) participation on the JSC and (iii) participation on the patent committee. The Company has determined that the options to extend the term of the agreement and the options to license product candidates are substantive options. Celgene is not contractually obligated to exercise the options. Additionally, as a result of the uncertain outcome of the discovery, research and development activities, the Company is at risk with regard to whether Celgene will exercise the options. Moreover, the Company has determined that the options are not priced at a significant and incremental discount. Accordingly, the options are not considered deliverables at the inception of the arrangement and the associated option fees are not included in allocable arrangement consideration.

The Company has concluded that each of the three deliverables identified at the inception of the arrangement (discovery, research and development services; participation on the JSC and participation on the patent committee) has standalone value from the other undelivered elements. Additionally, the Collaboration Agreement does not include return rights related to the initial collaboration term. Accordingly, each deliverable qualifies as a separate unit of accounting.

The Company has identified the allocable arrangement consideration as the \$75,000 up-front payment. The Company determined that each of the identified deliverables have the same period of performance (the three year initial term) and have the same pattern of revenue recognition, ratably over the period of performance. As a result, the \$75,000 arrangement consideration will be recognized over the three year initial term.

The Company has evaluated all of the milestones that may be received in connection with Celgene's option to license a product candidate resulting from the collaboration. In evaluating if a milestone is substantive, the Company assesses whether: (i) the consideration is commensurate with either the Company's performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the Company's performance to achieve the milestone, (ii) the consideration relates solely to past performance and (iii) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. All clinical and regulatory milestones are considered substantive on the basis of the contingent nature of the milestone, specifically reviewing factors such as the scientific, clinical, regulatory, commercial and other risks that must be overcome to achieve the milestone as well as the level of effort and investment required. Accordingly, such amounts will

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be recognized as revenue in full in the period in which the associated milestone is achieved, assuming all other revenue recognition criteria are met. All commercial milestones will be accounted for in the same manner as royalties and recorded as revenue upon achievement of the milestone, assuming all other revenue recognition criteria are met.

During the three months ended March 31, 2013, the Company recognized \$1,042 of revenue associated with its collaboration with Celgene related to the recognition of discovery, research and development services. As of March 31, 2013, there is \$73,958 of deferred revenue related to the Company's collaboration with Celgene which is classified as current or long-term in the accompanying balance sheet based on the contractual term of the arrangement.

### ***Association Francaise contre les Myopathies***

In January 2011, the Company entered into a research funding agreement with the Association Francaise contre les Myopathies ("AFM"), a nonprofit organization dedicated to curing rare neuromuscular diseases and providing treatments to reduce the associated disabilities of such diseases. As part of the agreement, AFM funded the Company 1 million Euros to be used to advance the Company's research, process development, manufacturing, preclinical development, and clinical development in gene therapy for beta-hemoglobinopathies in  $\beta$ -thalassemia and/or in Sickle Cell Disease.

The funding, or a portion thereof depending on timing, shall be repaid to AFM upon any of the following events: (i) upon out-licensing or sale of the program, (ii) upon obtaining the first product authorization for the market, or (iii) upon sale of the Company, provided that the development is active at the time of such sale. The agreement is for a period of four years. The Company believes that repayment of the funds paid under the agreement is not probable at the date of the agreement or at December 31, 2011 and 2012. The Company recognizes the revenue under this arrangement on a straight-line basis over the term of the agreement. The Company will reassess the probability of repayment at the end of each reporting period.

### ***CIRM***

In October 2012, the California Institute for Regenerative Medicine ("CIRM") approved a \$9.3 million award to the Company. The Company is in the process of negotiating the terms of the award with CIRM. The award is to support a Phase I/II study to evaluate the safety and efficacy of LentiGlobin, the Company's development-stage program for the treatment of  $\beta$ -thalassemia, which is expected to be initiated in the United States in 2013. The grant will be issued in quarterly installments and is expected to be utilized over a four-year period starting in the second quarter of 2013. As of December 31, 2012, the Company had not received or recognized any amounts under this award.

### ***Massachusetts life science center***

In October 2011, the Company was awarded a \$242 tax incentive from the Massachusetts Life Sciences Center as part of the Life Sciences Tax Incentive Program. The program was established

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in 2008 to incentivize life science companies to create new sustained jobs in Massachusetts. If the Company does not meet and maintain its job creation commitment for at least five years, the total amount awarded may be recovered by the Massachusetts Department of Revenue. The Company recognized this award as grant revenue in 2011, as the Company had satisfied its job creation commitments and the Company's long-range hiring plan was significantly in excess of the requirement. The Company concluded that the likelihood of refund was remote.

### 12. Stock-based compensation

In December 1996, the board of directors adopted the 1996 Stock Option Plan. In April 2002, the Board of Directors canceled the 1996 Stock Option Plan and adopted the 2002 Employee, Director and Consultant Plan. In September 2010, the board of directors adopted the 2010 Stock Option and Grant Plan (the "Plan"). With the adoption of the Plan, the 2002 Employee, Director and Consultant Plan was terminated; and no further options or awards are permitted to be granted under this plan. Any options or awards outstanding under the 2002 Employee, Director and Consultant Plan at the time of adoption of the Plan remained outstanding and effective. The Plan allows for the granting of incentive stock options, non-qualified stock options, and restricted stock awards to the Company's employees, members of the board of directors, and consultants of the Company.

Originally, upon the adoption of the Plan, the number of shares of Common Stock authorized pursuant to stock-based compensation plans was 33,520. On April 15, 2011, the Plan was amended to increase the number shares of Common Stock authorized to 48,720; on April 24, 2012, the Plan was amended to increase the number of shares of Common Stock authorized to 53,220; on July 23, 2012, the Plan was amended to increase the number of shares of Common Stock authorized under the Plan to 71,089; and on January 16, 2013, the Plan was amended to increase the number of common shares that may be issued under the Plan to 87,719. Approximately 10,354 remain available for grant as of March 31, 2013 (unaudited). This number can be increased by the board of directors, subject to the approval of the shareholders.

The Plan provides for the issuance of stock options, restricted stock awards, unrestricted stock awards, and restricted stock units to employees, officers, directors, consultants, and key personnel of the Company. The Company has not granted unrestricted stock awards or restricted stock unit awards under the Plan since its inception. Options generally expire ten years following the date of grant, unless the award recipient is an owner of more than ten percent of the combined voting power of all classes of stock of the Company, in which case the contractual term cannot exceed five years following the date of grant. Options typically vest in four years, but vesting provisions can vary based on the discretion of the administrator to the Plan. Options carry an exercise price equal to the estimated fair value of the Company's common stock on the date of grant, unless the award recipient is an owner of more than ten percent of the combined voting power of all classes of stock of the Company, in which case the exercise price cannot be less than 110 percent of the estimated fair value of the Company's common stock on the date of grant. Generally options to purchase shares of the Company's common stock are exercisable on a physical settlement basis, but net share settlement is permitted in certain instances. Restricted stock awards have varying vesting terms. Recipients of restricted stock awards are entitled to

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voting rights and to receive dividends, if and when declared. Awards of restricted stock generally carry a purchase price equal to the estimated fair value of the Company's common stock on the date of grant. Upon termination, the unvested portion of an award of restricted stock is subject to a right of repurchase by the Company in an amount equal to the original purchase price.

Shares of the Company's common stock underlying any awards that are forfeited, canceled, withheld upon exercise of an option, or settlement of an award to cover the exercise price or tax withholding, reacquired by the Company prior to vesting, satisfied without the issuance of shares of the Company's common stock, or otherwise terminated other than by exercise will be added back to the shares of common stock available for issuance under the Plan. Shares available for issuance under the Plan may be authorized but unissued shares of the Company's common stock or shares of the Company's common stock that have been reacquired by the Company. The Plan will expire on September 15, 2020, the tenth anniversary of its approval by the Company's board of directors.

### Stock-based compensation expense

The Company recognized stock-based compensation expense totaling \$758, \$822, \$233 and \$661 during the years ended December 31, 2011 and 2012, and the three months ended March 31, 2012 and 2013 (unaudited), respectively. Share-based compensation expense recognized by award type is as follows:

	Year ended December 31,		Three months ended March 31,	
	2011	2012	2012	2013
			(unaudited)	
Stock options	\$ 574	\$ 742	\$ 215	\$ 639
Warrants	102	—	—	—
Restricted stock awards	82	80	18	22
	<u>\$ 758</u>	<u>\$ 822</u>	<u>\$ 233</u>	<u>\$ 661</u>

Total compensation cost recognized for all stock-based compensation awards in the consolidated statements of operations and comprehensive loss is as follows:

	Year ended December 31,		Three months ended March 31,	
	2011	2012	2012	2013
			(unaudited)	
Research and development	\$ 325	\$ 408	\$ 118	\$ 347
General and administrative	433	414	115	314
	<u>\$ 758</u>	<u>\$ 822</u>	<u>\$ 233</u>	<u>\$ 661</u>

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The fair value of each option issued to employees was estimated at the date of grant using the Black-Scholes option pricing model with the following weighted-average assumptions:

	Year ended December 31,		Three months ended March 31,	
	2011	2012	2012	2013
			(unaudited)	
Fair value of the underlying instrument	\$ 0.11	\$ 0.12	\$ 0.11	\$ 0.29
Expected volatility	83.0%	79.6%	78.8%	82.0%
Expected term (in years)	6.1	6.1	6.1	6.1
Risk-free interest rate	1.7%	1.0%	1.1%	1.0%
Expected dividend yield	0.0%	0.0%	0.0%	0.0%

The intrinsic value of options exercised during the years ended December 31, 2011 and 2012, and the three months ended March 31, 2012 and 2013 (unaudited), was \$1, \$17, \$0 and \$31, respectively.

The aggregate fair value of restricted stock awards that vested during the years ended December 31, 2011 and 2012, and the three months ended March 31, 2012 and 2013 (unaudited), based on the estimated fair value of the underlying stock on the day of vesting was \$116, \$97, \$28 and \$23, respectively.

As of March 31, 2013, there was \$6,467 of unrecognized compensation expense related to unvested stock options and restricted stock awards that is expected to be recognized over a weighted-average period of 3.6 years.

### Restricted common stock

A summary of the Company's restricted stock activity and related information is as follows:

	Shares	Weighted-average grant date fair value
Unvested balance at December 31, 2011	4,715	\$ 0.05
Granted	—	—
Vested	(1,784)	\$ 0.05
Forfeited	—	—
Unvested balance at December 31 2012	2,931	\$ 0.05
Granted (unaudited)	—	—
Vested (unaudited)	(425)	\$ 0.05
Forfeited (unaudited)	—	—
Unvested balance at March 31, 2013 (unaudited)	2,506	\$ 0.05

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## Notes to consolidated financial statements

(In thousands, except per share data)

### Stock options

The following table summarizes the stock option activity under the Plan:

	Shares	Weighted-average exercise price per share	Weighted-average contractual life (in years)	Aggregate intrinsic value (a)
Outstanding at December 31, 2011	29,000	\$ 0.11	9.0	\$ 313
Granted	14,899			
Exercised	(185)			
Canceled or forfeited	(1,963)			
Outstanding at December 31 2012	41,751	\$ 0.11	8.5	\$ 7,502
Granted (unaudited)	27,844			
Exercised (unaudited)	(310)			
Canceled or forfeited (unaudited)	—			
Outstanding at March 31, 2013 (unaudited)	69,285	\$ 0.18	8.9	\$ 17,146
Exercisable at December 31, 2012	17,538	\$ 0.11	7.8	\$ 3,128
Vested and expected to vest at December 31, 2012	41,751	\$ 0.11	8.5	\$ 7,502
Exercisable at March 31, 2013 (unaudited)	20,053	\$ 0.11	7.7	\$ 6,405
Vested and expected to vest at March 31, 2013 (unaudited)	69,285	\$ 0.18	8.9	\$ 17,146

(a) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the estimated fair value of the Common Stock for the options that were in the money at December 31, 2011 and 2012 and March 31, 2013 (unaudited).

During the years ended December 31, 2011 and 2012, and the three months ended March 31, 2012 and 2013 (unaudited), current and former employees of the Company exercised a total of 32, 185, 0 and 310 options, respectively, resulting in total proceeds of \$1, \$21, \$0 and \$35, respectively. In accordance with Company policy, the shares were issued from a pool of shares reserved for issuance under the stock plans described above.

### Warrants

During the year ended December 31, 2011, the Company issued warrants to purchase an aggregate of 962 shares of Common Stock. The awards were granted in exchange for consulting services provided by a non-employee pursuant to standalone award agreements that are independent of an equity incentive plan. The warrants vested immediately and are outstanding for ten years from the date of issuance. The Company determined the fair value of the warrants using the Black-Scholes option pricing model. The aggregate fair value of the warrants was recognized in full on the date of grant. The Company recognized \$102 of share-based compensation expense associated with the warrants issued during the year ended December 31, 2011.



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(In thousands, except per share data)

### Note receivable

In November 2010, the Company received a non-recourse note from its Chief Executive Officer (“CEO”) in exchange for the purchase of 6,245 shares of restricted stock. Interest accrues on the note on an annual basis at a rate of four percent. The note is payable in cash and due on November 15, 2020, and allowed to be prepaid. As of March 31, 2013, the outstanding principal balance of the note was \$312. This note is collateralized by the underlying restricted common stock and has been accounted for similar to a stock option within the accompanying consolidated financial statements. Accordingly, neither the note nor the issuance of the shares has been recorded. The CEO expects to repay the note prior to the conclusion of the proposed initial public offering. The Company recorded stock-based compensation expense of \$63 in connection with this restricted stock for each of the years ended December 31, 2011 and 2012 and \$16 for each of the three months ended March 31, 2012 and 2013 (unaudited).

### 13. 401(k) Savings plan

In 1997, the Company established a defined-contribution savings plan under Section 401(k) of the Internal Revenue Code (“the 401(k) Plan”). The 401(k) Plan covers all employees who meet defined minimum age and service requirements, and allows participants to defer a portion of their annual compensation on a pretax basis. The Company has not made any contributions to the 401(k) Plan for the two years ended December 31, 2012 and three months ended March 31, 2013 (unaudited).

### 14. Income taxes

For the years ended December 31, 2011 and 2012, the Company did not record a current or deferred income tax expense or benefit.

The components of loss before income taxes were as follows:

	Year ended December 31,	
	2011	2012
U.S.	\$ (15,300)	\$ (23,700)
Foreign	(298)	30
Total	\$ (15,598)	\$ (23,670)

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Deferred taxes are recognized for temporary differences between the basis of assets and liabilities for financial statement and income tax purposes. The significant components of the Company's deferred tax assets are comprised of the following:

	Year ended December 31,	
	2011	2012
Deferred tax assets:		
U.S. net operating loss carryforwards	\$ 16,082	\$ 24,044
Foreign net operating loss carryforwards	584	587
Tax credit carryforwards	1,283	1,910
Capitalized research and development expenses, net	2,898	2,334
Accruals and other	867	556
Total deferred tax assets	21,714	29,431
Less valuation allowance	(21,714)	(29,431)
Net deferred tax assets	\$ —	\$ —

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based on the Company's history of operating losses, the Company has concluded that it is more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company has provided a full valuation allowance for deferred tax assets as of December 31, 2011 and 2012. The valuation allowance increased approximately \$7,717 during the year ended December 31, 2012, due primarily to net operating losses. The valuation allowance increased approximately \$6,240 during the year ended December 31, 2011, due primarily to net operating losses generated during the period and research credits.

A reconciliation of income tax expense computed at the statutory federal income tax rate to income taxes as reflected in the financial statements is as follows:

	Year ended December 31,	
	2011	2012
Federal income tax expense at statutory rate	34.0%	34.0%
State income tax, net of federal benefit	5.0%	4.4%
Permanent differences	(1.3%)	(0.8%)
Research and development credit	3.4%	0.8%
Other	(0.6%)	0.6%
Change in valuation allowance	(40.5%)	(39.0%)
Effective income tax rate	0.0%	0.0%

As of December 31, 2011 and 2012, the Company had U.S. federal net operating loss carryforwards of approximately \$42,400 and \$62,600, respectively, which may be available to offset future income tax liabilities and expire at various dates through 2032. As of December 31, 2011 and 2012, the Company also had U.S. state net operating loss carryforwards of approximately \$31,300 and \$52,300, respectively, which may be available to offset future income tax liabilities and expire at various dates through 2032. At December 31, 2011 and 2012, the Company also had approximately \$1,800 and \$1,800, respectively, of foreign net operating loss

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carryforwards which may be available to offset future income tax liabilities; these carryforwards do not expire. As a result of the up-front payment pursuant to the Company's collaboration agreement with Celgene, the Company expects that it will use a significant portion of its net operating loss carryforwards.

As of December 31, 2011 and 2012, the Company had federal research and development tax credit carryforwards of approximately \$1,000 and \$1,300, respectively, available to reduce future tax liabilities which expire at various dates through 2032. As of December 31, 2011 and 2012, the Company had state research and development tax credit carryforwards of approximately \$400 and \$900, respectively, available to reduce future tax liabilities which expire at various dates through 2027.

Under the provisions of the Internal Revenue Code, the net operating loss and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. Net operating loss and tax credit carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50 percent, as defined under Sections 382 and 383 of the Internal Revenue Code, respectively, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of the Company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. The Company has completed several financings since its inception which may have resulted in a change in control as defined by Sections 382 and 383 of the Internal Revenue Code, or could result in a change in control in the future.

The Company will recognize interest and penalties related to uncertain tax positions in income tax expense. As of December 31, 2011 and 2012, the Company had no accrued interest or penalties related to uncertain tax positions and no amounts have been recognized in the Company's consolidated statements of operations and comprehensive loss.

For all years through December 31, 2012, the Company generated research credits but has not conducted a study to document the qualified activities. This study may result in an adjustment to the Company's research and development credit carryforwards; however, until a study is completed and any adjustment is known, no amounts are being presented as an uncertain tax position for these two years. A full valuation allowance has been provided against the Company's research and development credits and, if an adjustment is required, this adjustment would be offset by an adjustment to the deferred tax asset established for the research and development credit carryforwards and the valuation allowance.

The Company or one of its subsidiaries files income tax returns in the United States, and various state and foreign jurisdictions. The federal, state and foreign income tax returns are generally subject to tax examinations for the tax years ended December 31, 2009 through December 31, 2012. To the extent the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service, state or foreign tax authorities to the extent utilized in a future period.

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(In thousands, except per share data)

### 15. Net loss per share

The following table reconciles net loss to net loss applicable to common stockholders:

	Year ended December 31,		Three months ended March 31,	
	2011	2012	2012	2013
			(unaudited)	
Numerator:				
Net loss	\$ (15,598)	\$ (23,670)	\$ (5,068)	\$ (6,544)
Accretion and dividends on convertible preferred stock	(4,993)	(3,057)	(1,285)	—
Gain on extinguishment of convertible preferred stock	—	23,114	—	—
Net loss applicable to common stockholders	<u>\$ (20,591)</u>	<u>\$ (3,613)</u>	<u>\$ (6,353)</u>	<u>\$ (6,544)</u>
Denominator:				
Weighted average common shares outstanding—basic and diluted	<u>2,285</u>	<u>4,972</u>	<u>4,236</u>	<u>6,226</u>
Net loss per share applicable to common stockholders—basic and diluted	<u>\$ (9.01)</u>	<u>\$ (0.73)</u>	<u>\$ (1.50)</u>	<u>\$ (1.05)</u>

The following common stock equivalents were excluded from the calculation of diluted net loss per share for the periods indicated because including them would have had an anti-dilutive effect:

	Year ended December 31,		Three months ended March 31,	
	2011	2012	2012	2013
			(unaudited)	
Preferred stock	190,431	310,841	190,431	310,841
Warrants	8,352	8,352	8,352	8,352
Outstanding stock options	29,000	41,751	33,090	69,285
Unvested restricted stock	4,715	2,931	4,204	2,506
	<u>232,498</u>	<u>363,875</u>	<u>236,077</u>	<u>390,984</u>

### 16. Subsequent events

The Company has completed an evaluation of all subsequent events through the filing date of this Registration Statement on Form S-1 with the SEC, to ensure that this filing includes appropriate disclosure of events both recognized in the financial statements as of March 31, 2013, and events which occurred subsequently but were not recognized in the financial statements. The Company has concluded that no subsequent event has occurred that requires disclosure.

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TRANSFORMING THE LIVES OF PATIENTS  
WITH SEVERE GENETIC AND ORPHAN DISEASES

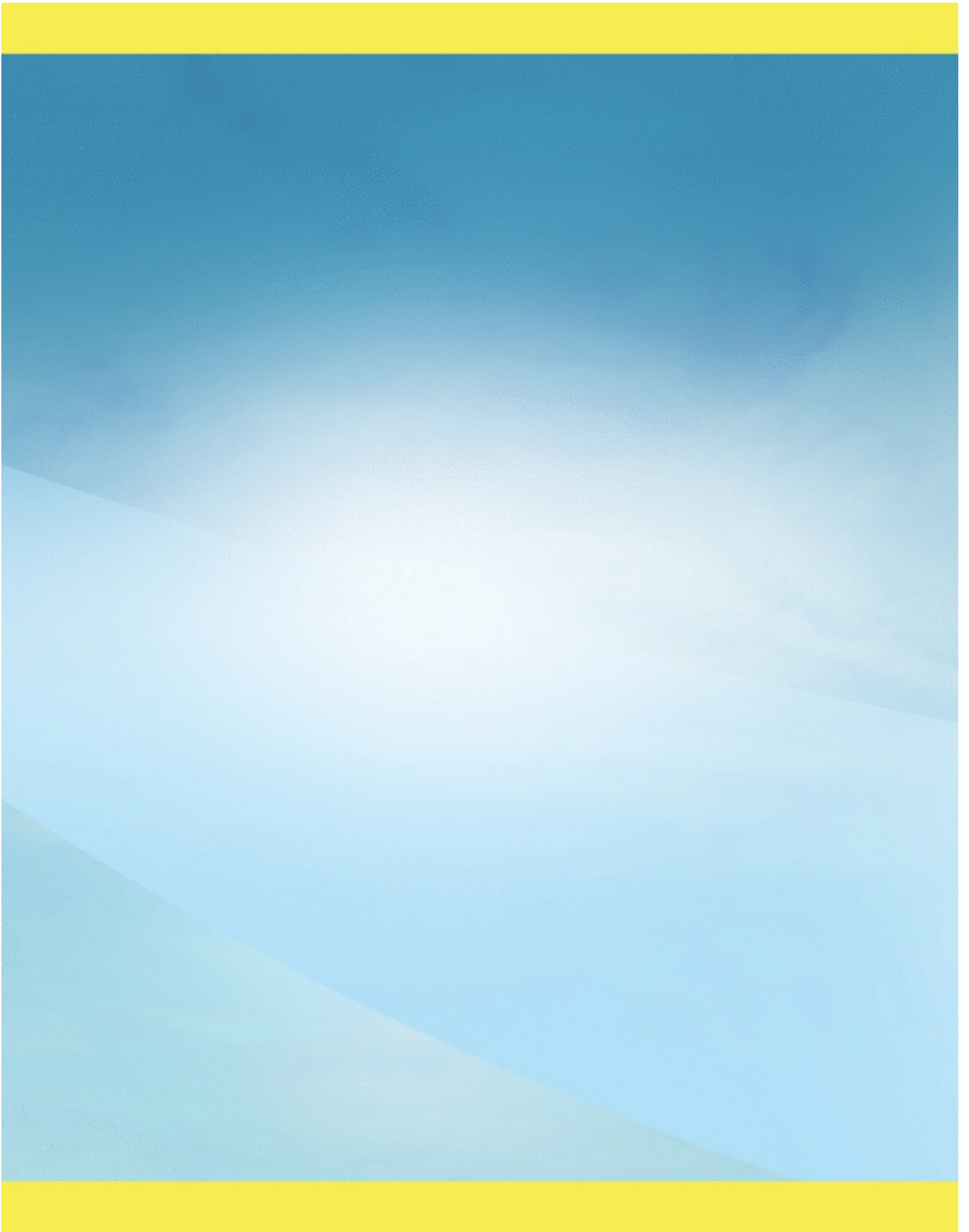


bluebirdbio™

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*shares*



*Common stock*

## Prospectus

**J.P. Morgan**

**BofA Merrill Lynch**

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**Cowen and Company**

**Canaccord Genuity**

**Wedbush PacGrow Life Sciences**

, 2013

We have not authorized anyone to provide you with information other than that contained in this prospectus or in any free writing prospectus prepared by or on behalf of us or to which we have referred you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We are offering to sell, and seeking offers to buy, shares of common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or of any sale of our common stock.

No action is being taken in any jurisdiction outside the United States to permit a public offering of the common stock or possession or distribution of this prospectus in that jurisdiction. Persons who come into possession of this prospectus in jurisdictions outside the United States are required to inform themselves about and to observe any restrictions as to this offering and the distribution of this prospectus applicable to that jurisdiction.

Until , 2013, all dealers that buy, sell or trade in our common stock, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

## Part II

### Information not required in prospectus

#### Item 13. Other expenses of issuance and distribution

The following table sets forth the costs and expenses, other than the underwriting discounts and commissions, payable by the registrant in connection with the sale of common stock being registered. All amounts are estimates except for the Securities and Exchange Commission, or SEC, registration fee, the FINRA filing fee and The Nasdaq Global Market listing fee.

Item	Amount to be paid
SEC registration fee	\$ 11,765
FINRA filing fee	*
Nasdaq Global Market listing fee	*
Printing and engraving expenses	*
Legal fees and expenses	*
Accounting fees and expenses	*
Transfer Agent fees and expenses	*
Miscellaneous expenses	*
Total	\$ *

\* To be provided by amendment

#### Item 14. Indemnification of directors and officers

Section 145(a) of the Delaware General Corporation Law provides, in general, that a corporation may indemnify any person who was or is a party or is threatened to be made a party to any threatened, pending or completed action, suit or proceeding, whether civil, criminal, administrative or investigative (other than an action by or in the right of the corporation), because he or she is or was a director, officer, employee or agent of the corporation, or is or was serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, against expenses (including attorneys' fees), judgments, fines and amounts paid in settlement actually and reasonably incurred by the person in connection with such action, suit or proceeding, if he or she acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the corporation and, with respect to any criminal action or proceeding, had no reasonable cause to believe his or her conduct was unlawful.

Section 145(b) of the Delaware General Corporation Law provides, in general, that a corporation may indemnify any person who was or is a party or is threatened to be made a party to any threatened, pending or completed action or suit by or in the right of the corporation to procure a judgment in its favor because the person is or was a director, officer, employee or agent of the corporation, or is or was serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, against expenses (including attorneys' fees) actually and reasonably incurred by the person in connection with the defense or settlement of such action or suit if he or she acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the corporation, except that no indemnification shall be made with respect to any claim, issue or matter as to which he or she shall have been adjudged to be liable to the

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corporation unless and only to the extent that the Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, he or she is fairly and reasonably entitled to indemnity for such expenses which the Court of Chancery or other adjudicating court shall deem proper.

Section 145(g) of the Delaware General Corporation Law provides, in general, that a corporation may purchase and maintain insurance on behalf of any person who is or was a director, officer, employee or agent of the corporation, or is or was serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise against any liability asserted against such person and incurred by such person in any such capacity, or arising out of his or her status as such, whether or not the corporation would have the power to indemnify the person against such liability under Section 145 of the Delaware General Corporation Law.

Article VII of our amended and restated certificate of incorporation (the "Charter"), provides that no director of our company shall be personally liable to us or our stockholders for monetary damages for any breach of fiduciary duty as a director, except for liability (1) for any breach of the director's duty of loyalty to us or our stockholders, (2) for acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law, (3) in respect of unlawful dividend payments or stock redemptions or repurchases, or (4) for any transaction from which the director derived an improper personal benefit. In addition, our Charter provides that if the Delaware General Corporation Law is amended to authorize the further elimination or limitation of the liability of directors, then the liability of a director of our company shall be eliminated or limited to the fullest extent permitted by the Delaware General Corporation Law, as so amended.

Article VII of the Charter further provides that any repeal or modification of such article by our stockholders or amendment to the Delaware General Corporation Law will not adversely affect any right or protection existing at the time of such repeal or modification with respect to any acts or omissions occurring before such repeal or modification of a director serving at the time of such repeal or modification.

Article V of our amended and restated by-laws (the "By-Laws"), provides that we will indemnify each of our directors and officers and, in the discretion of our board of directors, certain employees, to the fullest extent permitted by the Delaware General Corporation Law as the same may be amended (except that in the case of amendment, only to the extent that the amendment permits us to provide broader indemnification rights than the Delaware General Corporation Law permitted us to provide prior to such the amendment) against any and all expenses, judgments, penalties, fines and amounts reasonably paid in settlement that are incurred by the director, officer or such employee or on the director's, officer's or employee's behalf in connection with any threatened, pending or completed proceeding or any claim, issue or matter therein, to which he or she is or is threatened to be made a party because he or she is or was serving as a director, officer or employee of our company, or at our request as a director, partner, trustee, officer, employee or agent of another corporation, partnership, joint venture, trust, employee benefit plan or other enterprise, if he or she acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of our company and, with respect to any criminal proceeding, had no reasonable cause to believe his or her conduct was unlawful. Article V of the By-Laws further provides for the advancement of expenses to each of our directors and, in the discretion of the board of directors, to certain officers and employees.

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In addition, Article V of the By-Laws provides that the right of each of our directors and officers to indemnification and advancement of expenses shall be a contract right and shall not be exclusive of any other right now possessed or hereafter acquired under any statute, provision of the Charter or By-Laws, agreement, vote of stockholders or otherwise. Furthermore, Article V of the By-Laws authorizes us to provide insurance for our directors, officers and employees, against any liability, whether or not we would have the power to indemnify such person against such liability under the Delaware General Corporation Law or the provisions of Article V of the By-Laws.

In connection with the sale of common stock being registered hereby, we have entered into indemnification agreements with each of our directors and our executive officers. These agreements will provide that we will indemnify each of our directors and such officers to the fullest extent permitted by law and the Charter and By-Laws.

We also maintain a general liability insurance policy which covers certain liabilities of directors and officers of our company arising out of claims based on acts or omissions in their capacities as directors or officers.

In any underwriting agreement we enter into in connection with the sale of common stock being registered hereby, the underwriters will agree to indemnify, under certain conditions, us, our directors, our officers and persons who control us within the meaning of the Securities Act of 1933, as amended, against certain liabilities.

## **Item 15. Recent sales of unregistered securities**

In the three years preceding the filing of this registration statement, we have issued the following securities that were not registered under the Securities Act:

### ***Grants and modifications of warrants***

In May 2007, December 2007, May 2008, August 2008, December 2008, April 2009, July 2009, October 2009 and December 2009, we issued warrants to purchase 1,133,100, 472,124, 472,124, 472,124, 472,124, 321,044, 321,044, 283,274, and 574,800 shares, respectively, of either (i) our Series A-1 Preferred Stock or (ii) such preferred stock that we may issue in a subsequent qualified financing. In March 2010, in connection with the Series B Preferred Stock financing, the 2007, 2008 and the April, July and October 2009 warrants were amended to provide that such warrants would be exercisable only for shares of our Series A-1 Preferred Stock at a per share price of \$0.6619 and the December 2009 warrants were amended to provide that such warrants would be exercisable only for shares of our Series B Preferred Stock at a per share price of \$0.3262. The warrant issuances were exempt pursuant to Section 4(2), as transactions by an issuer not involving a public offering. The shares of preferred stock issued upon exercise of warrants and the shares of common stock issued upon conversion of the preferred stock are deemed restricted securities for the purposes of the Securities Act.

### ***Grants and exercises of stock options***

Since January 1, 2010, we have granted stock options to purchase an aggregate of 74,066,242 shares of our common stock at exercise prices ranging from \$0.05 to \$0.43. Since January 1, 2010, we have issued an aggregate of 550,746 shares of our common stock upon exercise of stock options granted pursuant to our 2002 Employee, Director and Consultant Plan and our 2010 Stock Option and Grant Plan for aggregate consideration of \$60,519.

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The option grants and the issuances of common stock upon exercise of the options were exempt either pursuant to Rule 701, as a transaction pursuant to a compensatory benefit plan, or pursuant to Section 4(2), as a transaction by an issuer not involving a public offering. The shares of common stock issued upon exercise of options are deemed restricted securities for the purposes of the Securities Act.

***Issuances of capital stock***

Since January 1, 2010, we have granted and issued an aggregate of 7,327,566 shares of our common stock pursuant to our 2010 Stock Option and Grant Plan. The issuances of common stock were exempt either pursuant to Rule 701, as a transaction pursuant to a compensatory benefit plan, or pursuant to Section 4(2), as a transaction by an issuer not involving a public offering. The shares of common stock issued pursuant to our 2010 Stock Option and Grant Plan are deemed restricted securities for the purposes of the Securities Act.

In March 2010, we issued an aggregate of 61,555,660 shares of our Series B Preferred Stock for aggregate consideration of \$16.8 million in cash and \$3.3 million in converted bridge notes to five investors. In April 2011, we issued an aggregate of 53,648,066 shares of our Series B Preferred Stock at a price per share of \$0.3262 for aggregate consideration of \$17.5 million to the same five investors. In April 2011, we issued an aggregate of 39,942,483 shares of our Series C Preferred Stock at a price per share of \$0.37554 to five investors for aggregate consideration of \$15.0 million to the same five investors. In July 2012, we issued an aggregate of 120,409,385 shares of our Series D Preferred Stock at a price per share of \$0.4983 for aggregate consideration of \$60.0 million to 17 investors. These preferred stock issuances were exempt under the Securities Act pursuant to Section 4(2) and/or Regulation D promulgated thereunder as transactions not involving a public offering.

**Item 16. Exhibits and financial statement schedules**

***(a) Exhibits***

See the Exhibit Index attached to this Registration Statement, which is incorporated by reference herein.

***(b) Financial statement schedules***

Schedules not listed above have been omitted because the information required to be set forth therein is not applicable or is shown in the financial statements or notes thereto.

**Item 17. Undertakings**

The undersigned Registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the Registrant pursuant to the foregoing provisions, or otherwise, the Registrant has been advised that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act, and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the

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Registrant of expenses incurred or paid by a director, officer, or controlling person of the Registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the Registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question of whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

The undersigned Registrant hereby undertakes that:

1. For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this Registration Statement in reliance upon Rule 430A and contained in a form of prospectus filed by the Registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this Registration Statement as of the time it was declared effective.
2. For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

## Signatures

Pursuant to the requirements of the Securities Act of 1933, as amended, the Registrant has duly caused this Registration Statement on Form S-1 to be signed on its behalf by the undersigned, thereunto duly authorized, in the city of Cambridge, Commonwealth of Massachusetts, on the 14th day of May, 2013.

**bluebird bio, Inc.**

<sup>By</sup> /s/ Nick Leschly  
Nick Leschly  
President and Chief Executive Officer

## Signatures and power of attorney

We, the undersigned directors and officers of bluebird bio, Inc. (the "Company"), hereby severally constitute and appoint Nick Leschly and Jeffrey T. Walsh, and each of them singly, our true and lawful attorneys, with full power to them, and to each of them singly, to sign for us and in our names in the capacities indicated below, the registration statement on Form S-1 filed herewith, and any and all pre-effective and post-effective amendments to said registration statement, and any registration statement filed pursuant to Rule 462(b) under the Securities Act of 1933, as amended, in connection with the registration under the Securities Act of 1933, as amended, of equity securities of the Company, and to file or cause to be filed the same, with all exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith, as fully to all intents and purposes as each of us might or could do in person, and hereby ratifying and confirming all that said attorneys, and each of them, or their substitute or substitutes, shall do or cause to be done by virtue of this Power of Attorney.

Pursuant to the requirements of the Securities Act, this Registration Statement on Form S-1 has been signed by the following persons in the capacities and on the dates indicated.

<b>Signature</b>	<b>Title</b>	<b>Date</b>
<u>/s/ Nick Leschly</u> Nick Leschly	President, Chief Executive Officer and Director (Principal Executive Officer)	May 14, 2013
<u>/s/ Jeffrey T. Walsh</u> Jeffrey T. Walsh	Chief Operating Officer and Secretary (Principal Financial Officer)	May 14, 2013
<u>/s/ Linda C. Bain</u> Linda C. Bain	Vice President, Finance and Business Operations and Treasurer (Principal Accounting Officer)	May 14, 2013
<u>/s/ Daniel S. Lynch</u> Daniel S. Lynch	Chairman of the Board	May 14, 2013
<u>/s/ Wendy L. Dixon, Ph.D.</u> Wendy L. Dixon, Ph.D.	Director	May 14, 2013
<u>/s/ Steven Gillis, Ph.D.</u> Steven Gillis, Ph.D.	Director	May 14, 2013

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<b>Signature</b>	<b>Title</b>	<b>Date</b>
<u>/s/ John M. Maraganore, Ph.D.</u> John M. Maraganore, Ph.D.	Director	May 14, 2013
<u>/s/ Geert-Jan Mulder, M.D.</u> Geert-Jan Mulder, M.D.	Director	May 14, 2013
<u>/s/ Dr. Axel Polack</u> Dr. Axel Polack	Director	May 14, 2013
<u>/s/ David P. Schenkein, M.D.</u> David P. Schenkein, M.D.	Director	May 14, 2013
<u>/s/ Robert I. Tepper, M.D.</u> Robert I. Tepper, M.D.	Director	May 14, 2013

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## Exhibit index

<b>Exhibit number</b>	<b>Description of exhibit</b>
1.1*	Form of Underwriting Agreement.
3.1*	Form of Amended and Restated Certificate of Incorporation (to be effective upon pricing of this offering).
3.2*	Form of Amended and Restated Certificate of Incorporation (to be effective upon completion of this offering).
3.3	Form of Amended and Restated By-laws.
4.1*	Specimen Common Stock Certificate.
4.2	Form of Common Stock Warrant.
4.3	Form of Series A-1 Preferred Stock Warrant.
4.4	Form of Series B Preferred Stock Warrant.
4.5	Amended and Restated Investors' Rights Agreement, dated as of July 23, 2012, by and among the Registrant and the Investors listed therein.
5.1*	Opinion of Goodwin Procter LLP.
10.1	Second Amended and Restated 2002 Employee, Director and Consultant Plan, as amended, and forms of award agreement thereunder.
10.2	2010 Stock Option and Grant Plan, as amended, and forms of award agreement thereunder.
10.3*	2013 Stock Option and Incentive Plan and forms of award agreement thereunder.
10.4	Form of Indemnification Agreement between the Registrant and each of its Executive Officers and Directors.
10.5	Amended and Restated Lease Agreement, dated May 18, 2007, by and between the Registrant and Rivertech Associates II, LLC, as amended.
10.6†	Patent License Agreement, dated December 11, 1996, by and between the Registrant (formerly known as Genetix Pharmaceuticals Inc., successor-in-interest to Innogene Pharmaceuticals Inc.) and Massachusetts Institute of Technology, as amended.
10.7†	Patent and Know-How License Agreement No. 07554F30, dated May 14, 2009, by and between the Registrant (formerly known as Genetix Pharmaceuticals Inc.) and INSERM-TRANSFERT, as amended.
10.8†	License Agreement, dated September 13, 2011, by and between the Registrant and Institut Pasteur, as amended.
10.9†	License Agreement, dated December 7, 2011, by and between the Registrant and Research Development Foundation.
10.10†	Novation Agreement, dated April 2, 2012, by and between the Registrant and The Board of Trustees of the Leland Stanford Junior University.
10.11†	Master Collaboration Agreement by and between the Registrant and Celgene Corporation, dated March 19, 2013.
10.12*	Form of Amended and Restated Employment Agreement by and between the Registrant and Nick Leschly.
10.13*	Form of Amended and Restated Employment Agreement by and between the Registrant and Jeffrey T. Walsh.

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<b>Exhibit number</b>	<b>Description of exhibit</b>
10.14*	Form of Amended and Restated Employment Agreement by and between the Registrant and Mitch Finer.
10.15*	Form of Amended and Restated Employment Agreement by and between the Registrant and David M. Davidson, M.D.
10.16*	Offer Letter, dated September 27, 2011 by and between the Registrant and Linda Bain.
10.17*	2013 Employee Stock Purchase Plan.
10.18	Executive Cash Incentive Bonus Plan.
21.1	Subsidiaries of Registrant.
23.1	Consent of Ernst & Young LLP.
23.2	Consent of McGladrey LLP.
23.3*	Consent of Goodwin Procter LLP (included in Exhibit 5.1).
24.1	Power of Attorney (included on signature page).

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\* To be filed by amendment.

† Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment and this exhibit has been submitted separately to the SEC.

**AMENDED AND RESTATED**  
**BY-LAWS**  
**OF**  
**BLUEBIRD BIO, INC.**  
(the "Corporation")

ARTICLE I  
Stockholders

SECTION 1. Annual Meeting. The annual meeting of stockholders of the Corporation (any such meeting being referred to in these By-laws as an "Annual Meeting") shall be held at the hour, date and place within or without the United States which is fixed by the Board of Directors, which time, date and place may subsequently be changed at any time by vote of the Board of Directors. If no Annual Meeting has been held for a period of thirteen (13) months after the Corporation's last Annual Meeting, a special meeting in lieu thereof may be held, and such special meeting shall have, for the purposes of these By-laws or otherwise, all the force and effect of an Annual Meeting. Any and all references hereafter in these By-laws to an Annual Meeting or Annual Meetings shall be deemed to also refer to any special meeting(s) in lieu thereof.

SECTION 2. Notice of Stockholder Business and Nominations.

(a) Annual Meetings of Stockholders.

(1) Nominations of persons for election to the Board of Directors of the Corporation and the proposal of other business to be considered by the stockholders may be brought before an Annual Meeting (i) by or at the direction of the Board of Directors or (ii) by any stockholder of the Corporation who was a stockholder of record at the time of giving of notice provided for in this By-law, who is entitled to vote at the meeting, who is present (in person or by proxy) at the meeting and who complies with the notice procedures set forth in this By-law as to such nomination or business. For the avoidance of doubt, the foregoing clause (ii) shall be the exclusive means for a stockholder to bring nominations or business properly before an Annual Meeting (other than matters properly brought under Rule 14a-8 or Rule 14a-11 (or any successor rules) under the Securities Exchange Act of 1934, as amended (the "Exchange Act")), and such stockholder must comply with the notice and other procedures set forth in Article I, Section 2(a)(2) and (3) of this By-law to bring such nominations or business properly before an Annual Meeting. In addition to the other requirements set forth in this By-law, for any proposal of business to be considered at an Annual Meeting, it must be a proper subject for action by stockholders of the Corporation under Delaware law.

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(2) For nominations or other business to be properly brought before an Annual Meeting by a stockholder pursuant to clause (ii) of Article I, Section 2(a)(1) of this By-law, the stockholder must (i) have given Timely Notice (as defined below) thereof in writing to the Secretary of the Corporation, (ii) have provided any updates or supplements to such notice at the times and in the forms required by this By-law and (iii) together with the beneficial owner(s), if any, on whose behalf the nomination or business proposal is made, have acted in accordance with the representations set forth in the Solicitation Statement (as defined below) required by this By-law. To be timely, a stockholder's written notice shall be received by the Secretary at the principal executive offices of the Corporation not later than the close of business on the ninetieth (90th) day nor earlier than the close of business on the one hundred twentieth (120th) day prior to the one-year anniversary of the preceding year's Annual Meeting; provided, however, that in the event the Annual Meeting is first convened more than thirty (30) days before or more than sixty (60) days after such anniversary date, or if no Annual Meeting were held in the preceding year, notice by the stockholder to be timely must be received by the Secretary of the Corporation not later than the close of business on the later of the ninetieth (90th) day prior to the scheduled date of such Annual Meeting or the tenth (10th) day following the day on which public announcement of the date of such meeting is first made (such notice within such time periods shall be referred to as "Timely Notice"). Notwithstanding anything to the contrary provided herein, for the first Annual Meeting following the initial public offering of common stock of the Corporation, a stockholder's notice shall be timely if received by the Secretary at the principal executive offices of the Corporation not later than the close of business on the later of the ninetieth (90th) day prior to the scheduled date of such Annual Meeting or the tenth (10th) day following the day on which public announcement of the date of such Annual Meeting is first made or sent by the Corporation. Such stockholder's Timely Notice shall set forth:

(A) as to each person whom the stockholder proposes to nominate for election or reelection as a director, all information relating to such person that is required to be disclosed in solicitations of proxies for election of directors in an election contest, or is otherwise required, in each case pursuant to Regulation 14A under the Exchange Act (including such person's written consent to being named in the proxy statement as a nominee and to serving as a director if elected);

(B) as to any other business that the stockholder proposes to bring before the meeting, a brief description of the business desired to be brought before the meeting, the reasons for conducting such business at the meeting, and any material interest in such business of each Proposing Person (as defined below);

(C) (i) the name and address of the stockholder giving the notice, as they appear on the Corporation's books, and the names and addresses of the other Proposing Persons (if any) and (ii) as to each Proposing Person, the following information: (a) the class or series and number of all shares of capital stock of the Corporation which are, directly or indirectly, owned beneficially or of record by such Proposing Person or any of its affiliates or associates (as such terms are defined in Rule 12b-2 promulgated under the Exchange Act), including any shares of any class or series of capital stock of the Corporation as to which such

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Proposing Person or any of its affiliates or associates has a right to acquire beneficial ownership at any time in the future; (b) all Synthetic Equity Interests (as defined below) in which such Proposing Person or any of its affiliates or associates, directly or indirectly, holds an interest including a description of the material terms of each such Synthetic Equity Interest, including without limitation, identification of the counterparty to each such Synthetic Equity Interest and disclosure, for each such Synthetic Equity Interest, as to (x) whether or not such Synthetic Equity Interest conveys any voting rights, directly or indirectly, in such shares to such Proposing Person, (y) whether or not such Synthetic Equity Interest is required to be, or is capable of being, settled through delivery of such shares and (z) whether or not such Proposing Person and/or, to the extent known, the counterparty to such Synthetic Equity Interest has entered into other transactions that hedge or mitigate the economic effect of such Synthetic Equity Interest; (c) any proxy (other than a revocable proxy given in response to a public proxy solicitation made pursuant to, and in accordance with, the Exchange Act), agreement, arrangement, understanding or relationship pursuant to which such Proposing Person has or shares a right to, directly or indirectly, vote any shares of any class or series of capital stock of the Corporation; (d) any rights to dividends or other distributions on the shares of any class or series of capital stock of the Corporation, directly or indirectly, owned beneficially by such Proposing Person that are separated or separable from the underlying shares of the Corporation; and (e) any performance-related fees (other than an asset based fee) that such Proposing Person, directly or indirectly, is entitled to based on any increase or decrease in the value of shares of any class or series of capital stock of the Corporation or any Synthetic Equity Interests (the disclosures to be made pursuant to the foregoing clauses (a) through (e) are referred to, collectively, as "Material Ownership Interests") and (iii) a description of the material terms of all agreements, arrangements or understandings (whether or not in writing) entered into by any Proposing Person or any of its affiliates or associates with any other person for the purpose of acquiring, holding, disposing or voting of any shares of any class or series of capital stock of the Corporation;

(D) (i) a description of all agreements, arrangements or understandings by and among any of the Proposing Persons, or by and among any Proposing Persons and any other person (including with any proposed nominee(s)), pertaining to the nomination(s) or other business proposed to be brought before the meeting of stockholders (which description shall identify the name of each other person who is party to such an agreement, arrangement or understanding), and (ii) identification of the names and addresses of other stockholders (including beneficial owners) known by any of the Proposing Persons to support such nominations or other business proposal(s), and to the extent known the class and number of all shares of the Corporation's capital stock owned beneficially or of record by such other stockholder(s) or other beneficial owner(s); and

(E) a statement whether or not the stockholder giving the notice and/or the other Proposing Person(s), if any, will deliver a proxy statement and form of proxy to holders of, in the case of a business proposal, at least the percentage of

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voting power of all of the shares of capital stock of the Corporation required under applicable law to approve the proposal or, in the case of a nomination or nominations, at least the percentage of voting power of all of the shares of capital stock of the Corporation reasonably believed by such Proposing Person to be sufficient to elect the nominee or nominees proposed to be nominated by such stockholder (such statement, the "Solicitation Statement").

For purposes of this Article I of these By-laws, the term "Proposing Person" shall mean the following persons: (i) the stockholder of record providing the notice of nominations or business proposed to be brought before a stockholders' meeting, and (ii) the beneficial owner(s), if different, on whose behalf the nominations or business proposed to be brought before a stockholders' meeting is made. For purposes of this Section 2 of Article I of these By-laws, the term "Synthetic Equity Interest" shall mean any transaction, agreement or arrangement (or series of transactions, agreements or arrangements), including, without limitation, any derivative, swap, hedge, repurchase or so-called "stock borrowing" agreement or arrangement, the purpose or effect of which is to, directly or indirectly: (a) give a person or entity economic benefit and/or risk similar to ownership of shares of any class or series of capital stock of the Corporation, in whole or in part, including due to the fact that such transaction, agreement or arrangement provides, directly or indirectly, the opportunity to profit or avoid a loss from any increase or decrease in the value of any shares of any class or series of capital stock of the Corporation, (b) mitigate loss to, reduce the economic risk of or manage the risk of share price changes for, any person or entity with respect to any shares of any class or series of capital stock of the Corporation, (c) otherwise provide in any manner the opportunity to profit or avoid a loss from any decrease in the value of any shares of any class or series of capital stock of the Corporation, or (d) increase or decrease the voting power of any person or entity with respect to any shares of any class or series of capital stock of the Corporation.

(3) A stockholder providing Timely Notice of nominations or business proposed to be brought before an Annual Meeting shall further update and supplement such notice, if necessary, so that the information (including, without limitation, the Material Ownership Interests information) provided or required to be provided in such notice pursuant to this By-law shall be true and correct as of the record date for the meeting and as of the date that is ten (10) business days prior to such Annual Meeting, and such update and supplement shall be received by the Secretary at the principal executive offices of the Corporation not later than the close of business on the fifth (5th) business day after the record date for the Annual Meeting (in the case of the update and supplement required to be made as of the record date), and not later than the close of business on the eighth (8th) business day prior to the date of the Annual Meeting (in the case of the update and supplement required to be made as of ten (10) business days prior to the meeting).

(4) Notwithstanding anything in the second sentence of Article I, Section 2(a)(2) of this By-law to the contrary, in the event that the number of directors to be elected to the Board of Directors of the Corporation is increased and there is no public announcement naming all of the nominees for director or specifying the size of the

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increased Board of Directors made by the Corporation at least ten (10) days before the last day a stockholder may deliver a notice of nomination in accordance with the second sentence of Article I, Section 2(a)(2), a stockholder's notice required by this By-law shall also be considered timely, but only with respect to nominees for any new positions created by such increase, if it shall be received by the Secretary of the Corporation not later than the close of business on the tenth (10th) day following the day on which such public announcement is first made by the Corporation.

(b) General.

(1) Only such persons who are nominated in accordance with the provisions of this By-law or in accordance with Rule 14a-11 under the Exchange Act shall be eligible for election and to serve as directors and only such business shall be conducted at an Annual Meeting as shall have been brought before the meeting in accordance with the provisions of this By-law or in accordance with Rule 14a-8 under the Exchange Act. The Board of Directors or a designated committee thereof shall have the power to determine whether a nomination or any business proposed to be brought before the meeting was made in accordance with the provisions of this By-law. If neither the Board of Directors nor such designated committee makes a determination as to whether any stockholder proposal or nomination was made in accordance with the provisions of this By-law, the presiding officer of the Annual Meeting shall have the power and duty to determine whether the stockholder proposal or nomination was made in accordance with the provisions of this By-law. If the Board of Directors or a designated committee thereof or the presiding officer, as applicable, determines that any stockholder proposal or nomination was not made in accordance with the provisions of this By-law, such proposal or nomination shall be disregarded and shall not be presented for action at the Annual Meeting.

(2) Except as otherwise required by law, nothing in this Article I, Section 2 shall obligate the Corporation or the Board of Directors to include in any proxy statement or other stockholder communication distributed on behalf of the Corporation or the Board of Directors information with respect to any nominee for director or any other matter of business submitted by a stockholder.

(3) Notwithstanding the foregoing provisions of this Article I, Section 2, if the nominating or proposing stockholder (or a qualified representative of the stockholder) does not appear at the Annual Meeting to present a nomination or any business, such nomination or business shall be disregarded, notwithstanding that proxies in respect of such vote may have been received by the Corporation. For purposes of this Article I, Section 2, to be considered a qualified representative of the proposing stockholder, a person must be authorized by a written instrument executed by such stockholder or an electronic transmission delivered by such stockholder to act for such stockholder as proxy at the meeting of stockholders and such person must produce such written instrument or electronic transmission, or a reliable reproduction of the written instrument or electronic transmission, to the presiding officer at the meeting of stockholders.

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(4) For purposes of this By-law, “public announcement” shall mean disclosure in a press release reported by the Dow Jones News Service, Associated Press or comparable national news service or in a document publicly filed by the Corporation with the Securities and Exchange Commission pursuant to Section 13, 14 or 15(d) of the Exchange Act.

(5) Notwithstanding the foregoing provisions of this By-law, a stockholder shall also comply with all applicable requirements of the Exchange Act and the rules and regulations thereunder with respect to the matters set forth in this By-law. Nothing in this By-law shall be deemed to affect any rights of (i) stockholders to have nominations or proposals included in the Corporation’s proxy statement pursuant to Rule 14a-8 or Rule 14a-11 (or any successor rules), as applicable, under the Exchange Act and, to the extent required by such rule, have such nominations or proposals considered and voted on at an Annual Meeting or (ii) the holders of any series of Undesignated Preferred Stock to elect directors under specified circumstances.

SECTION 3. Special Meetings. Except as otherwise required by statute and subject to the rights, if any, of the holders of any series of Undesignated Preferred Stock, special meetings of the stockholders of the Corporation may be called only by the Board of Directors acting pursuant to a resolution approved by the affirmative vote of a majority of the Directors then in office. The Board of Directors may postpone or reschedule any previously scheduled special meeting of stockholders. Only those matters set forth in the notice of the special meeting may be considered or acted upon at a special meeting of stockholders of the Corporation. Nominations of persons for election to the Board of Directors of the Corporation and stockholder proposals of other business shall not be brought before a special meeting of stockholders to be considered by the stockholders unless such special meeting is held in lieu of an annual meeting of stockholders in accordance with Article I, Section 1 of these By-laws, in which case such special meeting in lieu thereof shall be deemed an Annual Meeting for purposes of these By-laws and the provisions of Article I, Section 2 of these By-laws shall govern such special meeting.

SECTION 4. Notice of Meetings: Adjournments.

(a) A notice of each Annual Meeting stating the hour, date and place, if any, of such Annual Meeting and the means of remote communication, if any, by which stockholders and proxyholders may be deemed to be present in person and vote at such meeting, shall be given not less than ten (10) days nor more than sixty (60) days before the Annual Meeting, to each stockholder entitled to vote thereat by delivering such notice to such stockholder or by mailing it, postage prepaid, addressed to such stockholder at the address of such stockholder as it appears on the Corporation’s stock transfer books. Without limiting the manner by which notice may otherwise be given to stockholders, any notice to stockholders may be given by electronic transmission in the manner provided in Section 232 of the Delaware General Corporation Law (“DGCL”).

(b) Notice of all special meetings of stockholders shall be given in the same manner as provided for Annual Meetings, except that the notice of all special meetings shall state the purpose or purposes for which the meeting has been called.



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(c) Notice of an Annual Meeting or special meeting of stockholders need not be given to a stockholder if a waiver of notice is executed, or waiver of notice by electronic transmission is provided, before or after such meeting by such stockholder or if such stockholder attends such meeting, unless such attendance is for the express purpose of objecting at the beginning of the meeting to the transaction of any business because the meeting was not lawfully called or convened.

(d) The Board of Directors may postpone and reschedule any previously scheduled Annual Meeting or special meeting of stockholders and any record date with respect thereto, regardless of whether any notice or public disclosure with respect to any such meeting has been sent or made pursuant to Section 2 of this Article I of these By-laws or otherwise. In no event shall the public announcement of an adjournment, postponement or rescheduling of any previously scheduled meeting of stockholders commence a new time period for the giving of a stockholder's notice under this Article I of these By-laws.

(e) When any meeting is convened, the presiding officer may adjourn the meeting if (i) no quorum is present for the transaction of business, (ii) the Board of Directors determines that adjournment is necessary or appropriate to enable the stockholders to consider fully information which the Board of Directors determines has not been made sufficiently or timely available to stockholders, or (iii) the Board of Directors determines that adjournment is otherwise in the best interests of the Corporation. When any Annual Meeting or special meeting of stockholders is adjourned to another hour, date or place, notice need not be given of the adjourned meeting other than an announcement at the meeting at which the adjournment is taken of the hour, date and place, if any, to which the meeting is adjourned and the means of remote communications, if any, by which stockholders and proxyholders may be deemed to be present in person and vote at such adjourned meeting; provided, however, that if the adjournment is for more than thirty (30) days from the meeting date, or if after the adjournment a new record date is fixed for the adjourned meeting, notice of the adjourned meeting and the means of remote communications, if any, by which stockholders and proxyholders may be deemed to be present in person and vote at such adjourned meeting shall be given to each stockholder of record entitled to vote thereat and each stockholder who, by law or under the Certificate of Incorporation of the Corporation (as the same may hereafter be amended and/or restated, the "Certificate") or these By-laws, is entitled to such notice.

SECTION 5. Quorum. A majority of the shares entitled to vote, present in person or represented by proxy, shall constitute a quorum at any meeting of stockholders. If less than a quorum is present at a meeting, the holders of voting stock representing a majority of the voting power present at the meeting or the presiding officer may adjourn the meeting from time to time, and the meeting may be held as adjourned without further notice, except as provided in Section 4 of this Article I. At such adjourned meeting at which a quorum is present, any business may be transacted which might have been transacted at the meeting as originally noticed. The stockholders present at a duly constituted meeting may continue to transact business until adjournment, notwithstanding the withdrawal of enough stockholders to leave less than a quorum.

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SECTION 6. Voting and Proxies. Stockholders shall have one vote for each share of stock entitled to vote owned by them of record according to the stock ledger of the Corporation as of the record date, unless otherwise provided by law or by the Certificate. Stockholders may vote either (i) in person, (ii) by written proxy or (iii) by a transmission permitted by Section 212(c) of the DGCL. Any copy, facsimile telecommunication or other reliable reproduction of the writing or transmission permitted by Section 212(c) of the DGCL may be substituted for or used in lieu of the original writing or transmission for any and all purposes for which the original writing or transmission could be used, provided that such copy, facsimile telecommunication or other reproduction shall be a complete reproduction of the entire original writing or transmission. Proxies shall be filed in accordance with the procedures established for the meeting of stockholders. Except as otherwise limited therein or as otherwise provided by law, proxies authorizing a person to vote at a specific meeting shall entitle the persons authorized thereby to vote at any adjournment of such meeting, but they shall not be valid after final adjournment of such meeting. A proxy with respect to stock held in the name of two or more persons shall be valid if executed by or on behalf of any one of them unless at or prior to the exercise of the proxy the Corporation receives a specific written notice to the contrary from any one of them.

SECTION 7. Action at Meeting. When a quorum is present at any meeting of stockholders, any matter before any such meeting (other than an election of a director or directors) shall be decided by a majority of the votes properly cast for and against such matter, except where a larger vote is required by law, by the Certificate or by these By-laws. Any election of directors by stockholders shall be determined by a plurality of the votes properly cast on the election of directors.

SECTION 8. Stockholder Lists. The Secretary or an Assistant Secretary, if any (or the Corporation's transfer agent or other person authorized by these By-laws or by law) shall prepare and make, at least ten (10) days before every Annual Meeting or special meeting of stockholders, a complete list of the stockholders entitled to vote at the meeting, arranged in alphabetical order, and showing the address of each stockholder and the number of shares registered in the name of each stockholder. Such list shall be open to the examination of any stockholder, for a period of at least ten (10) days prior to the meeting in the manner provided by law. The list shall also be open to the examination of any stockholder during the whole time of the meeting as provided by law.

SECTION 9. Presiding Officer. The Board of Directors shall designate a representative to preside over all Annual Meetings or special meetings of stockholders, provide that if the Board of Directors does not so designate such a presiding officer, then the Chairman of the Board, if one is elected, shall preside over such meetings. If the Board of Directors does not so designate such a presiding officer and there is no Chairman of the Board or the Chairman of the Board is unable to so preside or is absent, then the Chief Executive Officer, if one is elected, shall preside over such meetings, provided further that if there is no Chief Executive Officer or the Chief Executive Officer is unable to so preside or is absent, then the President shall preside over such meetings. The presiding officer at any Annual Meeting or special meeting of stockholders shall have the power, among other things, to adjourn such meeting at any time and from time to time, subject to Sections 4 and 5 of this Article I. The order of business and all other matters of procedure at any meeting of the stockholders shall be determined by the presiding officer.

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SECTION 10. Inspectors of Elections. The Corporation shall, in advance of any meeting of stockholders, appoint one or more inspectors to act at the meeting and make a written report thereof. The Corporation may designate one or more persons as alternate inspectors to replace any inspector who fails to act. If no inspector or alternate is able to act at a meeting of stockholders, the presiding officer shall appoint one or more inspectors to act at the meeting. Any inspector may, but need not, be an officer, employee or agent of the Corporation. Each inspector, before entering upon the discharge of his or her duties, shall take and sign an oath faithfully to execute the duties of inspector with strict impartiality and according to the best of his or her ability. The inspectors shall perform such duties as are required by the DGCL, including the counting of all votes and ballots. The inspectors may appoint or retain other persons or entities to assist the inspectors in the performance of the duties of the inspectors. The presiding officer may review all determinations made by the inspectors, and in so doing the presiding officer shall be entitled to exercise his or her sole judgment and discretion and he or she shall not be bound by any determinations made by the inspectors. All determinations by the inspectors and, if applicable, the presiding officer, shall be subject to further review by any court of competent jurisdiction.

## ARTICLE II

### Directors

SECTION 1. Powers. The business and affairs of the Corporation shall be managed by or under the direction of the Board of Directors except as otherwise provided by the Certificate or required by law.

SECTION 2. Number and Terms. The number of directors of the Corporation shall be fixed solely and exclusively by resolution duly adopted from time to time by the Board of Directors. The directors shall hold office in the manner provided in the Certificate.

SECTION 3. Qualification. No director need be a stockholder of the Corporation.

SECTION 4. Vacancies. Vacancies in the Board of Directors shall be filled in the manner provided in the Certificate.

SECTION 5. Removal. Directors may be removed from office only in the manner provided in the Certificate.

SECTION 6. Resignation. A director may resign at any time by giving written notice to the Chairman of the Board, if one is elected, the President or the Secretary. A resignation shall be effective upon receipt, unless the resignation otherwise provides.

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SECTION 7. Regular Meetings. The regular annual meeting of the Board of Directors shall be held, without notice other than this Section 7, on the same date and at the same place as the Annual Meeting following the close of such meeting of stockholders. Other regular meetings of the Board of Directors may be held at such hour, date and place as the Board of Directors may by resolution from time to time determine and publicize by means of reasonable notice given to any director who is not present at the meeting at which such resolution is adopted.

SECTION 8. Special Meetings. Special meetings of the Board of Directors may be called, orally or in writing, by or at the request of a majority of the directors, the Chairman of the Board, if one is elected, or the President. The person calling any such special meeting of the Board of Directors may fix the hour, date and place thereof.

SECTION 9. Notice of Special Meetings. Notice of the hour, date and place of all special meetings of the Board of Directors shall be given to each director by the Secretary or an Assistant Secretary, or in case of the death, absence, incapacity or refusal of such persons, by the Chairman of the Board, if one is elected, or the President or such other officer designated by the Chairman of the Board, if one is elected, or the President. Notice of any special meeting of the Board of Directors shall be given to each director in person, by telephone, or by facsimile, electronic mail or other form of electronic communication, sent to his or her business or home address, at least twenty-four (24) hours in advance of the meeting, or by written notice mailed to his or her business or home address, at least forty-eight (48) hours in advance of the meeting. Such notice shall be deemed to be delivered when hand-delivered to such address, read to such director by telephone, deposited in the mail so addressed, with postage thereon prepaid if mailed, dispatched or transmitted if sent by facsimile transmission or by electronic mail or other form of electronic communications. A written waiver of notice signed before or after a meeting by a director and filed with the records of the meeting shall be deemed to be equivalent to notice of the meeting. The attendance of a director at a meeting shall constitute a waiver of notice of such meeting, except where a director attends a meeting for the express purpose of objecting at the beginning of the meeting to the transaction of any business because such meeting is not lawfully called or convened. Except as otherwise required by law, by the Certificate or by these By-laws, neither the business to be transacted at, nor the purpose of, any meeting of the Board of Directors need be specified in the notice or waiver of notice of such meeting.

SECTION 10. Quorum. At any meeting of the Board of Directors, a majority of the total number of directors shall constitute a quorum for the transaction of business, but if less than a quorum is present at a meeting, a majority of the directors present may adjourn the meeting from time to time, and the meeting may be held as adjourned without further notice. Any business that might have been transacted at the meeting as originally noticed may be transacted at such adjourned meeting at which a quorum is present. For purposes of this section, the total number of directors includes any unfilled vacancies on the Board of Directors.

SECTION 11. Action at Meeting. At any meeting of the Board of Directors at which a quorum is present, the vote of a majority of the directors present shall constitute action by the Board of Directors, unless otherwise required by law, by the Certificate or by these By-laws.

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SECTION 12. Action by Consent. Any action required or permitted to be taken at any meeting of the Board of Directors may be taken without a meeting if all members of the Board of Directors consent thereto in writing or by electronic transmission and the writing or writings or electronic transmission or transmissions are filed with the records of the meetings of the Board of Directors. Such filing shall be in paper form if the minutes are maintained in paper form and shall be in electronic form if the minutes are maintained in electronic form. Such consent shall be treated as a resolution of the Board of Directors for all purposes.

SECTION 13. Manner of Participation. Directors may participate in meetings of the Board of Directors by means of conference telephone or other communications equipment by means of which all directors participating in the meeting can hear each other, and participation in a meeting in accordance herewith shall constitute presence in person at such meeting for purposes of these By-laws.

SECTION 14. Presiding Director. The Board of Directors shall designate a representative to preside over all meetings of the Board of Directors, provided that if the Board of Directors does not so designate such a presiding director or such designated presiding director is unable to so preside or is absent, then the Chairman of the Board, if one is elected, shall preside over all meetings of the Board of Directors. If both the designated presiding director, if one is so designated, and the Chairman of the Board, if one is elected, are unable to preside or are absent, the Board of Directors shall designate an alternate representative to preside over a meeting of the Board of Directors.

SECTION 15. Committees. The Board of Directors, by vote of a majority of the directors then in office, may elect one or more committees, including, without limitation, a Compensation Committee, a Nominating & Corporate Governance Committee and an Audit Committee, and may delegate thereto some or all of its powers except those which by law, by the Certificate or by these By-laws may not be delegated. Except as the Board of Directors may otherwise determine, any such committee may make rules for the conduct of its business, but unless otherwise provided by the Board of Directors or in such rules, its business shall be conducted so far as possible in the same manner as is provided by these By-laws for the Board of Directors. All members of such committees shall hold such offices at the pleasure of the Board of Directors. The Board of Directors may abolish any such committee at any time. Any committee to which the Board of Directors delegates any of its powers or duties shall keep records of its meetings and shall report its action to the Board of Directors.

SECTION 16. Compensation of Directors. Directors shall receive such compensation for their services as shall be determined by a majority of the Board of Directors, or a designated committee thereof, provided that directors who are serving the Corporation as employees and who receive compensation for their services as such, shall not receive any salary or other compensation for their services as directors of the Corporation.

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ARTICLE III

Officers

SECTION 1. Enumeration. The officers of the Corporation shall consist of a President, a Treasurer, a Secretary and such other officers, including, without limitation, a Chairman of the Board of Directors, a Chief Executive Officer and one or more Vice Presidents (including Executive Vice Presidents or Senior Vice Presidents), Assistant Vice Presidents, Assistant Treasurers and Assistant Secretaries, as the Board of Directors may determine.

SECTION 2. Election. At the regular annual meeting of the Board of Directors following the Annual Meeting, the Board of Directors shall elect the President, the Treasurer and the Secretary. Other officers may be elected by the Board of Directors at such regular annual meeting of the Board of Directors or at any other regular or special meeting.

SECTION 3. Qualification. No officer need be a stockholder or a director. Any person may occupy more than one office of the Corporation at any time.

SECTION 4. Tenure. Except as otherwise provided by the Certificate or by these By-laws, each of the officers of the Corporation shall hold office until the regular annual meeting of the Board of Directors following the next Annual Meeting and until his or her successor is elected and qualified or until his or her earlier resignation or removal.

SECTION 5. Resignation. Any officer may resign by delivering his or her written resignation to the Corporation addressed to the President or the Secretary, and such resignation shall be effective upon receipt, unless the resignation otherwise provides.

SECTION 6. Removal. Except as otherwise provided by law, the Board of Directors may remove any officer with or without cause by the affirmative vote of a majority of the directors then in office.

SECTION 7. Absence or Disability. In the event of the absence or disability of any officer, the Board of Directors may designate another officer to act temporarily in place of such absent or disabled officer.

SECTION 8. Vacancies. Any vacancy in any office may be filled for the unexpired portion of the term by the Board of Directors.

SECTION 9. President. The President shall, subject to the direction of the Board of Directors, have such powers and shall perform such duties as the Board of Directors may from time to time designate.

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SECTION 10. Chairman of the Board. The Chairman of the Board, if one is elected, shall have such powers and shall perform such duties as the Board of Directors may from time to time designate.

SECTION 11. Chief Executive Officer. The Chief Executive Officer, if one is elected, shall have such powers and shall perform such duties as the Board of Directors may from time to time designate.

SECTION 12. Vice Presidents and Assistant Vice Presidents. Any Vice President (including any Executive Vice President or Senior Vice President) and any Assistant Vice President shall have such powers and shall perform such duties as the Board of Directors or the Chief Executive Officer may from time to time designate.

SECTION 13. Treasurer and Assistant Treasurers. The Treasurer shall, subject to the direction of the Board of Directors and except as the Board of Directors or the Chief Executive Officer may otherwise provide, have general charge of the financial affairs of the Corporation and shall cause to be kept accurate books of account. The Treasurer shall have custody of all funds, securities, and valuable documents of the Corporation. He or she shall have such other duties and powers as may be designated from time to time by the Board of Directors or the Chief Executive Officer. Any Assistant Treasurer shall have such powers and perform such duties as the Board of Directors or the Chief Executive Officer may from time to time designate.

SECTION 14. Secretary and Assistant Secretaries. The Secretary shall record all the proceedings of the meetings of the stockholders and the Board of Directors (including committees of the Board of Directors) in books kept for that purpose. In his or her absence from any such meeting, a temporary secretary chosen at the meeting shall record the proceedings thereof. The Secretary shall have charge of the stock ledger (which may, however, be kept by any transfer or other agent of the Corporation). The Secretary shall have custody of the seal of the Corporation, and the Secretary, or an Assistant Secretary shall have authority to affix it to any instrument requiring it, and, when so affixed, the seal may be attested by his or her signature or that of an Assistant Secretary. The Secretary shall have such other duties and powers as may be designated from time to time by the Board of Directors or the Chief Executive Officer. In the absence of the Secretary, any Assistant Secretary may perform his or her duties and responsibilities. Any Assistant Secretary shall have such powers and perform such duties as the Board of Directors or the Chief Executive Officer may from time to time designate.

SECTION 15. Other Powers and Duties. Subject to these By-laws and to such limitations as the Board of Directors may from time to time prescribe, the officers of the Corporation shall each have such powers and duties as generally pertain to their respective offices, as well as such powers and duties as from time to time may be conferred by the Board of Directors or the Chief Executive Officer.

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ARTICLE IV

Capital Stock

SECTION 1. Certificates of Stock. Each stockholder shall be entitled to a certificate of the capital stock of the Corporation in such form as may from time to time be prescribed by the Board of Directors. Such certificate shall be signed by the Chairman of the Board, the President or a Vice President and by the Treasurer or an Assistant Treasurer, or the Secretary or an Assistant Secretary. The Corporation seal and the signatures by the Corporation's officers, the transfer agent or the registrar may be facsimiles. In case any officer, transfer agent or registrar who has signed or whose facsimile signature has been placed on such certificate shall have ceased to be such officer, transfer agent or registrar before such certificate is issued, it may be issued by the Corporation with the same effect as if he or she were such officer, transfer agent or registrar at the time of its issue. Every certificate for shares of stock which are subject to any restriction on transfer and every certificate issued when the Corporation is authorized to issue more than one class or series of stock shall contain such legend with respect thereto as is required by law. Notwithstanding anything to the contrary provided in these Bylaws, the Board of Directors of the Corporation may provide by resolution or resolutions that some or all of any or all classes or series of its stock shall be uncertificated shares (except that the foregoing shall not apply to shares represented by a certificate until such certificate is surrendered to the Corporation), and by the approval and adoption of these Bylaws the Board of Directors has determined that all classes or series of the Corporation's stock may be uncertificated, whether upon original issuance, re-issuance, or subsequent transfer.

SECTION 2. Transfers. Subject to any restrictions on transfer and unless otherwise provided by the Board of Directors, shares of stock that are represented by a certificate may be transferred on the books of the Corporation by the surrender to the Corporation or its transfer agent of the certificate theretofore properly endorsed or accompanied by a written assignment or power of attorney properly executed, with transfer stamps (if necessary) affixed, and with such proof of the authenticity of signature as the Corporation or its transfer agent may reasonably require. Shares of stock that are not represented by a certificate may be transferred on the books of the Corporation by submitting to the Corporation or its transfer agent such evidence of transfer and following such other procedures as the Corporation or its transfer agent may require.

SECTION 3. Record Holders. Except as may otherwise be required by law, by the Certificate or by these By-laws, the Corporation shall be entitled to treat the record holder of stock as shown on its books as the owner of such stock for all purposes, including the payment of dividends and the right to vote with respect thereto, regardless of any transfer, pledge or other disposition of such stock, until the shares have been transferred on the books of the Corporation in accordance with the requirements of these By-laws.

SECTION 4. Record Date. In order that the Corporation may determine the stockholders entitled to notice of or to vote at any meeting of stockholders or any adjournment thereof or entitled to receive payment of any dividend or other distribution or allotment of any rights, or entitled to exercise any rights in respect of any change, conversion or exchange of



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stock or for the purpose of any other lawful action, the Board of Directors may fix a record date, which record date shall not precede the date upon which the resolution fixing the record date is adopted by the Board of Directors, and which record date: (a) in the case of determination of stockholders entitled to vote at any meeting of stockholders, shall, unless otherwise required by law, not be more than sixty (60) nor less than ten (10) days before the date of such meeting and (b) in the case of any other action, shall not be more than sixty (60) days prior to such other action. If no record date is fixed: (i) the record date for determining stockholders entitled to notice of or to vote at a meeting of stockholders shall be at the close of business on the day next preceding the day on which notice is given, or, if notice is waived, at the close of business on the day next preceding the day on which the meeting is held; and (ii) the record date for determining stockholders for any other purpose shall be at the close of business on the day on which the Board of Directors adopts the resolution relating thereto.

SECTION 5. Replacement of Certificates. In case of the alleged loss, destruction or mutilation of a certificate of stock of the Corporation, a duplicate certificate may be issued in place thereof, upon such terms as the Board of Directors may prescribe.

## ARTICLE V

### Indemnification

SECTION 1. Definitions. For purposes of this Article:

(a) "Corporate Status" describes the status of a person who is serving or has served (i) as a Director of the Corporation, (ii) as an Officer of the Corporation, (iii) as a Non-Officer Employee of the Corporation, or (iv) as a director, partner, trustee, officer, employee or agent of any other corporation, partnership, limited liability company, joint venture, trust, employee benefit plan, foundation, association, organization or other legal entity which such person is or was serving at the request of the Corporation. For purposes of this Section 1(a), a Director, Officer or Non-Officer Employee of the Corporation who is serving or has served as a director, partner, trustee, officer, employee or agent of a Subsidiary shall be deemed to be serving at the request of the Corporation. Notwithstanding the foregoing, "Corporate Status" shall not include the status of a person who is serving or has served as a director, officer, employee or agent of a constituent corporation absorbed in a merger or consolidation transaction with the Corporation with respect to such person's activities prior to said transaction, unless specifically authorized by the Board of Directors or the stockholders of the Corporation;

(b) "Director" means any person who serves or has served the Corporation as a director on the Board of Directors of the Corporation;

(c) "Disinterested Director" means, with respect to each Proceeding in respect of which indemnification is sought hereunder, a Director of the Corporation who is not and was not a party to such Proceeding;

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(d) “Expenses” means all attorneys’ fees, retainers, court costs, transcript costs, fees of expert witnesses, private investigators and professional advisors (including, without limitation, accountants and investment bankers), travel expenses, duplicating costs, printing and binding costs, costs of preparation of demonstrative evidence and other courtroom presentation aids and devices, costs incurred in connection with document review, organization, imaging and computerization, telephone charges, postage, delivery service fees, and all other disbursements, costs or expenses of the type customarily incurred in connection with prosecuting, defending, preparing to prosecute or defend, investigating, being or preparing to be a witness in, settling or otherwise participating in, a Proceeding;

(e) “Liabilities” means judgments, damages, liabilities, losses, penalties, excise taxes, fines and amounts paid in settlement;

(f) “Non-Officer Employee” means any person who serves or has served as an employee or agent of the Corporation, but who is not or was not a Director or Officer;

(g) “Officer” means any person who serves or has served the Corporation as an officer of the Corporation appointed by the Board of Directors of the Corporation;

(h) “Proceeding” means any threatened, pending or completed action, suit, arbitration, alternate dispute resolution mechanism, inquiry, investigation, administrative hearing or other proceeding, whether civil, criminal, administrative, arbitrative or investigative; and

(i) “Subsidiary” shall mean any corporation, partnership, limited liability company, joint venture, trust or other entity of which the Corporation owns (either directly or through or together with another Subsidiary of the Corporation) either (i) a general partner, managing member or other similar interest or (ii) (A) fifty percent (50%) or more of the voting power of the voting capital equity interests of such corporation, partnership, limited liability company, joint venture or other entity, or (B) fifty percent (50%) or more of the outstanding voting capital stock or other voting equity interests of such corporation, partnership, limited liability company, joint venture or other entity.

#### SECTION 2. Indemnification of Directors and Officers.

(a) Subject to the operation of Section 4 of this Article V of these By-laws, each Director and Officer shall be indemnified and held harmless by the Corporation to the fullest extent authorized by the DGCL, as the same exists or may hereafter be amended (but, in the case of any such amendment, only to the extent that such amendment permits the Corporation to provide broader indemnification rights than such law permitted the Corporation to provide prior to such amendment), and to the extent authorized in this Section 2.

(1) Actions, Suits and Proceedings Other than By or In the Right of the Corporation . Each Director and Officer shall be indemnified and held harmless by the Corporation against any and all Expenses and Liabilities that are incurred or paid by such Director or Officer or on such Director’s or Officer’s behalf in connection with any Proceeding or any claim, issue or matter therein (other than an action by or in the right of the Corporation), which such Director or Officer is, or is threatened to be made, a party to or participant in by reason of such Director’s or Officer’s Corporate Status, if such Director or Officer acted in good faith and in a manner such Director or Officer reasonably believed to be in or not opposed to the best interests of the Corporation and, with respect to any criminal proceeding, had no reasonable cause to believe his or her conduct was unlawful.

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(2) Actions, Suits and Proceedings By or In the Right of the Corporation. Each Director and Officer shall be indemnified and held harmless by the Corporation against any and all Expenses that are incurred by such Director or Officer or on such Director's or Officer's behalf in connection with any Proceeding or any claim, issue or matter therein by or in the right of the Corporation, which such Director or Officer is, or is threatened to be made, a party to or participant in by reason of such Director's or Officer's Corporate Status, if such Director or Officer acted in good faith and in a manner such Director or Officer reasonably believed to be in or not opposed to the best interests of the Corporation; provided, however, that no indemnification shall be made under this Section 2(a)(2) in respect of any claim, issue or matter as to which such Director or Officer shall have been finally adjudged by a court of competent jurisdiction to be liable to the Corporation, unless, and only to the extent that, the Court of Chancery or another court in which such Proceeding was brought shall determine upon application that, despite adjudication of liability, but in view of all the circumstances of the case, such Director or Officer is fairly and reasonably entitled to indemnification for such Expenses that such court deems proper.

(3) Survival of Rights. The rights of indemnification provided by this Section 2 shall continue as to a Director or Officer after he or she has ceased to be a Director or Officer and shall inure to the benefit of his or her heirs, executors, administrators and personal representatives.

(4) Actions by Directors or Officers. Notwithstanding the foregoing, the Corporation shall indemnify any Director or Officer seeking indemnification in connection with a Proceeding initiated by such Director or Officer only if such Proceeding (including any parts of such Proceeding not initiated by such Director or Officer) was authorized in advance by the Board of Directors of the Corporation, unless such Proceeding was brought to enforce such Officer's or Director's rights to indemnification or, in the case of Directors, advancement of Expenses under these By-laws in accordance with the provisions set forth herein.

SECTION 3. Indemnification of Non-Officer Employees. Subject to the operation of Section 4 of this Article V of these By-laws, each Non-Officer Employee may, in the discretion of the Board of Directors of the Corporation, be indemnified by the Corporation to the fullest extent authorized by the DGCL, as the same exists or may hereafter be amended, against any or all Expenses and Liabilities that are incurred by such Non-Officer Employee or on such Non-Officer Employee's behalf in connection with any threatened, pending or completed Proceeding, or any claim, issue or matter therein, which such Non-Officer Employee is, or is threatened to be made, a party to or participant in by reason of such Non-Officer Employee's Corporate Status, if such Non-Officer Employee acted in good faith and in a manner such Non-Officer Employee reasonably believed to be in or not opposed to the best interests of the Corporation and, with respect to any criminal proceeding, had no reasonable cause to believe his or her conduct was unlawful. The rights of indemnification provided by this Section 3 shall exist as to a

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Non-Officer Employee after he or she has ceased to be a Non-Officer Employee and shall inure to the benefit of his or her heirs, personal representatives, executors and administrators. Notwithstanding the foregoing, the Corporation may indemnify any Non-Officer Employee seeking indemnification in connection with a Proceeding initiated by such Non-Officer Employee only if such Proceeding was authorized in advance by the Board of Directors of the Corporation.

SECTION 4. Determination. Unless otherwise ordered by a court, no indemnification shall be provided pursuant to this Article V to a Director, to an Officer or to a Non-Officer Employee unless a determination shall have been made that such person acted in good faith and in a manner such person reasonably believed to be in or not opposed to the best interests of the Corporation and, with respect to any criminal Proceeding, such person had no reasonable cause to believe his or her conduct was unlawful. Such determination shall be made by (a) a majority vote of the Disinterested Directors, even though less than a quorum of the Board of Directors, (b) a committee comprised of Disinterested Directors, such committee having been designated by a majority vote of the Disinterested Directors (even though less than a quorum), (c) if there are no such Disinterested Directors, or if a majority of Disinterested Directors so directs, by independent legal counsel in a written opinion, or (d) by the stockholders of the Corporation.

SECTION 5. Advancement of Expenses to Directors Prior to Final Disposition.

(a) The Corporation shall advance all Expenses incurred by or on behalf of any Director in connection with any Proceeding in which such Director is involved by reason of such Director's Corporate Status within thirty (30) days after the receipt by the Corporation of a written statement from such Director requesting such advance or advances from time to time, whether prior to or after final disposition of such Proceeding. Such statement or statements shall reasonably evidence the Expenses incurred by such Director and shall be preceded or accompanied by an undertaking by or on behalf of such Director to repay any Expenses so advanced if it shall ultimately be determined that such Director is not entitled to be indemnified against such Expenses. Notwithstanding the foregoing, the Corporation shall advance all Expenses incurred by or on behalf of any Director seeking advancement of expenses hereunder in connection with a Proceeding initiated by such Director only if such Proceeding (including any parts of such Proceeding not initiated by such Director) was (i) authorized by the Board of Directors of the Corporation, or (ii) brought to enforce such Director's rights to indemnification or advancement of Expenses under these By-laws.

(b) If a claim for advancement of Expenses hereunder by a Director is not paid in full by the Corporation within thirty (30) days after receipt by the Corporation of documentation of Expenses and the required undertaking, such Director may at any time thereafter bring suit against the Corporation to recover the unpaid amount of the claim and if successful in whole or in part, such Director shall also be entitled to be paid the expenses of prosecuting such claim. The failure of the Corporation (including its Board of Directors or any committee thereof, independent legal counsel, or stockholders) to make a determination concerning the permissibility of such advancement of Expenses under this Article V shall not be a defense to an action brought by a Director for recovery of the unpaid amount of an advancement claim and shall not create a presumption that such advancement is not permissible. The burden of proving that a Director is not entitled to an advancement of expenses shall be on the Corporation.

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(c) In any suit brought by the Corporation to recover an advancement of expenses pursuant to the terms of an undertaking, the Corporation shall be entitled to recover such expenses upon a final adjudication that the Director has not met any applicable standard for indemnification set forth in the DGCL.

SECTION 6. Advancement of Expenses to Officers and Non-Officer Employees Prior to Final Disposition.

(a) The Corporation may, at the discretion of the Board of Directors of the Corporation, advance any or all Expenses incurred by or on behalf of any Officer or any Non-Officer Employee in connection with any Proceeding in which such person is involved by reason of his or her Corporate Status as an Officer or Non-Officer Employee upon the receipt by the Corporation of a statement or statements from such Officer or Non-Officer Employee requesting such advance or advances from time to time, whether prior to or after final disposition of such Proceeding. Such statement or statements shall reasonably evidence the Expenses incurred by such Officer or Non-Officer Employee and shall be preceded or accompanied by an undertaking by or on behalf of such person to repay any Expenses so advanced if it shall ultimately be determined that such Officer or Non-Officer Employee is not entitled to be indemnified against such Expenses.

(b) In any suit brought by the Corporation to recover an advancement of expenses pursuant to the terms of an undertaking, the Corporation shall be entitled to recover such expenses upon a final adjudication that the Officer or Non-Officer Employee has not met any applicable standard for indemnification set forth in the DGCL.

SECTION 7. Contractual Nature of Rights.

(a) The provisions of this Article V shall be deemed to be a contract between the Corporation and each Director and Officer entitled to the benefits hereof at any time while this Article V is in effect, in consideration of such person's past or current and any future performance of services for the Corporation. Neither amendment, repeal or modification of any provision of this Article V nor the adoption of any provision of the Certificate of Incorporation inconsistent with this Article V shall eliminate or reduce any right conferred by this Article V in respect of any act or omission occurring, or any cause of action or claim that accrues or arises or any state of facts existing, at the time of or before such amendment, repeal, modification or adoption of an inconsistent provision (even in the case of a proceeding based on such a state of facts that is commenced after such time), and all rights to indemnification and advancement of Expenses granted herein or arising out of any act or omission shall vest at the time of the act or omission in question, regardless of when or if any proceeding with respect to such act or omission is commenced. The rights to indemnification and to advancement of expenses provided by, or granted pursuant to, this Article V shall continue notwithstanding that the person has ceased to be a director or officer of the Corporation and shall inure to the benefit of the estate, heirs, executors, administrators, legatees and distributees of such person.

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(b) If a claim for indemnification hereunder by a Director or Officer is not paid in full by the Corporation within sixty (60) days after receipt by the Corporation of a written claim for indemnification, such Director or Officer may at any time thereafter bring suit against the Corporation to recover the unpaid amount of the claim, and if successful in whole or in part, such Director or Officer shall also be entitled to be paid the expenses of prosecuting such claim. The failure of the Corporation (including its Board of Directors or any committee thereof, independent legal counsel, or stockholders) to make a determination concerning the permissibility of such indemnification under this Article V shall not be a defense to an action brought by a Director or Officer for recovery of the unpaid amount of an indemnification claim and shall not create a presumption that such indemnification is not permissible. The burden of proving that a Director or Officer is not entitled to indemnification shall be on the Corporation.

(c) In any suit brought by a Director or Officer to enforce a right to indemnification hereunder, it shall be a defense that such Director or Officer has not met any applicable standard for indemnification set forth in the DGCL.

SECTION 8. Non-Exclusivity of Rights. The rights to indemnification and to advancement of Expenses set forth in this Article V shall not be exclusive of any other right which any Director, Officer, or Non-Officer Employee may have or hereafter acquire under any statute, provision of the Certificate or these By-laws, agreement, vote of stockholders or Disinterested Directors or otherwise.

SECTION 9. Insurance. The Corporation may maintain insurance, at its expense, to protect itself and any Director, Officer or Non-Officer Employee against any liability of any character asserted against or incurred by the Corporation or any such Director, Officer or Non-Officer Employee, or arising out of any such person's Corporate Status, whether or not the Corporation would have the power to indemnify such person against such liability under the DGCL or the provisions of this Article V.

SECTION 10. Other Indemnification. The Corporation's obligation, if any, to indemnify or provide advancement of Expenses to any person under this Article V as a result of such person serving, at the request of the Corporation, as a director, partner, trustee, officer, employee or agent of another corporation, partnership, joint venture, trust, employee benefit plan or other enterprise shall be reduced by any amount such person may collect as indemnification or advancement of Expenses from such other corporation, partnership, joint venture, trust, employee benefit plan or enterprise (the "Primary Indemnitor"). Any indemnification or advancement of Expenses under this Article V owed by the Corporation as a result of a person serving, at the request of the Corporation, as a director, partner, trustee, officer, employee or agent of another corporation, partnership, joint venture, trust, employee benefit plan or other enterprise shall only be in excess of, and shall be secondary to, the indemnification or advancement of Expenses available from the applicable Primary Indemnitor(s) and any applicable insurance policies.

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ARTICLE VI

Miscellaneous Provisions

SECTION 1. Fiscal Year. The fiscal year of the Corporation shall be determined by the Board of Directors.

SECTION 2. Seal. The Board of Directors shall have power to adopt and alter the seal of the Corporation.

SECTION 3. Execution of Instruments. All deeds, leases, transfers, contracts, bonds, notes and other obligations to be entered into by the Corporation in the ordinary course of its business without director action may be executed on behalf of the Corporation by the Chairman of the Board, if one is elected, the President or the Treasurer or any other officer, employee or agent of the Corporation as the Board of Directors or the executive committee of the Board may authorize.

SECTION 4. Voting of Securities. Unless the Board of Directors otherwise provides, the Chairman of the Board, if one is elected, the President or the Treasurer may waive notice of and act on behalf of the Corporation, or appoint another person or persons to act as proxy or attorney in fact for the Corporation with or without discretionary power and/or power of substitution, at any meeting of stockholders or shareholders of any other corporation or organization, any of whose securities are held by the Corporation.

SECTION 5. Resident Agent. The Board of Directors may appoint a resident agent upon whom legal process may be served in any action or proceeding against the Corporation.

SECTION 6. Corporate Records. The original or attested copies of the Certificate, By-laws and records of all meetings of the incorporators, stockholders and the Board of Directors and the stock transfer books, which shall contain the names of all stockholders, their record addresses and the amount of stock held by each, may be kept outside the State of Delaware and shall be kept at the principal office of the Corporation, at an office of its counsel, at an office of its transfer agent or at such other place or places as may be designated from time to time by the Board of Directors.

SECTION 7. Certificate. All references in these By-laws to the Certificate shall be deemed to refer to the Amended and Restated Certificate of Incorporation of the Corporation, as amended and/or restated and in effect from time to time.

SECTION 8. Amendment of By-laws.

(a) Amendment by Directors. Except as provided otherwise by law, these By-laws may be amended or repealed by the Board of Directors by the affirmative vote of a majority of the directors then in office.

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(b) Amendment by Stockholders. These By-laws may be amended or repealed at any Annual Meeting, or special meeting of stockholders called for such purpose in accordance with these By-Laws, by the affirmative vote of at least seventy-five percent (75%) of the outstanding shares entitled to vote on such amendment or repeal, voting together as a single class; provided, however, that if the Board of Directors recommends that stockholders approve such amendment or repeal at such meeting of stockholders, such amendment or repeal shall only require the affirmative vote of the majority of the outstanding shares entitled to vote on such amendment or repeal, voting together as a single class. Notwithstanding the foregoing, stockholder approval shall not be required unless mandated by the Certificate, these By-laws, or other applicable law.

SECTION 9. Notices. If mailed, notice to stockholders shall be deemed given when deposited in the mail, postage prepaid, directed to the stockholder at such stockholder's address as it appears on the records of the Corporation. Without limiting the manner by which notice otherwise may be given to stockholders, any notice to stockholders may be given by electronic transmission in the manner provided in Section 232 of the DGCL.

SECTION 10. Waivers. A written waiver of any notice, signed by a stockholder or director, or waiver by electronic transmission by such person, whether given before or after the time of the event for which notice is to be given, shall be deemed equivalent to the notice required to be given to such person. Neither the business to be transacted at, nor the purpose of, any meeting need be specified in such a waiver.

**Adopted by the Board of Directors on May 5, 2013, subject to effectiveness of the Company's Registration Statement on Form S-1.**



## FORM OF COMMON STOCK WARRANT

THIS WARRANT AND THE SHARES OF COMMON STOCK ISSUED UPON ANY EXERCISE HEREOF HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED (THE "SECURITIES ACT"), OR ANY APPLICABLE STATE SECURITIES LAWS AND MAY NOT BE SOLD OR OTHERWISE TRANSFERRED BY ANY PERSON, INCLUDING A PLEDGEE, UNLESS (1) EITHER (A) A REGISTRATION WITH RESPECT THERETO SHALL BE EFFECTIVE UNDER THE SECURITIES ACT, OR (B) THE COMPANY SHALL HAVE RECEIVED AN OPINION OF COUNSEL REASONABLY SATISFACTORY TO THE COMPANY THAT AN EXEMPTION FROM REGISTRATION UNDER THE SECURITIES ACT IS AVAILABLE, AND (2) THERE SHALL HAVE BEEN COMPLIANCE WITH ALL APPLICABLE STATE SECURITIES OR "BLUE SKY" LAWS. THERE IS NO AND THERE IS NOT EXPECTED TO BE A PUBLIC MARKET FOR THE SHARES OF COMMON STOCK ISSUABLE UPON ANY EXERCISE HEREOF. INVESTORS SHOULD BE AWARE THAT THEY WILL BE REQUIRED TO BEAR THE FINANCIAL RISKS OF THIS INVESTMENT FOR AN INDEFINITE PERIOD OF TIME.

Void After [\_\_\_\_\_]

Right to Purchase [\_\_\_\_\_] Shares of  
Common Stock of bluebird bio, Inc.**BLUEBIRD BIO, INC.**

## Common Stock Purchase Warrant

bluebird bio, Inc., a Delaware corporation (the "Company"), hereby certifies that for value received, [\_\_\_\_\_] or assigns (the "Warrantholder"), is entitled to purchase, subject to the terms and conditions hereinafter set forth, an aggregate of [\_\_\_\_\_] shares of Common Stock, par value \$.01 per share of the Company (subject to adjustment as hereinafter provided) at a purchase price of \$.01 per share (subject to adjustment as hereinafter provided), payable as hereinafter provided.

1. Definitions. As used herein, the following terms shall have the following meanings, unless the context otherwise requires:

- (a) "Common Stock" shall mean the Company's Common Stock, \$.01 par value per share.
- (b) "Stated Purchase Price" shall mean the purchase price to be paid upon exercise of this Warrant in accordance with the terms hereof, which price initially shall be \$.01 per share of Common Stock. The Stated Purchase Price shall be subject to adjustment from time to time pursuant to the provisions of Section 6 hereof.
- (c) "Warrant Expiration Date" shall mean [\_\_\_\_\_] p.m., Eastern Time, on [\_\_\_\_\_]; provided that if such date shall be a holiday or a day on which banks are authorized to close in the Commonwealth of Massachusetts, then [\_\_\_\_\_] p.m., Eastern Time, on the next following day which in the Commonwealth of Massachusetts is not a holiday or a day on which banks are authorized to close.

2. Notice. In case at any time: (a) the Company shall pay any dividend or make any distribution (other than regular cash dividends from earnings or earned surplus paid at an established rate) to the holders of the Common Stock; (b) there shall be any capital reorganization or reclassification of the capital stock of the Company or consolidation or merger

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of the Company with or sale of all or substantially all of its assets to another corporation; or (c) there shall be a voluntary or involuntary dissolution, liquidation or winding-up of the Company; then, in any one or more of such cases, the Company shall give written notice, by first class mail, postage prepaid, addressed to the registered holder at the address of such registered holder as shown on the books of the Company of the date on which (i) the books of the Company shall close or a record date shall be fixed for determining the shareholders entitled to such dividend or distribution, or (ii) such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation, winding-up, conversion, redemption or other event shall take place, as the case may be. Such notice shall also provide reasonable details of the proposed transaction and specify the date as of which the holders of Common Stock of record shall participate in such dividend or distribution, or shall be entitled to exchange their Common Stock for securities or other property deliverable upon such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation, winding-up, conversion, redemption or other event, as the case may be. Such written notice shall be given at least fifteen days prior to the action in question and not less than fifteen days prior to the record date or the date on which the Company's transfer books are closed in respect thereto.

3. Exercise.

- (a) Manner of Exercise. This Warrant may be exercised at any time or from time to time, on any day which is not a Saturday, Sunday or holiday under the laws of the State of Delaware, for all or any part of the number of shares of Common Stock purchasable upon its exercise; provided, however, that this Warrant shall be void and all rights represented hereby shall cease unless exercised before the Warrant Expiration Date. In order to exercise this Warrant, in whole or in part, the holder hereof shall deliver to the Company at its principal executive offices, or at such other office as the Company may designate by notice in writing, (i) this Warrant, (ii) a written notice of such holder's election to exercise its Warrant substantially in the form of Exhibit A attached hereto, and (iii) the documents described in Section 10, and shall pay to the Company by check made payable to the order of the Company or wire transfer of funds to a bank account designated by the Company an amount equal to the aggregate purchase price for all shares of Common Stock as to which this Warrant is exercised. In lieu of such exercise of this Warrant, the holder may from time to time convert this Warrant, in whole or in part, into a number of shares of Common Stock determined by using the following formula:

$$X=(P)(Y)(A-B)/A$$

where

X = the number of shares of Common Stock to be issued to the holder for the portion of this Warrant being exercised;

P = the percentage of this Warrant being exercised;

Y = the total number of shares of Common Stock issuable upon exercise of this Warrant in full;

A = the Fair Market Value of one share of Common Stock as of the exercise date; and

B = the Stated Purchase Price as in effect on the exercise date.

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Any portion of this Warrant that is exercised shall be immediately canceled. For purposes of the foregoing, "Fair Market Value" shall be determined pursuant to Section 8 hereof.

- (b) Issuance of Common Stock. Upon receipt of the documents and payments described in Section 3(a), the Company shall, as promptly as practicable, and in any event within 30 days thereafter, execute or cause to be executed, and deliver to such holder a certificate or certificates representing the aggregate number of full shares of Common Stock (or such other stock or securities that may be issuable upon exercise of the Warrant) issuable upon such exercise, together with an amount in cash in lieu of any fraction of a share, as hereinafter provided. The stock certificate or certificates so delivered shall be in the denomination specified in said notice and shall be registered in the name of the holder hereof. This Warrant shall be deemed to have been exercised and a certificate or certificates for shares of Common Stock shall be deemed to have been issued, and the holder hereof or any other person so designated to be named therein shall be deemed to have become a holder of record of such shares for all purposes as of the date said notice, together with this Warrant and the documents and payments described in Section 3(a), are received by the Company as aforesaid. If this Warrant shall have been exercised in part, the Company shall, at the time of delivery of said certificate or certificates, deliver to the holder hereof a new Warrant evidencing the rights of such holder to purchase the unpurchased shares of Common Stock called for by this Warrant, which new Warrant shall in all other respects be identical with this Warrant.

4. Reservation of Shares; State Securities Laws. The Company covenants that it will at all times until the Warrant Expiration Date reserve and keep available out of its authorized and unissued Common Stock and its authorized and unissued Common Stock ("Common Stock"), solely for the purpose of issue upon exercise of this Warrant or conversion of Common Stock issued upon such exercise, such number of shares of Common Stock as shall then be issuable upon the exercise of this Warrant and such number of shares of Common Stock as shall then be issuable upon conversion of such Common Stock. If any securities to be reserved for the purpose of exercise of this Warrant require approvals or registrations under applicable state "blue sky" or federal securities laws, the Company will use its reasonable efforts to obtain such approvals or registrations as may be appropriate.

5. Loss or Mutilation. Upon receipt of evidence satisfactory to the Company of the loss, theft, destruction or mutilation of this Warrant (including a reasonably detailed affidavit with respect to the circumstances of any loss, theft or destruction of such Warrant), and, in the case of any such mutilation, upon surrender and cancellation of this Warrant, the Company at its expense will execute and deliver, in lieu hereof, a new Warrant of like tenor.

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6. Subdivision or Combination of Common Stock. If the Company at any time subdivides (by any stock split, stock dividend, recapitalization or otherwise) its outstanding shares of Common Stock into a greater number of shares, the Stated Purchase Price in effect immediately prior to such subdivision will be proportionately reduced and the number of shares issuable upon exercise of this Warrant will be proportionately increased, and if the Company at any time combines (by reverse stock split, recapitalization or otherwise) its outstanding shares of Common Stock into a smaller number of shares, the Stated Purchase Price in effect immediately prior to such combination will be proportionately increased and the number of shares issuable upon exercise of this Warrant will be proportionately decreased.

7. Consolidation, Merger, etc. If any consolidation or merger of the Company with another corporation that involves a transfer of more than 50% of the voting power of the Company, any transfer of shares representing more than 50% of the voting power of the Company are transferred to any person that is not, on the date hereof, a holder of stock of any class or preference of the Company or the sale of all or substantially all of the Company's assets to another entity (each an "Extraordinary Event") shall be effected, then, while this Warrant remains outstanding, the Company may terminate this Warrant as of the effective date of such Extraordinary Event, provided that written notice of such termination shall be given to the Warrantholder and the Warrantholder shall have the right to exercise this Warrant during the twenty (20) day period preceding the effective date of such Extraordinary Event.

8. Fractional Shares. If the number of shares of Common Stock purchasable upon the exercise of this Warrant is adjusted pursuant to Section 6 hereof, the Company shall nevertheless not be required to issue fractions of shares, upon exercise of this Warrant or otherwise, or to distribute certificates that evidence fractional shares. With respect to any fraction of a share called for upon any exercise hereof, such fraction shall neither be issued nor extinguished until the final exercise of this Warrant, in which event if a fraction is issuable, the Company shall pay to the holder hereof an amount in cash equal to such fraction multiplied by the current "Fair Market Value" of the security into which such fractional share is convertible, determined as follows:

- (a) If the security is listed on a National Securities Exchange or admitted to unlisted trading privileges on such exchange or quoted in the NASDAQ System, the current fair market value shall be the last reported sale price of the security on such exchange or market system on the last business day prior to the date of exercise of this Warrant or, if no such sale is made on such day, the average closing bid and asked price for such day on such exchange or market system; or
- (b) If the security is not listed or admitted to unlisted trading privileges, the current fair market value shall be the mean of the last reported bid and asked prices reported by the National Quotation Bureau, on the last business day prior to the date of the exercise of this Warrant; or
- (c) If the security is not so listed or admitted to unlisted trading privileges and bid and asked prices are not so reported, the current fair market value shall be an amount determined in good faith by the Board of Directors of the Company.

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9. Transfers by Warrantholder. If the Warrantholder proposes to Transfer any shares of Common Stock issued upon exercise of the Warrant (the “Warrant Shares”), then the Warrantholder shall comply with the terms and provisions of that certain Right of First Refusal and Co-Sale Agreement by and among the Company and certain of its stockholders, as may be amended from time to time.

10. Agreements. As a condition precedent to any exercise of this Warrant, the holder hereof understands and agrees that it may be required to execute certain documents and agreements (in form and substance substantially identical to those governing the sale of Common Stock) relating to the purchase and sale of such Common Stock as well as registration, co-sale and voting rights, if any, relating to such Common Stock. Upon the execution and delivery of such documents and agreements, the holder will become a party to, and bound by, such agreements, as so amended or restated, as to the shares of Common Stock acquired upon exercise of this Warrant.

Each holder of this Warrant, and each holder of shares of Common Stock acquired upon the exercise of this Warrant, by acceptance hereof and thereof, agrees to furnish to the Company such information concerning such holder as may be requested by the Company which is necessary in connection with any registration or qualification of shares of Common Stock purchasable hereunder.

11. Lock-Up. If, in connection with a registration statement filed by the Company pursuant to the 1933 Act, the Company or its underwriter so requests, the Warrantholder will agree not to sell any Warrant Shares for a period not to exceed (i) in the event of an initial public offering, 180 days following the effectiveness of such registration and (ii) in connection with any additional registration statement filed after the closing date of the initial public offering, 90 days following the effectiveness of such registration, unless such period is extended to the extent required by any FINRA rules, for an additional period of up to thirty-four (34) days if the Company issues or proposes to issue an earnings or other public release within fifteen (15) days of the expiration of such lockup period.

12. Warrant Holder Not Deemed Stockholder. The holder of this Warrant shall not, as such, be entitled to vote or to receive dividends or be deemed the holder of Common Stock that may at any time be issuable upon exercise of this Warrant for any purpose whatsoever, nor shall anything contained herein be construed to confer upon the holder of this Warrant, as such, any of the rights of a stockholder of the Company or any right to vote for the election of directors or upon any matter submitted to stockholders at any meeting thereof, or to receive dividends or subscription rights, until such holder shall have exercised this Warrant and been issued shares of Common Stock in accordance with the provisions hereof.

13. Rights of Action; Remedies. All rights of action with respect to this Warrant are vested in the holder of this Warrant, and the holder may enforce against the Company its right to exercise this Warrant for the purchase of shares of Common Stock in the manner provided in this Warrant. The Company stipulates that the remedies at law of the holder of this Warrant in the event of any default or threatened default by the Company in the performance of or compliance with any of the terms of this Warrant are not and will not be adequate, and that such terms may be specifically enforced by a decree for the specific performance of any agreement contained herein or by an injunction against a violation of any of the terms hereof or otherwise.

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14. Modification of Warrant. This Warrant shall not be modified, supplemented or altered in any respect except with the consent in writing of the holder hereof and the Company; and no change in the number or nature of the securities purchasable upon the exercise of this Warrant, or the Stated Purchase Price therefor, or the acceleration of the Warrant Expiration Date, shall be made without the consent in writing of the holder hereof, other than such changes as are specifically prescribed by this Warrant as originally executed.

15. Miscellaneous. This Warrant shall be governed by, and construed and enforced in accordance with, the laws of the Commonwealth of Massachusetts, without regard to its principles of conflicts of laws. The headings in this Warrant are for purposes of reference only, and shall not limit or otherwise affect any of the terms hereof. This Warrant is being executed as an instrument under seal. The invalidity or unenforceability of any provision hereof shall in no way affect the validity or enforceability of any other provision.

[Remainder of page intentionally left blank.]

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IN WITNESS WHEREOF, the Company has caused this Warrant to be duly executed as of \_\_\_\_\_, 2011.

BLUEBIRD BIO, INC.

By: \_\_\_\_\_

Nick Leschly  
President and Chief Executive Officer

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EXHIBIT A

EXERCISE FORM

(To be signed only on exercise of Warrant)

bluebird bio, Inc.  
840 Memorial Drive  
Cambridge, MA 02139

The undersigned hereby irrevocably elects to exercise the right to purchase represented by the within Warrant for, and to purchase thereunder, \_\_\_\_\_ shares of the stock provided for therein, and requests that certificates for such shares be issued in the name of:

\_\_\_\_\_  
(Please print name, address, and social security number)

\_\_\_\_\_  
and, if said number of shares shall not be all the shares purchasable thereunder, that a new Warrant for the balance remaining of the shares purchasable under the within Warrant be registered in the name of the undersigned holder of the within Warrant or his Assignee as below indicated and delivered to the address stated below.

NAME OF HOLDER OR ASSIGNEE: \_\_\_\_\_  
(Please print)

ADDRESS OF HOLDER  
OR ASSIGNEE: \_\_\_\_\_

SIGNATURE OF HOLDER: \_\_\_\_\_

DATED: \_\_\_\_\_

Note: The above signature must correspond with the name exactly as written upon the face of the within Warrant in every particular, without alteration or enlargement or any change whatever, unless the within Warrant has been assigned.



## FORM OF SERIES A-1 PREFERRED WARRANT

NEITHER THIS WARRANT NOR ANY SECURITIES THAT MAY BE ISSUED UPON EXERCISE HEREOF HAVE BEEN REGISTERED OR QUALIFIED UNDER THE SECURITIES ACT OF 1933, AS AMENDED, OR APPLICABLE STATE SECURITIES LAWS. THIS WARRANT AND SUCH SECURITIES MAY NOT BE TRANSFERRED OR OTHERWISE DISPOSED OF UNLESS THEY HAVE BEEN SO REGISTERED AND QUALIFIED OR AN OPINION OF COUNSEL SATISFACTORY TO THE COMPANY TO THE EFFECT THAT SUCH REGISTRATION AND QUALIFICATION IS NOT REQUIRED HAS BEEN DELIVERED TO THE COMPANY.

## STOCK PURCHASE WARRANT

No. [ ]

[ ]

Genetix Pharmaceuticals Inc., a Delaware corporation (the "Company"), hereby certifies that, subject to the terms and conditions set forth herein, [ ] (the "Holder"), is entitled to purchase the Warrant Share Number of shares of Warrant Stock (both as defined below) from the Company at any time or from time to time before the earlier of (i) [ ] p.m. ([ ] time) on [ ] or (ii) the effective time of an Extraordinary Event (as defined in the Notes (as defined below)), for the Exercise Price (as defined below), subject to adjustments as set forth in Sections 4 and 6.

This Stock Purchase Warrant (this "Warrant") is being issued in connection with the purchase by the Holder of a Convertible Term Note (the "Note") of the Company in the principal amount set forth on the signature page hereto. The Note is one of a series of similar Convertible Term Notes (the Note and such Convertible Term Notes shall collectively be referred to as the "Notes") being issued by the Company to certain investors in the aggregate principal amount of up to \$[ ] pursuant to the terms of a Convertible Note and Warrant Purchase Agreement dated as of [ ] (the "Purchase Agreement"), by and among the Company and certain Investors named therein (the "Investors") including the Holder, and this Warrant is one of a series of similar Stock Purchase Warrants (this Warrant and such Stock Purchase Warrants shall collectively be referred to as the "Warrants") being issued by the Company to such Investors in connection with the issuance of the Notes.

1. Determination of Warrant Stock, Warrant Share Number and Warrant Exercise Price.

(a) Warrant Stock. "Warrant Stock" shall mean Series A-1 Convertible Preferred Stock, \$.01 par value per share, of the Company (the "Series A-1 Preferred Stock").

(b) Warrant Share Number. This Warrant shall be exercisable for a number of shares of Warrant Stock (the "Warrant Share Number") equal to a fraction, the numerator of which is the product of the original principal amount of such Note (the "Principal Amount") times twenty-five hundredths (0.25) and the denominator of which is the Exercise Price described in Section 1(c) below, such Warrant Share Number to be rounded to the nearest whole number.

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(c) Exercise Price. The exercise price per share of Warrant Stock (the "Exercise Price") shall be (i) \$0.6619 per share if the Warrant Stock is Series A-1 Preferred Stock or (ii) the lowest price per share paid by any purchaser of Next Round Preferred Stock in the Qualified Financing if the Warrant Stock is Next Round Preferred Stock.

2. Exercise of Warrant.

(a) Mechanics of Exercise. This Warrant may be exercised by the Holder by surrender to the Company of this Warrant, with the attached form of subscription agreement duly executed by the Holder, accompanied by payment equal to the aggregate purchase price for the securities for which this Warrant is then being exercised according to Section 3 hereof.

(b) Warrant Agent. In the event that a bank or trust company is appointed as trustee for the Holder pursuant to Section 4(b) hereof, such bank or trust company will have all the powers and duties of a warrant agent appointed pursuant to Section 9 hereof and will accept, in its own name for the account of the Company or such successor entity as may be entitled thereto, all amounts otherwise payable to the Company or such successor, as the case may be, upon exercise of this Warrant.

(c) Expiration. This Warrant and the Holder's rights hereunder will expire at the earlier of (i) [ \_\_\_\_\_ ] P.M. ([ \_\_\_\_\_ ] time) on [ \_\_\_\_\_ ] or (ii) the effective time of an Extraordinary Event (the "Expiration Date"); provided, however, that if the Holder has not previously exercised the Warrant, this Warrant shall be automatically exercised by the net issuance method upon the effective time of an Extraordinary Event; and provided further that upon and after the Warrant Stock Conversion Effective Time (as defined in Section 6), the right to purchase shares of Warrant Stock granted herein shall terminate, and this Warrant shall represent the right to purchase shares of the Common Stock, \$0.01 par value per share, of the Company ("Common Stock") as provided in Section 6 hereof.

(d) Delivery of Certificates. As soon as is practicable after any exercise of this Warrant, the Company, at its own expense, will deliver to the Holder one or more certificates representing the securities to which the Holder is entitled in respect of such exercise, together, in the case of any partial exercise, with a new Warrant representing the unexercised portion hereof.

(e) Fractional Shares. In the event that any exercise of this Warrant would, but for the provisions of this Section 2(e), result in the issuance of any fractional share of capital stock, then in lieu of such fractional share the Holder will be entitled to cash equal to the fair market value of such fractional share, as determined in good faith by the Company's Board of Directors.

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3. Payment of Exercise Price. The Exercise Price may be paid at the Holder's election either:

(a) by cash, certified or official bank check payable to the order of the Company, or wire transfer to its account, or

(b) by the net issuance method as described below:

(i) Prior to the Warrant Stock Conversion Effective Time (as defined in Section 6 hereof), the Company shall issue Warrant Stock under the net issuance method in accordance with the following formula:

$$X = (Y)(A-B)/A$$

Where X = the number of shares of Warrant Stock to be issued to the Holder

Y = the number of shares of Warrant Stock requested to be exercised under this Warrant

A = the current fair market value of one (1) share of Warrant Stock

B = the Exercise Price

As used herein, the current fair market value of a share of Warrant Stock shall mean the price per share which the Company could obtain from a willing buyer (not a current employee or director) for shares of Warrant Stock, as determined in good faith by the Company's Board of Directors, unless the Company shall become subject to a merger, consolidation or other acquisition pursuant to which the holders of Warrant Stock receive securities and/or other property in exchange for their Warrant Stock, in which case the fair market value of Warrant Stock shall be deemed to be the value (determined in good faith by the Company's Board of Directors) of the securities and other property received by the holders of the Warrant Stock per share of Warrant Stock pursuant to such merger, consolidation or other acquisition.

(ii) Upon and after the Warrant Stock Conversion Effective Time, the Company shall issue Common Stock under the net issuance method in accordance with the following formula:

$$X = (Y)(A-B)/A$$

Where X = the number of shares of Common Stock to be issued to the Holder

Y = the number of shares of Common Stock requested to be exercised under this Warrant

A = the current fair market value of one (1) share of Common Stock

B = the Exercise Price

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As used herein, current fair market value of Common Stock shall mean with respect to each share of Common Stock:

(A) if the exercise is in connection with the Company's initial public offering of Common Stock, and if the Company's registration statement relating to such public offering, has been declared effective by the Securities and Exchange Commission, then the initial "Price to Public" specified in the final prospectus with respect to the offering;

(B) if this Warrant is exercised after, and not in connection with, the Company's initial public offering of Common Stock and

(1) if the Common Stock is traded on an internationally recognized securities exchange that publishes closing prices, the fair market value shall be deemed to be the average of the closing prices over a fourteen (14) day period ending three (3) days before the day the current fair market value of the Common Stock is being determined; or

(2) if the Common Stock is not listed on an internationally recognized securities exchange that publishes closing prices but is actively traded over-the-counter, the fair market value shall be deemed to be the average of the closing bid and asked prices reported by the National Quotation Bureau (or similar system) over the twenty-one (21) day period ending three (3) days before the day the current fair market value of the Common Stock is being determined;

(3) if at any time the Common Stock is not listed on an internationally recognized securities exchange that publishes closing prices or actively traded over-the-counter, the current fair market value of Common Stock shall be the price per share which the Company could obtain from a willing buyer (not a current employee or director) for shares of Common Stock sold by the Company, from authorized but unissued shares, as determined in good faith by its Board of Directors, unless the Company shall become subject to a merger, consolidation or other acquisition pursuant to which the holders of Common Stock receive securities and/or other property in exchange for their Common Stock, in which case the fair market value of Common Stock shall be deemed to be the value of the securities and other property received by the holders of the Common Stock per share of Common Stock pursuant to such merger, consolidation or other acquisition.

#### 4. Adjustment for Reorganizations, Etc.

##### (a) Certain Adjustments.

(i) If at any time or from time to time prior to the exercise of this Warrant but before the Warrant Stock Conversion Effective Time, the Company effects a "Warrant Stock Adjustment Event" (as hereafter defined), then in each such case, (A) the number of shares of Warrant Stock purchasable hereunder shall be adjusted to the number obtained by multiplying the number of shares of Warrant Stock purchasable

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hereunder immediately before such Warrant Stock Adjustment Event by a fraction, the numerator of which shall be the number of shares of Warrant Stock outstanding (excluding treasury stock) immediately after such Warrant Stock Adjustment Event and the denominator of which shall be the number of shares of Warrant Stock outstanding (excluding treasury stock) immediately before such Warrant Stock Adjustment Event, and (B) the Exercise Price shall be adjusted to the number obtained by multiplying the Exercise Price in effect immediately before such Warrant Stock Adjustment Event by a fraction, the numerator of which shall be the number of shares of Warrant Stock outstanding (excluding treasury stock) immediately before such Warrant Stock Adjustment Event and the denominator of which shall be the number of shares of Warrant Stock outstanding (excluding treasury stock) immediately after such Warrant Stock Adjustment Event, in each case subject to further adjustment thereafter as provided herein. The term “Warrant Stock Adjustment Event” shall mean (x) the issuance of additional shares of Warrant Stock as a dividend or other distribution on outstanding Warrant Stock, (y) the subdivision of outstanding shares of Warrant Stock into a greater number of shares of Warrant Stock, or (z) the combination of outstanding shares of Warrant Stock into a smaller number of shares of Warrant Stock, but shall not include the Recapitalization as defined in the Company’s Third Amended and Restated Certificate of Incorporation, adjustments with respect to which have already been made.

(ii) If at any time or from time to time prior to the exercise of this Warrant but after the Warrant Stock Conversion Effective Time, the Company effects a “Common Stock Adjustment Event” (as hereafter defined), then in each such case, (A) the number of shares of Common Stock purchasable hereunder shall be adjusted to the number obtained by multiplying the number of shares of Common Stock purchasable hereunder immediately before such Common Stock Adjustment Event by a fraction, the numerator of which shall be the number of shares of Common Stock outstanding (excluding treasury stock) immediately after such Common Stock Adjustment Event and the denominator of which shall be the number of shares of Common Stock outstanding (excluding treasury stock) immediately before such Common Stock Adjustment Event, and (B) the Exercise Price shall be adjusted to the number obtained by multiplying the Exercise Price in effect immediately before such Common Stock Adjustment Event by a fraction, the numerator of which shall be the number of shares of Common Stock outstanding (excluding treasury stock) immediately before such Common Stock Adjustment Event and the denominator of which shall be the number, of shares of Common Stock outstanding (excluding treasury stock) immediately after such Common Stock Adjustment Event, in each case subject to further adjustment thereafter as provided herein. The term “Common Stock Adjustment Event” shall mean (x) the issuance of additional shares of Common Stock as a dividend or other distribution on outstanding Common Stock, (y) the subdivision of outstanding shares of Common Stock into a greater number of shares of Common Stock, or (z) the combination of outstanding shares of Common Stock into a smaller number of shares of Common Stock.

(iii) Subject to earlier termination of this Warrant upon the effective time of an Extraordinary Event, at any time or from time to time prior to the exercise of this Warrant, the Company (A) effects a capital reorganization, reclassification or recapitalization, or (B) consolidates with or merges with or into any other person or

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entity, then in each such case, the Holder, upon exercise hereof at any time after or simultaneously with the consummation of such reorganization, reclassification, recapitalization, consolidation or merger, as the case may be, will receive, in lieu of the securities issuable upon such exercise before such consummation or effective date, the other securities, cash, and/or property to which the Holder would have been entitled upon such consummation or in connection with such dissolution, as the case may be, if the Holder had exercised this Warrant immediately prior thereto, all subject to further adjustment thereafter as provided herein.

(b) Appointment of Trustee for Warrant Holders Upon Dissolution. In the event of any dissolution of the Company, the Company, prior to such dissolution, will, at its expense, deliver or cause to be delivered the securities, property, and/or cash receivable by the Holder after the effective date of such dissolution pursuant to this Section 4 to a bank or trust company, as trustee for the Holder.

(c) Continuation of Terms. Subject to earlier termination of this Warrant upon a sale of the Company, upon any reorganization, consolidation or merger referred to in this Section 4, this Warrant will continue in full force and effect, and the terms hereof will be applicable to the securities, cash, and/or property receivable on the exercise of this Warrant after or simultaneously with the consummation of such reorganization, consolidation or merger and will be binding upon the issuer of any such stock or other securities.

5. No Dilution or Impairment. The Company will not, by amendment of its Certificate of Incorporation (as amended from time to time, the “Certificate of Incorporation”) or through any reorganization, transfer of assets, consolidation, merger, dissolution, issue or sale of securities, or any other action, avoid or seek to avoid the observance or performance of any of the terms of this Warrant, but will at all times in good faith assist in the carrying out of all such terms and in the taking of all such action as may be necessary or appropriate in order to protect the rights of the Holder against dilution. Without limiting the generality of the foregoing, the Company will seek to take all such action as may be necessary or appropriate in order that the Company may validly and legally issue fully paid and nonassessable shares of stock upon exercise of this Warrant from time to time.

6. Automatic Conversion of the Warrant Stock. If at any time the issued and outstanding shares of the Warrant Stock shall be automatically converted into shares of Common Stock under the terms of the Certificate of Incorporation, then upon and after the effective time of such automatic conversion of the Warrant Stock (the “Warrant Stock Conversion Effective Time”), the right to purchase Warrant Stock granted herein shall terminate, and this Warrant shall represent the right to purchase a number of shares of Common Stock calculated as follows:

$$X = (Y)(Z)$$

Where X = the number of shares of Common Stock purchasable under this Warrant upon and after the Warrant Stock Conversion Effective Time

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- Y = the number of shares of Warrant Stock purchasable under this Warrant immediately prior to the Warrant Stock Conversion Effective Time
- Z = the number of shares of Common Stock issuable upon conversion of each share of Warrant Stock immediately prior to the Warrant Stock Conversion Effective Time

and the Exercise Price per share of Common Stock shall be a price calculated as follows:

$$A = (B)(X)/Y$$

- Where
- A = the Exercise Price per share of Common Stock upon and after the Warrant Stock Conversion Effective Time
- B = the Exercise Price per share of Warrant Stock immediately prior to the Warrant Stock Conversion Effective Time
- X = the number of shares of Warrant Stock purchasable under this Warrant immediately prior to the Warrant Stock Conversion Effective Time
- Y = the number of shares of Common Stock purchasable under this Warrant upon and after the Warrant Preferred Stock Conversion Effective Time

Thereafter, the number of shares of Common Stock purchasable hereunder and the Exercise Price per share shall be subject to adjustment for the types of events described in Section 4 above that occur with respect to the Common Stock.

7. Notices of Record Date, Etc. In the event from time to time of any proposed or contemplated:

- (a) taking by the Company of a record of the holders of any class of securities for the purpose of determining the holders thereof who are entitled to receive any dividend or other distribution, or any right to subscribe for, purchase, or otherwise acquire any shares of stock of any class or any other securities or property, or to receive any other right;
- (b) capital reorganization of the Company, any reclassification or recapitalization of the capital stock of the Company, or any transfer of all or substantially all the assets of the Company to, or any consolidation or merger of the Company with or into, any other person or entity; or
- (c) voluntary or involuntary dissolution, liquidation, or winding-up of the Company;

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then, and in each such event the Company will mail or cause to be mailed to the Holder a notice specifying (i) the date on which any such record is to be taken for the purpose of such dividend, distribution, or right, and stating the amount and character of such dividend, distribution, or right, or (ii) the date on which any such reorganization, reclassification, recapitalization, transfer, consolidation, merger, dissolution, liquidation, or winding-up is anticipated to take place and the time, if any is to be fixed, as of which the holders of record of any class or series of the Company's capital stock or other securities will be entitled to exchange such stock or other securities for other securities, cash, and/or other property deliverable on such reorganization, reclassification, recapitalization, transfer, consolidation, merger, dissolution, liquidation, or winding-up. Such notice will be mailed at least twenty (20) days prior to the earliest date specified in such notice on which any such action or transaction is to be taken or consummated

8. Reservation of Securities Issuable on Exercise of Warrant. The Company at all times and from time to time will reserve and keep available, solely for issuance and delivery on the exercise of this Warrant, the quality and quantities of securities from time to time issuable upon exercise of this Warrant. If at any time the Company does not have sufficient authorized securities to comply with the foregoing sentence, the Company promptly will take all steps (including, without limitation, amending the Certificate of Incorporation) necessary to provide the quality and quantity of securities sufficient to effect the exercise in full of this Warrant.

9. Warrant Agent. The Company may, by written notice to the Holder, appoint an agent for the purpose of issuing securities upon exercise of this Warrant, exchanging or replacing this Warrant, or any of the foregoing, and thereafter any such issuance, exchange, or replacement as the case may be, will be made at such office by such agent.

10. Transfer and Exchange of Warrant.

(a) Transfer. Subject to evidence of compliance with the Securities Act of 1933, as amended, and applicable state securities laws, this Warrant may be transferred or succeeded to by any person; provided, however, that the Company is given written notice at the time of such transfer stating the name and address of the transferee.

(b) Exchange. Upon surrender of this Warrant for transfer or exchange, a new Warrant or new Warrants of the same tenor and exercisable for the same aggregate number of shares, of Warrant Stock as the Warrant so surrendered will be issued to, and registered in the name of, the transferee or transferees. The Company may treat the person in whose name this Warrant is registered as the Holder for all purposes.

11. Captions. The captions of sections or subsections of this Warrant are for reference only and will not affect the interpretation or construction of this Warrant.

12. Equitable Relief. The Company hereby acknowledges that any breach by it of its obligations under this Warrant would cause substantial and irreparable damage to the Holder and that money damages would be an inadequate remedy therefor, and accordingly, acknowledges and agrees that, in addition to any other rights and remedies to which the Holder may be entitled in respect of any breach of such obligations, the Holder will be entitled to an injunction, specific performance and/or other equitable relief to prevent the breach of such obligations.

13. No Waiver. No failure or other delay by the Holder exercising any right, power, or privilege hereunder will be or operate as a waiver thereof, nor will any single or partial exercise thereof preclude any other or further exercise thereof or the exercise of any other right, power, or privilege.



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14. Governing Law. This Warrant will be governed by and interpreted and construed in accordance, with the internal laws of The Commonwealth of Massachusetts (without reference to principles of conflicts or choice of law).

15. Amendment/Waiver. This Warrant and the other Warrants may be amended by the written agreement of the Company and holders of outstanding Warrants that when exercised could be exercised into at least two-thirds of the aggregate number of shares of Warrant Stock into which all then outstanding Warrants could then be exercised. Any provision of this Warrant and the other Warrants may be waived with the written agreement of the holders of outstanding Warrants that when exercised could be exercised into at least two-thirds of the aggregate number of shares of Warrant Stock into which all then outstanding Warrants could then be exercised. Notwithstanding the foregoing, no amendment of or waiver with respect to any provision of this Warrant or the other Warrants shall be effected unless the holders of all then outstanding Warrants are treated similarly as a class and not disproportionately.

*[Remainder of page intentionally left blank.]*

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Executed and delivered under seal on and as of the date first above written.

**Original Principal Amount of Note:**  
\$[\_\_\_\_\_]

**GENETIX PHARMACEUTICALS INC.**

By: \_\_\_\_\_  
Name:  
Its:

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**SUBSCRIPTION FORM**

The undersigned, the Holder of the within Stock Purchase Warrant, hereby elects to exercise the purchase right represented by such Warrant as follows:

The undersigned hereby elects to purchase shares of Warrant Stock (as defined in this Warrant) and herewith makes payment of \$ \_\_\_\_\_ therefor.

The undersigned hereby elects to exercise this Warrant by the net issuance method described in Section 3 of this Warrant and to exercise for that purpose \_\_\_\_\_ shares of Warrant Stock (as defined in this Warrant).

The undersigned further requests that the certificates representing such shares be issued in the name of and delivered to \_\_\_\_\_ and if such shares shall not include all of the shares issuable under this Warrant, that a new Warrant of like tenor and date be delivered to the undersigned for the shares not issued.

Dated: \_\_\_\_\_

\_\_\_\_\_  
Name of Holder

## FORM OF SERIES B PREFERRED WARRANT

NEITHER THIS WARRANT NOR ANY SECURITIES THAT MAY BE ISSUED UPON EXERCISE HEREOF HAVE BEEN REGISTERED OR QUALIFIED UNDER THE SECURITIES ACT OF 1933, AS AMENDED, OR APPLICABLE STATE SECURITIES LAWS. THIS WARRANT AND SUCH SECURITIES MAY NOT BE TRANSFERRED OR OTHERWISE DISPOSED OF UNLESS THEY HAVE BEEN SO REGISTERED AND QUALIFIED OR AN OPINION OF COUNSEL SATISFACTORY TO THE COMPANY TO THE EFFECT THAT SUCH REGISTRATION AND QUALIFICATION IS NOT REQUIRED HAS BEEN DELIVERED TO THE COMPANY.

## STOCK PURCHASE WARRANT

No. [ ]

[ ]

Genetix Pharmaceuticals Inc., a Delaware corporation (the "Company"), hereby certifies that, subject to the terms and conditions set forth herein, [ ] (the "Holder"), is entitled to purchase the Warrant Share Number of shares of Warrant Stock (both as defined below) from the Company at any time or from time to time before the earlier of (i) [ ] p.m. ([ ] time) on [ ] or (ii) the effective time of an Extraordinary Event (as defined in the Notes (as defined below)), for the Exercise Price (as defined below), subject to adjustments as set forth in Sections 4 and 6.

This Stock Purchase Warrant (this "Warrant") is being issued in connection with the purchase by the Holder of a Convertible Term Note (the "Note") of the Company in the principal amount set forth on the signature page hereto. The Note is one of a series of similar Convertible Term Notes (the Note and such Convertible Term Notes shall collectively be referred to as the "Notes") being issued by the Company to certain investors in the aggregate principal amount of up to \$[ ] pursuant to the terms of a Convertible Note and Warrant Purchase Agreement dated as of [ ] by and among the Company and certain Investors named therein (the "Investors") including the Holder, as amended (as so amended, the "Purchase Agreement"), and this Warrant is one of a series of similar Stock Purchase Warrants (this Warrant and such Stock Purchase Warrants shall collectively be referred to as the "Warrants") being issued by the Company to such Investors in connection with the issuance of the Notes.

1. Determination of Warrant Stock, Warrant Share Number and Warrant Exercise Price.

(a) Warrant Stock. "Warrant Stock" shall mean (i) Series B Convertible Preferred Stock, \$.01 par value per share, of the Company (the "Series B Preferred Stock").

(b) Warrant Share Number. This Warrant shall be exercisable for a number of shares of Warrant Stock (the "Warrant Share Number") equal to a fraction, the numerator of which is the product of the original principal amount of such Note (the "Principal Amount") times twenty-five hundredths (0.25) and the denominator of which is the Exercise Price described in Section 1(c) below, such Warrant Share Number to be rounded to the nearest whole number.

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(c) Exercise Price. The exercise price per share of Warrant Stock (the "Exercise Price") shall be (i) \$0.6619 per share if the Warrant Stock is Series A-1 Preferred Stock or (ii) the lowest price per share paid by any purchaser of Next Round Preferred Stock in the Qualified Financing if the Warrant Stock is Next Round Preferred Stock.

## 2. Exercise of Warrant.

(a) Mechanics of Exercise. This Warrant may be exercised by the Holder by surrender to the Company of this Warrant, with the attached form of subscription agreement duly executed by the Holder, accompanied by payment equal to the aggregate purchase price for the securities for which this Warrant is then being exercised according to Section 3 hereof.

(b) Warrant Agent. In the event that a bank or trust company is appointed as trustee for the Holder pursuant to Section 4(b) hereof, such bank or trust company will have all the powers and duties of a warrant agent appointed pursuant to Section 9 hereof and will accept, in its own name for the account of the Company or such successor entity as may be entitled thereto, all amounts otherwise payable to the Company or such successor, as the case may be, upon exercise of this Warrant.

(c) Expiration. This Warrant and the Holder's rights hereunder will expire at the earlier of (i) [\_\_\_\_\_] P.M. ([\_\_\_\_\_] time) on [\_\_\_\_\_] or (ii) the effective time of an Extraordinary Event (the "Expiration Date"); provided, however, that if the Holder has not previously exercised the Warrant, this Warrant shall be automatically exercised by the net issuance method upon the effective time of an Extraordinary Event; and provided further that upon and after the Warrant Stock Conversion Effective Time (as defined in Section 6), the right to purchase shares of Warrant Stock granted herein shall terminate, and this Warrant shall represent the right to purchase shares of the Common Stock, \$0.01 par value per share, of the Company ("Common Stock") as provided in Section 6 hereof.

(d) Delivery of Certificates. As soon as is practicable after any exercise of this Warrant, the Company, at its own expense, will deliver to the Holder one or more certificates representing the securities to which the Holder is entitled in respect of such exercise, together, in the case of any partial exercise, with a new Warrant representing the unexercised portion hereof.

(e) Fractional Shares. In the event that any exercise of this Warrant would, but for the provisions of this Section 2(e), result in the issuance of any fractional share of capital stock, then in lieu of such fractional share the Holder will be entitled to cash equal to the fair market value of such fractional share, as determined in good faith by the Company's Board of Directors.

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3. Payment of Exercise Price. The Exercise Price may be paid at the Holder's election either

(a) by cash, certified or official bank check payable to the order of the Company, or wire transfer to its account, or

(b) by the net issuance method as described below:

(i) Prior to the Warrant Stock Conversion Effective Time (as defined in Section 6 hereof), the Company shall issue Warrant Stock under the net issuance method in accordance with the following formula:

$$X = (Y)(A-B)/A$$

Where: X = the number of shares of Warrant Stock to be issued to the Holder

Y = the number of shares of Warrant Stock requested to be exercised under this Warrant

A = the current fair market value of one (1) share of Warrant Stock

B = the Exercise Price

As used herein, the current fair market value of a share of Warrant Stock shall mean the price per share which the Company could obtain from a willing buyer (not a current employee or director) for shares of Warrant Stock, as determined in good faith by the Company's Board of Directors, unless the Company shall become subject to a merger, consolidation or other acquisition pursuant to which the holders of Warrant Stock receive securities and/or other property in exchange for their Warrant Stock, in which case the fair market value of Warrant Stock shall be deemed to be the value (determined in good faith by the Company's Board of Directors) of the securities and other property received by the holders of the Warrant Stock per share of Warrant Stock pursuant to such merger, consolidation or other acquisition.

(ii) Upon and after the Warrant Stock Conversion Effective Time, the Company shall issue Common Stock under the net issuance method in accordance with the following formula:

$$X = (Y)(A-B)/A$$

Where: X = the number of shares of Common Stock to be issued to the Holder

Y = the number of shares of Common Stock requested to be exercised under this Warrant

A = the current fair market value of one (1) share of Common Stock

B = the Exercise Price

As used herein, current fair market value of Common Stock shall mean with respect to each share of Common Stock:

(A) if the exercise is in connection with the Company's initial public offering of Common Stock, and if the Company's registration statement relating to such public offering has been declared effective by the Securities and Exchange Commission, then the initial "Price to Public" specified in the final prospectus with respect to the offering;

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(B) if this Warrant is exercised after, and not in connection with, the Company's initial public offering of Common Stock and

(1) if the Common Stock is traded on an internationally recognized securities exchange that publishes closing prices, the fair market value shall be deemed to be the average of the closing prices over a fourteen (14) day period ending three (3) days before the day the current fair market value of the Common Stock is being determined; or

(2) if the Common Stock is not listed on an internationally recognized securities exchange that publishes closing prices but is actively traded over-the-counter, the fair market value shall be deemed to be the average of the closing bid and asked prices reported by the National Quotation Bureau (or similar system) over the twenty-one (21) day period ending three (3) days before the day the current fair market value of the Common Stock is being determined;

(3) if at any time the Common Stock is not listed on an internationally recognized securities exchange that publishes closing prices or actively traded over-the-counter, the current fair market value of Common Stock shall be the price per share which the Company could obtain from a willing buyer (not a current employee or director) for shares of Common Stock sold by the Company, from authorized but unissued shares, as determined in good faith by its Board of Directors, unless the Company shall become subject to a merger, consolidation or other acquisition pursuant to which the holders of Common Stock receive securities and/or other property in exchange for their Common Stock, in which case the fair market value of Common Stock shall be deemed to be the value of the securities and other property received by the holders of the Common Stock per share of Common Stock pursuant to such merger, consolidation or other acquisition.

4. Adjustment for Reorganizations, Etc.

(a) Certain Adjustments.

(i) If at any time or from time to time prior to the exercise of this Warrant but before the Warrant Stock Conversion Effective Time, the Company effects a "Warrant Stock Adjustment Event" (as hereafter defined), then in each such case, (A) the number of shares of Warrant Stock purchasable hereunder shall be adjusted to the number obtained by multiplying the number of shares of Warrant Stock purchasable hereunder immediately before such Warrant Stock Adjustment Event by a fraction, the numerator of which shall be the number of shares of Warrant Stock outstanding (excluding treasury stock) immediately after such Warrant Stock Adjustment Event and the denominator of which shall be the number of shares of Warrant Stock outstanding (excluding treasury stock) immediately before such Warrant Stock Adjustment Event, and (B) the Exercise Price shall be adjusted to the number obtained by multiplying the Exercise Price in effect immediately before such Warrant Stock Adjustment Event by a fraction, the numerator of which shall be the number of shares of

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Warrant Stock outstanding (excluding treasury stock) immediately before such Warrant Stock Adjustment Event and the denominator of which shall be the number of shares of Warrant Stock outstanding (excluding treasury stock) immediately after such Warrant Stock Adjustment Event, in each case subject to further adjustment thereafter as provided herein. The term “Warrant Stock Adjustment Event” shall mean (x) the issuance of additional shares of Warrant Stock as a dividend or other distribution on outstanding Warrant Stock, (y) the subdivision of outstanding shares of Warrant Stock into a greater number of shares of Warrant Stock, or (z) the combination of outstanding shares of Warrant Stock into a smaller number of shares of Warrant Stock, but shall not include the Recapitalization as defined in the Company’s Third Amended and Restated Certificate of Incorporation, adjustments with respect to which have already been made.

(ii) If at any time or from time to time prior to the exercise of this Warrant but after the Warrant Stock Conversion Effective Time, the Company effects a “Common Stock Adjustment Event” (as hereafter defined), then in each such case, (A) the number of shares of Common Stock purchasable hereunder shall be adjusted to the number obtained by multiplying the number of shares of Common Stock purchasable hereunder immediately before such Common Stock Adjustment Event by a fraction, the numerator of which shall be the number of shares of Common Stock outstanding (excluding treasury stock) immediately after such Common Stock Adjustment Event and the denominator of which shall be the number of shares of Common Stock outstanding (excluding treasury stock) immediately before such Common Stock Adjustment Event, and (B) the Exercise Price shall be adjusted to the number obtained by multiplying the Exercise Price in effect immediately before such Common Stock Adjustment Event by a fraction, the numerator of which shall be the number of shares of Common Stock outstanding (excluding treasury stock) immediately before such Common Stock Adjustment Event and the denominator of which shall be the number of shares of Common Stock outstanding (excluding treasury stock) immediately after such Common Stock Adjustment Event, in each case subject to further adjustment thereafter as provided herein. The term “Common Stock Adjustment Event” shall mean (x) the issuance of additional shares of Common Stock as a dividend or other distribution on outstanding Common Stock, (y) the subdivision of outstanding shares of Common Stock into a greater number of shares of Common Stock, or (z) the combination of outstanding shares of Common Stock into a smaller number of shares of Common Stock.

(iii) Subject to earlier termination of this Warrant upon the effective time of an Extraordinary Event, at any time or from time to time prior to the exercise of this Warrant, the Company (A) effects a capital reorganization, reclassification or recapitalization, or (B) consolidates with or merges with or into any other person or entity, then in each such case, the Holder, upon exercise hereof at any time after or simultaneously with the consummation of such reorganization, reclassification, recapitalization, consolidation or merger, as the case may be, will receive, in lieu of the securities issuable upon such exercise before such consummation or effective date, the other securities, cash, and/or property to which the Holder would have been entitled upon such consummation or in connection with such dissolution, as the case may be, if the Holder had exercised this Warrant immediately prior thereto, all subject to further adjustment thereafter as provided herein.



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(b) Appointment of Trustee for Warrant Holders Upon Dissolution. In the event of any dissolution of the Company, the Company, prior to such dissolution, will, at its expense, deliver or cause to be delivered the securities, property, and/or cash receivable by the Holder after the effective date of such dissolution pursuant to this Section 4 to a bank or trust company, as trustee for the Holder.

(c) Continuation of Terms. Subject to earlier termination of this Warrant upon a sale of the Company, upon any reorganization, consolidation or merger referred to in this Section 4, this Warrant will continue in full force and effect and the terms hereof will be applicable to the securities, cash, and/or property receivable on the exercise of this Warrant after or simultaneously with the consummation of such reorganization, consolidation or merger and will be binding upon the issuer of any such stock or other securities.

5. No Dilution or Impairment. The Company will not, by amendment of its Certificate of Incorporation (as amended from time to time, the “Certificate of Incorporation”) or through any reorganization, transfer of assets, consolidation, merger, dissolution, issue or sale of securities, or any other action, avoid or seek to avoid the observance or performance of any of the terms of this Warrant, but will at all times in good faith assist in the carrying out of all such terms and in the taking of all such action as may be necessary or appropriate in order to protect the rights of the Holder against dilution. Without limiting the generality of the foregoing, the Company will seek to take all such action as may be necessary or appropriate in order that the Company may validly and legally issue fully paid and nonassessable shares of stock upon exercise of this Warrant from time to time.

6. Automatic Conversion of the Warrant Stock. If at any time the issued and outstanding shares of the Warrant Stock shall be automatically converted into shares of Common Stock under the terms of the Certificate of Incorporation, then upon and after the effective time of such automatic conversion of the Warrant Stock (the “Warrant Stock Conversion Effective Time”), the right to purchase Warrant Stock granted herein shall terminate, and this Warrant shall represent the right to purchase a number of shares of Common Stock calculated as follows:

$$X = (Y)(Z)$$

Where: X = the number of shares of Common Stock purchasable under this Warrant upon and after the Warrant Stock Conversion Effective Time

Y = the number of shares of Warrant Stock purchasable under this Warrant immediately prior to the Warrant Stock Conversion Effective Time

Z = the number of shares of Common Stock issuable upon conversion of each share of Warrant Stock immediately prior to the Warrant Stock Conversion Effective Time

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and the Exercise Price per share of Common Stock shall be a price calculated as follows:

$$A = (B)(X)/Y$$

Where: A = the Exercise Price per share of Common Stock upon and after the Warrant Stock Conversion Effective Time

B = the Exercise Price per share of Warrant Stock immediately prior to the Warrant Stock Conversion Effective Time

X = the number of shares of Warrant Stock purchasable under this Warrant immediately prior to the Warrant Stock Conversion Effective Time

Y = the number of shares of Common Stock purchasable under this Warrant upon and after the Warrant Preferred Stock Conversion Effective Time

Thereafter, the number of shares of Common Stock purchasable hereunder and the Exercise Price per share shall be subject to adjustment for the types of events described in Section 4 above that occur with respect to the Common Stock.

7. Notices of Record Date, Etc. In the event from time to time of any proposed or contemplated:

(a) taking by the Company of a record of the holders of any class of securities for the purpose of determining the holders thereof who are entitled to receive any dividend or other distribution, or any right to subscribe for, purchase, or otherwise acquire any shares of stock of any class or any other securities or property, or to receive any other right;

(b) capital reorganization of the Company, any reclassification or recapitalization of the capital stock of the Company, or any transfer of all or substantially all the assets of the Company to, or any consolidation or merger of the Company with or into, any other person or entity; or

(c) voluntary or involuntary dissolution, liquidation, or winding-up of the Company;

then, and in each such event the Company will mail or cause to be mailed to the Holder a notice specifying (i) the date on which any such record is to be taken for the purpose of such dividend, distribution, or right, and stating the amount and character of such dividend, distribution, or right, or (ii) the date on which any such reorganization, reclassification, recapitalization, transfer, consolidation, merger, dissolution, liquidation, or winding-up is anticipated to take place and the time, if any is to be fixed, as of which the holders of record of any class or series of the Company's capital stock or other securities will be entitled to exchange such stock or other securities for other securities, cash, and/or other property deliverable on such reorganization, reclassification, recapitalization, transfer, consolidation, merger, dissolution, liquidation, or winding-up. Such notice will be mailed at least twenty (20) days prior to the earliest date specified in such notice on which any such action or transaction is to be taken or consummated.

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8. Reservation of Securities Issuable on Exercise of Warrant. The Company at all times and from time to time will reserve and keep available, solely for issuance and delivery on the exercise of this Warrant, the quality and quantities of securities from time to time issuable upon exercise of this Warrant. If at any time the Company does not have sufficient authorized securities to comply with the foregoing sentence, the Company promptly will take all steps (including, without limitation, amending the Certificate of Incorporation) necessary to provide the quality and quantity of securities sufficient to effect the exercise in full of this Warrant.

9. Warrant Agent. The Company may, by written notice to the Holder, appoint an agent for the purpose of issuing securities upon exercise of this Warrant, exchanging or replacing this Warrant, or any of the foregoing, and thereafter any such issuance, exchange, or replacement as the case may be, will be made at such office by such agent.

10. Transfer and Exchange of Warrant.

(a) Transfer. Subject to evidence of compliance with the Securities Act of 1933, as amended, and applicable state securities laws, this Warrant may be transferred or succeeded to by any person; provided, however, that the Company is given written notice at the time of such transfer stating the name and address of the transferee.

(b) Exchange. Upon surrender of this Warrant for transfer or exchange, a new Warrant or new Warrants of the same tenor and exercisable for the same aggregate number of shares of Warrant Stock as the Warrant so surrendered will be issued to, and registered in the name of, the transferee or transferees. The Company may treat the person in whose name this Warrant is registered as the Holder for all purposes.

11. Captions. The captions of sections or subsections of this Warrant are for reference only and will not affect the interpretation or construction of this Warrant.

12. Equitable Relief. The Company hereby acknowledges that any breach by it of its obligations under this Warrant would cause substantial and irreparable damage to the Holder and that money damages would be an inadequate remedy therefor, and accordingly, acknowledges and agrees that, in addition to any other rights and remedies to which the Holder may be entitled in respect of any breach of such obligations, the Holder will be entitled to an injunction, specific performance and/or other equitable relief to prevent the breach of such obligations.

13. No Waiver. No failure or other delay by the Holder exercising any right, power, or privilege hereunder will be or operate as a waiver thereof, nor will any single or partial exercise thereof preclude any other or further exercise thereof or the exercise of any other right, power, or privilege.

14. Governing Law. This Warrant will be governed by and interpreted and construed in accordance with the internal laws of The Commonwealth of Massachusetts (without reference to principles of conflicts or choice of law).

15. Amendment/Waiver. This Warrant and the other Warrants may be amended by the written agreement of the Company and holders of outstanding Warrants that when exercised could be exercised into at least two-thirds of the aggregate number of shares of Warrant Stock into which all then outstanding Warrants could then be exercised. Any provision of this Warrant and the other Warrants may be waived with the written agreement of the holders of outstanding

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Warrants that when exercised could be exercised into at least two-thirds of the aggregate number of shares of Warrant Stock into which all then outstanding Warrants could then be exercised. Notwithstanding the foregoing, no amendment of or waiver with respect to any provision of this Warrant or the other Warrants shall be effected unless the holders of all then outstanding Warrants are treated similarly as a class and not disproportionately.

**[Remainder of page intentionally left blank]**

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Executed and delivered under seal on and as of the date first above written.

**Original Principal Amount of Note:**

\$                    

**GENETIX PHARMACEUTICALS INC.**

By:

Name:

Its:

\_\_\_\_\_

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**SUBSCRIPTION FORM**

The undersigned, the Holder of the within Stock Purchase Warrant, hereby elects to exercise the purchase right represented by such Warrant as follows:

The undersigned hereby elects to purchase shares of Warrant Stock (as defined in this Warrant) and herewith makes payment of \$ \_\_\_\_\_ therefor.

The undersigned hereby elects to exercise this Warrant by the net issuance method described in Section 3 of this Warrant and to exercise for that purpose \_\_\_\_\_ shares of Warrant Stock (as defined in this Warrant).

The undersigned further requests that the certificates representing such shares be issued in the name of and delivered to \_\_\_\_\_ and if such shares shall not include all of the shares issuable under this Warrant, that a new Warrant of like tenor and date be delivered to the undersigned for the shares not issued.

Dated: \_\_\_\_\_

\_\_\_\_\_  
Name of Holder

**AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

THIS AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT is made as of the 23rd day of July, 2012, by and among bluebird bio, Inc., a Delaware corporation (the "**Company**"), each of the investors listed on Schedule A hereto, each of which is referred to in this Agreement as an "**Investor**", and any additional investor that becomes a party to this Agreement in accordance with Section 6.9 hereof.

**RECITALS**

**WHEREAS**, certain of the Investors are parties to an Investors' Rights Agreement dated as of April 15, 2011 by and among the Company and such Investors (the "**Prior Agreement**" and such Investors, the "**Existing Investors**");

**WHEREAS**, certain Investors (the "**Series D Investors**") are entering into that certain Series D Preferred Stock Purchase Agreement of even date herewith between the Company and the Series D Investors (the "**Series D Purchase Agreement**"), pursuant to which the Company is selling, and the Series D Investors are purchasing, shares of the Company's newly designated Series D Convertible Preferred Stock, par value \$0.01 per share (the "**Series D Preferred Stock**"); and

**WHEREAS**, Existing Investors that hold (i) a majority of the outstanding Series C Preferred Stock (as defined below), (ii) at least sixty percent (60%) of the outstanding Series B Preferred Stock (as defined below) and (iii) at least fifteen percent (15%) of the outstanding Series A Preferred Stock (as defined below), desire to amend and restate the Prior Agreement as provided herein and to provide the Series D Investors with certain registration rights, information rights, rights of first offer, and other rights in accordance with the terms of this Agreement.

**NOW, THEREFORE**, the Existing Investors hereby agree that the Prior Agreement shall be superseded and replaced in its entirety by this Agreement, and the parties to this Agreement further agree as follows:

1. **Definitions.** For purposes of this Agreement:

1.1 "**Affiliate**" means, with respect to any specified Person, any other Person who or which, directly or indirectly, controls, is controlled by, or is under common control with such specified Person, including without limitation any general partner, officer, director or manager of such Person and any venture capital fund now or hereafter existing that is controlled by or under common control with one or more general partners or managing members of, or shares the same management company with, such Person.

1.2 "**Common Stock**" means shares of the Company's common stock, par value \$0.01 per share.

1.3 "**Damages**" means any loss, damage, or liability (joint or several) to which a party hereto may become subject under the Securities Act, the Exchange Act, or other federal or state law, insofar as such loss, damage, or liability (or any action in respect thereof) arises out of or is based upon (i) any untrue statement or alleged untrue statement of a material

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fact contained in any registration statement of the Company, including any preliminary prospectus or final prospectus contained therein or any amendments or supplements thereto; (ii) an omission or alleged omission to state therein a material fact required to be stated therein, or necessary to make the statements therein not misleading; or (iii) any violation or alleged violation by the indemnifying party (or any of its agents or Affiliates) of the Securities Act, the Exchange Act, any state securities law, or any rule or regulation promulgated under the Securities Act, the Exchange Act, or any state securities law.

1.4 “**Derivative Securities**” means any securities or rights convertible into, or exercisable or exchangeable for (in each case, directly or indirectly), Common Stock, including options and warrants.

1.5 “**Exchange Act**” means the Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

1.6 “**Excluded Registration**” means (i) a registration relating to the sale of securities to employees of the Company or a subsidiary pursuant to a stock option, stock purchase, or similar plan; (ii) a registration relating to an SEC Rule 145 transaction; (iii) a registration on any form that does not include substantially the same information as would be required to be included in a registration statement covering the sale of the Registrable Securities; or (iv) a registration in which the only Common Stock being registered is Common Stock issuable upon conversion of debt securities that are also being registered.

1.7 “**Form S-1**” means such form under the Securities Act as in effect on the date hereof or any successor registration form under the Securities Act subsequently adopted by the SEC.

1.8 “**Form S-3**” means such form under the Securities Act as in effect on the date hereof or any registration form under the Securities Act subsequently adopted by the SEC that permits incorporation of substantial information by reference to other documents filed by the Company with the SEC.

1.9 “**GAAP**” means generally accepted accounting principles in the United States.

1.10 “**Holder**” means any holder of Registrable Securities, or holder of Derivative Securities convertible into, or exercisable or exchangeable for, Registrable Securities, who is a party to this Agreement. For purposes hereof, a holder of Derivative Securities convertible into, or exercisable or exchangeable for, Registrable Securities, shall be deemed to hold such Registrable Securities.

1.11 “**Immediate Family Member**” means a child, stepchild, grandchild, parent, stepparent, grandparent, spouse, sibling, mother-in-law, father-in-law, son-in-law, daughter-in-law, brother-in-law, or sister-in-law, including adoptive relationships, of a natural person referred to herein.

1.12 “**Initiating Holders**” means, collectively, Holders who properly initiate a registration request under this Agreement.



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1.13 “**IPO**” means the Company’s first underwritten public offering of its Common Stock under the Securities Act.

1.14 “**Key Employee**” means any executive-level employee (including division director and vice president-level positions).

1.15 “**New Securities**” means, collectively, equity or debt securities of the Company, whether or not currently authorized, as well as rights, options, or warrants to purchase such equity or debt securities, or securities of any type whatsoever that are, or may become, convertible or exchangeable into or exercisable for such equity or debt securities.

1.16 “**Person**” means any individual, corporation, partnership, trust, limited liability company, association or other entity.

1.17 “**Preferred Stock**” means, collectively, shares of the Company’s Series A Preferred Stock, Series B Preferred Stock, Series C Preferred Stock and Series D Preferred Stock.

1.18 “**Qualified Public Offering**” shall have the meaning ascribed to such term in the Company’s Certificate of Incorporation, as amended from time to time.

1.19 “**Preferred Directors**” means the director(s) of the Company that the holders of record of the Series A Preferred Stock, Series B Preferred Stock and Series C Preferred Stock, respectively, are entitled to elect pursuant to the Company’s Certificate of Incorporation.

1.20 “**Registrable Securities**” means (i) the Common Stock issuable or issued upon conversion of the Preferred Stock and (ii) any Common Stock issued as (or issuable upon the conversion or exercise of any warrant, right, or other security that is issued as) a dividend or other distribution with respect to, or in exchange for or in replacement of, the shares referenced in clause (i) above; excluding in all cases, however, any Registrable Securities sold by a Person in a transaction in which the applicable rights under this Agreement are not assigned pursuant to Section 6.1, and excluding for purposes of Section 2 any shares for which registration rights have terminated pursuant to Section 2.13 of this Agreement.

1.21 “**Registrable Securities then outstanding**” means the number of shares at a point in time determined by adding the number of shares of outstanding Common Stock that are Registrable Securities at such time and the number of shares of Common Stock issuable (directly or indirectly) at such time pursuant to outstanding Derivative Securities.

1.22 “**Requisite Holders**” means the holders of (i) a majority of the then outstanding shares of Preferred Stock, voting together as a single class and on an as-converted basis.

1.23 “**Requisite Preferred Directors**” means a majority of the Preferred Directors.

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1.24 “**Restricted Securities**” means the securities of the Company required to bear the legend set forth in Section 2.12(b) hereof.

1.25 “**SEC**” means the Securities and Exchange Commission.

1.26 “**SEC Rule 144**” means Rule 144 promulgated by the SEC under the Securities Act, or any successor provisions.

1.27 “**SEC Rule 145**” means Rule 145 promulgated by the SEC under the Securities Act, or any successor provisions.

1.28 “**Securities Act**” means the Securities Act of 1933, as amended, and the rules and regulations promulgated thereunder.

1.29 “**Selling Expenses**” means all underwriting discounts, selling commissions, and stock transfer taxes applicable to the sale of Registrable Securities, and fees and disbursements of counsel for any Holder, except for the fees and disbursements of the Selling Holder Counsel borne and paid by the Company as provided in Section 2.6.

1.30 “**Series A Preferred Stock**” means shares of the Company’s Series A-1 Convertible Preferred Stock and Series A-2 Convertible Preferred Stock, par value \$0.01 per share.

1.31 “**Series A Registrable Securities**” means the Common Stock issuable or issued upon conversion of the Series A Preferred Stock.

1.32 “**Series B Preferred Stock**” means shares of the Company’s Series B Convertible Preferred Stock, par value \$0.01 per share.

1.33 “**Series B Registrable Securities**” means the Common Stock issuable or issued upon conversion of the Series B Preferred Stock.

1.34 “**Series C Preferred Stock**” means shares of the Company’s Series C Convertible Preferred Stock, par value \$0.01 per share.

1.35 “**Series C Registrable Securities**” means the Common Stock issuable or issued upon conversion of the Series C Preferred Stock.

1.36 “**Series D Registrable Securities**” means the Common Stock issuable or issued upon conversion of the Series D Preferred Stock.

2. Registration Rights. The Company covenants and agrees as follows:

2.1 Demand Registration.

(a) Form S-1 Demand. Beginning upon the earlier of (i) five (5) years after the date of this Agreement or (ii) one hundred eighty (180) days after the effective date of the registration statement for the IPO, the Company receives a request from Holders of at least a majority of the Registrable Securities then outstanding, voting together as a single class and on

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an as-converted basis (the “**Demand Holders**”) that the Company file a Form S-1 registration statement with respect to the Registrable Securities then outstanding, which Registrable Securities represent an anticipated aggregate offering price of at least \$5 million, then the Company shall (i) within ten (10) days after the date such request is given, give notice thereof (the “**Demand Notice**”) to all Holders other than the Initiating Holders; and (ii) as soon as practicable, and in any event within sixty (60) days after the date such request is given by the Initiating Holders, file a Form S-1 registration statement under the Securities Act covering all Registrable Securities that the Initiating Holders requested to be registered and any additional Registrable Securities requested to be included in such registration by any other Holders, as specified by notice given by each such Holder to the Company within twenty (20) days of the date the Demand Notice is given, and in each case, subject to the limitations of Section 2.1(c) and Section 2.3.

(b) Form S-3 Demand. If at any time when it is eligible to use a Form S-3 registration statement and the Company receives a request from any Holder or Holders that the Company file a Form S-3 registration statement with respect to outstanding Registrable Securities of such Holders having an anticipated aggregate offering price of at least \$1 million, then the Company shall (i) within ten (10) days after the date such request is given, give a Demand Notice to all Holders other than the Initiating Holders; and (ii) as soon as practicable, and in any event within forty-five (45) days after the date such request is given by the Initiating Holders, file a Form S-3 registration statement under the Securities Act covering all Registrable Securities requested to be included in such registration by any other Holders, as specified by notice given by each such Holder to the Company within twenty (20) days of the date the Demand Notice is given, and in each case, subject to the limitations of Section 2.1(c) and Section 2.3.

(c) Notwithstanding the foregoing obligations, if the Company furnishes to Holders requesting a registration pursuant to this Section 2.1 a certificate signed by the Company’s chief executive officer stating that in the good faith judgment of the Company’s Board of Directors it would be materially detrimental to the Company and its stockholders for such registration statement to either become effective or remain effective for as long as such registration statement otherwise would be required to remain effective, because such action would (i) materially interfere with a significant acquisition, corporate reorganization, or other similar transaction involving the Company; (ii) require premature disclosure of material information that the Company has a bona fide business purpose for preserving as confidential; or (iii) render the Company unable to comply with requirements under the Securities Act or Exchange Act, then the Company shall have the right to defer taking action with respect to such filing, and any time periods with respect to filing or effectiveness thereof shall be tolled correspondingly, for a period of not more than ninety (90) days after the request of the Initiating Holders is given; provided, however, that the Company may not invoke this right more than once in any twelve (12) month period; and provided further that the Company shall not register any securities for its own account or that of any other stockholder during such ninety (90) day period other than an Excluded Registration.

(d) The Company shall not be obligated to effect, or to take any action to effect, any registration pursuant to Section 2.1(a) (i) during the period that is sixty (60) days before the Company’s good faith estimate of the date of filing of, and ending on a date that is one

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hundred eighty (180) days after the effective date of, a Company-initiated registration, provided, that the Company is actively employing its good faith commercially reasonable efforts to cause such registration statement to become effective; (ii) after the Company has effected two (2) registrations pursuant to Section 2.1(a), provided that a registration pursuant to Section 2.1(a) shall only be considered “effected” for purposes of this Section 2.1(d)(ii) if the registration statement registers all the Registrable Securities requested to be registered; or (iii) if the requesting Holders propose to dispose of shares of Registrable Securities that may be immediately registered on Form S-3 pursuant to a request made pursuant to Section 2.1(b). The Company shall not be obligated to effect, or to take any action to effect, any registration pursuant to Section 2.1(b), (i) during the period that is thirty (30) days before the Company’s good faith estimate of the date of filing of, and ending on a date that is ninety (90) days after the effective date of, a Company-initiated registration relating to shares to be sold by the Company, provided, that the Company is actively employing its good faith commercially reasonable efforts to cause such registration statement to become effective; or (ii) if the Company has effected two registration statements pursuant to Section 2.1(b) within the twelve (12) month period immediately preceding the date of such request. A registration shall not be counted as “effected” for purposes of this Section 2.1(d) until such time as the applicable registration statement has been declared effective by the SEC, unless the Initiating Holders withdraw their request for such registration (other than as a result of a material adverse change to the Company), elect not to pay the registration expenses therefor, and forfeit their right to one demand registration statement pursuant to Section 2.6, in which case such withdrawn registration statement shall be counted as “effected” for purposes of this Section 2.1(d).

2.2 Company Registration. If the Company proposes to register (including, for this purpose, a registration effected by the Company for stockholders other than the Holders) any of its Common Stock under the Securities Act in connection with the public offering of such securities solely for cash (other than in an Excluded Registration), the Company shall, at such time, promptly give each Holder notice of such registration. Upon the request of each Holder given within twenty (20) days after such notice is given by the Company, the Company shall, subject to the provisions of Section 2.3, cause to be registered all of the Registrable Securities that each such Holder has requested to be included in such registration. The Company shall have the right to terminate or withdraw any registration initiated by it under this Section 2.2 before the effective date of such registration, whether or not any Holder has elected to include Registrable Securities in such registration. The expenses (other than Selling Expenses) of such withdrawn registration shall be borne by the Company in accordance with Section 2.6.

### 2.3 Underwriting Requirements.

(a) If, pursuant to Section 2.1, the Initiating Holders intend to distribute the Registrable Securities covered by their request by means of an underwriting, they shall so advise the Company as a part of their request made pursuant to Section 2.1, and the Company shall include such information in the Demand Notice. The underwriter(s) will be selected by the Company and shall be reasonably acceptable to a majority in interest of the Initiating Holders. In such event, the right of any Holder to include such Holder’s Registrable Securities in such registration shall be conditioned upon such Holder’s participation in such underwriting and the inclusion of such Holder’s Registrable Securities in the underwriting to the extent provided herein. All Holders proposing to distribute their securities through such

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underwriting shall (together with the Company as provided in Section 2.4(e)) enter into an underwriting agreement in customary form with the underwriter(s) selected for such underwriting. Notwithstanding any other provision of this Section 2.3, if the managing underwriter(s) advise(s) the Initiating Holders in writing that marketing factors require a limitation on the number of shares to be underwritten, then the Initiating Holders shall so advise all Holders of Registrable Securities that otherwise would be underwritten pursuant hereto, and the number of Registrable Securities that may be included in the underwriting shall be allocated among such Holders of Registrable Securities, including the Initiating Holders, in proportion (as nearly as practicable) to the number of Registrable Securities owned by each Holder or in such other proportion as shall mutually be agreed to by all such selling Holders; provided, however, that the number of Registrable Securities held by the Holders to be included in such underwriting shall not be reduced unless all other securities are first entirely excluded from the underwriting. To facilitate the allocation of shares in accordance with the above provisions, the Company or the underwriters may round the number of shares allocated to any Holder to the nearest 100 shares.

(b) In connection with any offering involving an underwriting of shares of the Company's capital stock pursuant to Section 2.2, the Company shall not be required to include any of the Holders' Registrable Securities in such underwriting unless the Holders accept the terms of the underwriting as agreed upon between the Company and its underwriters, and then only in such quantity as the underwriters in their sole discretion determine will not jeopardize the success of the offering by the Company. If the total number of securities, including Registrable Securities, requested by stockholders to be included in such offering exceeds the number of securities to be sold (other than by the Company) that the underwriters in their reasonable discretion determine is compatible with the success of the offering, then the Company shall be required to include in the offering only that number of such securities, including Registrable Securities, which the underwriters and the Company in their sole discretion determine will not jeopardize the success of the offering. If the underwriters determine that less than all of the Registrable Securities requested to be registered can be included in such offering, then the Registrable Securities that are included in such offering shall be allocated among the selling Holders in proportion (as nearly as practicable to) the number of Registrable Securities owned by each selling Holder or in such other proportions as shall mutually be agreed to by all such selling Holders. To facilitate the allocation of shares in accordance with the above provisions, the Company or the underwriters may round the number of shares allocated to any Holder to the nearest 100 shares. Notwithstanding the foregoing, in no event shall (i) the number of Registrable Securities included in the offering be reduced unless all other securities (other than securities to be sold by the Company) are first entirely excluded from the offering (in which case the number of Series B Registrable Securities, Series C Registrable Securities and Series D Registrable Securities included in the offering (A) shall not be reduced unless all Series A Registrable Securities are first excluded from the offering and (B) shall be reduced on a *pari passu* basis), or (ii) the number of Registrable Securities included in the offering be reduced below thirty percent (30%) of the total number of securities included in such offering, unless such offering is the IPO, in which case the selling Holders may be excluded further if the underwriters make the determination described above and no other stockholder's securities are included in such offering. For purposes of the provision in this Section 2.3(b) concerning apportionment, for any selling Holder that is a partnership, limited liability company, or corporation, the partners, members, retired partners, retired members, stockholders, and

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Affiliates of such Holder, or the estates and Immediate Family Members of any such partners, retired partners, members, and retired members and any trusts for the benefit of any of the foregoing Persons, shall be deemed to be a single “selling Holder,” and any pro rata reduction with respect to such “selling Holder” shall be based upon the aggregate number of Registrable Securities owned by all Persons included in such “selling Holder,” as defined in this sentence.

(c) For purposes of Section 2.1, a registration shall not be counted as “effected” if, as a result of an exercise of the underwriter’s cutback provisions in Section 2.3(a), fewer than all of the Registrable Securities that Holders have requested to be included in such registration statement are actually included.

**2.4 Obligations of the Company.** Whenever required under this Section 2 to effect the registration of any Registrable Securities, the Company shall, as expeditiously as reasonably possible:

(a) prepare and file with the SEC a registration statement with respect to such Registrable Securities and use its commercially reasonable efforts to cause such registration statement to become effective and, upon the request of the Holders of a majority of the Registrable Securities registered thereunder, keep such registration statement effective for a period of up to one hundred twenty (120) days or, if earlier, until the distribution contemplated in the registration statement has been completed; provided, however, that such one hundred twenty (120) day period shall be extended for a period of time equal to the period the Holder refrains, at the request of an underwriter of Common Stock (or other securities) of the Company, from selling any securities included in such registration;

(b) prepare and file with the SEC such amendments and supplements to such registration statement, and the prospectus used in connection with such registration statement, as may be necessary to comply with the Securities Act in order to enable the disposition of all securities covered by such registration statement;

(c) furnish to the selling Holders such numbers of copies of a prospectus, including a preliminary prospectus, as required by the Securities Act, and such other documents as the Holders may reasonably request in order to facilitate their disposition of their Registrable Securities;

(d) use its commercially reasonable efforts to register and qualify the securities covered by such registration statement under such other securities or blue-sky laws of such jurisdictions as shall be reasonably requested by the selling Holders; provided that the Company shall not be required to qualify to do business or to file a general consent to service of process in any such states or jurisdictions, unless the Company is already subject to service in such jurisdiction and except as may be required by the Securities Act;

(e) in the event of any underwritten public offering, enter into and perform its obligations under an underwriting agreement, in usual and customary form, with the underwriter(s) of such offering;

(f) use its commercially reasonable efforts to cause all such Registrable Securities covered by such registration statement to be listed on a national securities exchange or trading system and each securities exchange and trading system (if any) on which similar securities issued by the Company are then listed;

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(g) provide a transfer agent and registrar for all Registrable Securities registered pursuant to this Agreement and provide a CUSIP number for all such Registrable Securities, in each case not later than the effective date of such registration;

(h) promptly make available for inspection by the selling Holders, any managing underwriter(s) participating in any disposition pursuant to such registration statement, and any attorney or accountant or other agent retained by any such underwriter or selected by the selling Holders, all financial and other records, pertinent corporate documents, and properties of the Company, and cause the Company's officers, directors, employees, and independent accountants to supply all information reasonably requested by any such seller, underwriter, attorney, accountant, or agent, in each case, as necessary or advisable to verify the accuracy of the information in such registration statement and to conduct appropriate due diligence in connection therewith;

(i) notify each selling Holder, promptly after the Company receives notice thereof, of the time when such registration statement has been declared effective or a supplement to any prospectus forming a part of such registration statement has been filed and in each case supply each selling Holder with a copy thereof; and

(j) after such registration statement becomes effective, notify each selling Holder of any request by the SEC that the Company amend or supplement such registration statement or prospectus.

2.5 Furnish Information. It shall be a condition precedent to the obligations of the Company to take any action pursuant to this Section 2 with respect to the Registrable Securities of any selling Holder that such Holder shall furnish to the Company such information regarding itself, the Registrable Securities held by it, and the intended method of disposition of such securities as is reasonably required to effect the registration of such Holder's Registrable Securities.

2.6 Expenses of Registration. All expenses (other than Selling Expenses) incurred in connection with registrations, filings, or qualifications pursuant to Section 2, including all registration, filing, and qualification fees; printers' and accounting fees; fees and disbursements of counsel for the Company; and the reasonable fees and disbursements of one counsel for the selling Holders (" **Selling Holder Counsel**") selected by the Demand Holders, shall be borne and paid by the Company; provided, however, that the Company shall not be required to pay for any expenses of any registration proceeding begun pursuant to Section 2.1 if the registration request is subsequently withdrawn at the request of the Holders of a majority of the Registrable Securities to be registered (in which case all selling Holders shall bear such expenses pro rata based upon the number of Registrable Securities that were to be included in the withdrawn registration), unless the Holders of a majority of the Registrable Securities agree to forfeit their right to one registration pursuant to Section 2.1(a); provided further that if, at the time of such withdrawal, the Holders shall have learned of a material adverse change in the condition, business or prospects of the Company from that known to the Holders at the time of their request and have withdrawn the request with reasonable promptness after learning of such

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information then the Holders shall not be required to pay any of such expenses and shall not forfeit their right to one registration pursuant to Section 2.1(a). All Selling Expenses relating to Registrable Securities registered pursuant to this Section 2 shall be borne and paid by the Holders pro rata on the basis of the number of Registrable Securities registered on their behalf.

2.7 Delay of Registration. No Holder shall have any right to obtain or seek an injunction restraining or otherwise delaying any registration pursuant to this Agreement as the result of any controversy that might arise with respect to the interpretation or implementation of this Section 2.

2.8 Indemnification. If any Registrable Securities are included in a registration statement under this Section 2:

(a) To the extent permitted by law, the Company will indemnify and hold harmless each selling Holder, and the partners, members, officers, directors, and stockholders of each such Holder; legal counsel and accountants for each such Holder; any underwriter (as defined in the Securities Act) for each such Holder; and each Person, if any, who controls such Holder or underwriter within the meaning of the Securities Act or the Exchange Act, against any Damages, and the Company will pay to each such Holder, underwriter, controlling Person, or other aforementioned Person any legal or other expenses reasonably incurred thereby in connection with investigating or defending any claim or proceeding from which Damages may result, as such expenses are incurred; provided, however, that the indemnity agreement contained in this Section 2.8(a) shall not apply to amounts paid in settlement of any such claim or proceeding if such settlement is effected without the consent of the Company, which consent shall not be unreasonably withheld, nor shall the Company be liable for any Damages to the extent that they arise out of or are based upon actions or omissions made in reliance upon and in conformity with written information furnished by or on behalf of any such Holder, underwriter, controlling Person, or other aforementioned Person expressly for use in connection with such registration.

(b) To the extent permitted by law, each selling Holder, severally and not jointly, will indemnify and hold harmless the Company, and each of its directors, each of its officers who has signed the registration statement, each Person (if any), who controls the Company within the meaning of the Securities Act, legal counsel and accountants for the Company, any underwriter (as defined in the Securities Act), any other Holder selling securities in such registration statement, and any controlling Person of any such underwriter or other Holder, against any Damages, in each case only to the extent that such Damages arise out of or are based upon actions or omissions made in reliance upon and in conformity with written information furnished by or on behalf of such selling Holder expressly for use in connection with such registration; and each such selling Holder will pay to the Company and each other aforementioned Person any legal or other expenses reasonably incurred thereby in connection with investigating or defending any claim or proceeding from which Damages may result, as such expenses are incurred; provided, however, that the indemnity agreement contained in this Section 2.8(b) shall not apply to amounts paid in settlement of any such claim or proceeding if such settlement is effected without the consent of the Holder, which consent shall not be unreasonably withheld; and provided further that in no event shall any indemnity under this Section 2.8(b) exceed the proceeds from the offering received by such Holder (net of any Selling Expenses paid by such Holder), except in the case of fraud or willful misconduct by such Holder.



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(c) Promptly after receipt by an indemnified party under this Section 2.8 of notice of the commencement of any action (including any governmental action) for which a party may be entitled to indemnification hereunder, such indemnified party will, if a claim in respect thereof is to be made against any indemnifying party under this Section 2.8, give the indemnifying party notice of the commencement thereof. The indemnifying party shall have the right to participate in such action and, to the extent the indemnifying party so desires, participate jointly with any other indemnifying party to which notice has been given, and to assume the defense thereof with counsel mutually satisfactory to the parties; provided, however, that an indemnified party (together with all other indemnified parties that may be represented without conflict by one counsel) shall have the right to retain one separate counsel, with the fees and expenses to be paid by the indemnifying party, if representation of such indemnified party by the counsel retained by the indemnifying party would be inappropriate due to actual or potential differing interests between such indemnified party and any other party represented by such counsel in such action. The failure to give notice to the indemnifying party within a reasonable time of the commencement of any such action shall relieve such indemnifying party of any liability to the indemnified party under this Section 2.8, to the extent that such failure materially prejudices the indemnifying party's ability to defend such action. The failure to give notice to the indemnifying party will not relieve it of any liability that it may have to any indemnified party otherwise than under this Section 2.8.

(d) Notwithstanding anything else herein to the contrary, the foregoing indemnity agreements of the Company and the selling Holders are subject to the condition that, insofar as they relate to any Damages arising from any untrue statement or alleged untrue statement of a material fact contained in, or omission or alleged omission of a material fact from, a preliminary prospectus (or necessary to make the statements therein not misleading) that has been corrected in the form of prospectus included in the registration statement at the time it becomes effective, or any amendment or supplement thereto filed with the SEC pursuant to Rule 424(b) under the Securities Act (the "**Final Prospectus**"), such indemnity agreement shall not inure to the benefit of any Person if a copy of the Final Prospectus was furnished to the indemnified party within a reasonable period prior to the applicable sale and such indemnified party failed to deliver, at or before the confirmation of the sale of the shares registered in such offering, a copy of the Final Prospectus to the Person asserting the loss, liability, claim, or damage in any case in which such delivery was required by the Securities Act.

(e) To provide for just and equitable contribution to joint liability under the Securities Act in any case in which either (i) any party otherwise entitled to indemnification hereunder makes a claim for indemnification pursuant to this Section 2.8 but it is judicially determined (by the entry of a final judgment or decree by a court of competent jurisdiction and the expiration of time to appeal or the denial of the last right of appeal) that such indemnification may not be enforced in such case, notwithstanding the fact that this Section 2.8 provides for indemnification in such case, or (ii) contribution under the Securities Act may be required on the part of any party hereto for which indemnification is provided under this Section 2.8, then, and in each such case, such parties will contribute to the aggregate losses, claims, damages, liabilities, or expenses to which they may be subject (after contribution from others) in such proportion as is appropriate to reflect the relative fault of each of the indemnifying party and the indemnified party in connection with the statements, omissions, or other actions that resulted in such loss, claim, damage, liability, or expense, as well as to reflect

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any other relevant equitable considerations. The relative fault of the indemnifying party and of the indemnified party shall be determined by reference to, among other things, whether the untrue or allegedly untrue statement of a material fact, or the omission or alleged omission of a material fact, relates to information supplied by the indemnifying party or by the indemnified party and the parties' relative intent, knowledge, access to information, and opportunity to correct or prevent such statement or omission; provided, however, that, in any such case, (x) no Holder will be required to contribute any amount in excess of the public offering price of all such Registrable Securities offered and sold by such Holder pursuant to such registration statement, and (y) no Person guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) will be entitled to contribution from any Person who was not guilty of such fraudulent misrepresentation; and provided further that in no event shall a Holder's liability pursuant to this Section 2.8(e), when combined with the amounts paid or payable by such Holder pursuant to Section 2.8(b), exceed the proceeds from the offering received by such Holder (net of any Selling Expenses paid by such Holder), except in the case of willful misconduct or fraud by such Holder.

(e) Notwithstanding the foregoing, to the extent that the provisions on indemnification and contribution contained in the underwriting agreement entered into in connection with the underwritten public offering are in conflict with the foregoing provisions, the provisions in the underwriting agreement shall control.

(f) Unless otherwise superseded by an underwriting agreement entered into in connection with the underwritten public offering, the obligations of the Company and Holders under this Section 2.8 shall survive the completion of any offering of Registrable Securities in a registration under this Section 2, and otherwise shall survive the termination of this Agreement.

2.9 Reports Under Exchange Act. With a view to making available to the Holders the benefits of SEC Rule 144 and any other rule or regulation of the SEC that may at any time permit a Holder to sell securities of the Company to the public without registration or pursuant to a registration on Form S-3, the Company shall:

(a) make and keep available adequate current public information, as those terms are understood and defined in SEC Rule 144, at all times after the effective date of the registration statement filed by the Company for the IPO;

(b) use commercially reasonable efforts to file with the SEC in a timely manner all reports and other documents required of the Company under the Securities Act and the Exchange Act (at any time after the Company has become subject to such reporting requirements); and

(c) furnish to any Holder, so long as the Holder owns any Registrable Securities, forthwith upon request (i) to the extent accurate, a written statement by the Company that it has complied with the reporting requirements of SEC Rule 144 (at any time after ninety (90) days after the effective date of the registration statement filed by the Company for the IPO), the Securities Act, and the Exchange Act (at any time after the Company has become subject to such reporting requirements), or that it qualifies as a registrant whose securities may be resold pursuant to Form S-3 (at any time after the Company so qualifies); (ii) a copy of the most recent

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annual or quarterly report of the Company and such other reports and documents so filed by the Company; and (iii) such other information as may be reasonably requested in availing any Holder of any rule or regulation of the SEC that permits the selling of any such securities without registration (at any time after the Company has become subject to the reporting requirements under the Exchange Act) or pursuant to Form S-3 (at any time after the Company so qualifies to use such form).

2.10 Limitations on Subsequent Registration Rights. From and after the date of this Agreement, the Company shall not, without the prior written consent of the Requisite Holders, enter into any agreement with any holder or prospective holder of any securities of the Company that would allow such holder or prospective holder (i) to include such securities in any registration unless, under the terms of such agreement, such holder or prospective holder may include such securities in any such registration only to the extent that the inclusion of such securities will not reduce the number of the Registrable Securities of the Holders that are included or (ii) to demand registration of any securities held by such holder or prospective holder; provided that this limitation shall not apply to any additional Investor who becomes a party to this Agreement in accordance with Section 6.9.

2.11 "Market Stand-off" Agreement. Each Holder hereby agrees that, if required by the managing underwriter and the Company, it will not, without the prior written consent of the managing underwriter, during the period commencing on the date of the final prospectus relating to the IPO and ending on the date specified by the Company and the managing underwriter (such period not to exceed one hundred eighty (180) days, which period may be extended upon the request of the managing underwriter, for an additional period of up to thirty-four (34) days in order to address FINRA Rule 2711(f) to the extent then applicable or any similar successor provision, (i) lend; offer; pledge; sell; contract to sell; sell any option or contract to purchase; purchase any option or contract to sell; grant any option, right, or warrant to purchase; or otherwise transfer or dispose of, directly or indirectly, any shares of Common Stock or any Derivative Securities (whether such shares or any such Derivative Securities are then owned by the Holder or are thereafter acquired but specifically excluding any shares of Common Stock or Derivative Securities that are purchased in the IPO or in the open market after the IPO, provided that no filing under Section 16(a) of the Exchange Act shall be required or shall be made during the 180-day period referred to above (and any extension thereof as provided above) in connection with subsequent sales of such shares of Common Stock or any Derivative Securities so purchased in the IPO or in the open market after the IPO) or (ii) enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of such securities, whether any such transaction described in clause (i) or (ii) above is to be settled by delivery of Common Stock or other securities, in cash, or otherwise. The foregoing provisions of this Section 2.11 shall apply only to the IPO, shall not apply to the sale of any shares to an underwriter pursuant to an underwriting agreement, and shall be applicable to the Holders only if all officers and directors, and all stockholders individually owning more than one percent (1%) of the Company's outstanding Common Stock (after giving effect to conversion into Common Stock of all outstanding Preferred Stock) are subject to substantially similar restrictions. The underwriters in connection with such registration are intended third-party beneficiaries of this Section 2.11 and shall have the right, power, and authority to enforce the provisions hereof as though they were a party hereto. Each Holder further agrees to execute such agreements as may be reasonably requested by the

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underwriters in connection with such registration that are consistent with this Section 2.11 or that are necessary to give further effect thereto. Notwithstanding the foregoing, the Company shall use commercially reasonable best efforts to obtain from the managing underwriter(s) an agreement to waive these restrictions in order to provide for periodic early releases of portions of the aforesaid securities upon the occurrence of certain specified events, any such release to apply pro rata to all Holders subject to this Section 2.11, based on the number of securities (determined on an as-converted basis) subject to the restrictions set forth in this Section 2.11.

#### 2.12 Restrictions on Transfer.

(a) The Preferred Stock and the Registrable Securities shall not be sold, pledged, or otherwise transferred, and the Company shall not recognize any such sale, pledge, or transfer, except upon the conditions specified in this Agreement, which conditions are intended to ensure compliance with the provisions of the Securities Act. A transferring Holder will cause any proposed purchaser, pledgee, or transferee of the Preferred Stock and the Registrable Securities held by such Holder to agree to take and hold such securities subject to the provisions and upon the conditions specified in this Agreement.

(b) Each certificate or instrument representing (i) the Preferred Stock, (ii) the Registrable Securities, and (iii) any other securities issued in respect of the securities referenced in clauses (i) and (ii), upon any stock split, stock dividend, recapitalization, merger, consolidation, or similar event, shall (unless otherwise permitted by the provisions of Section 2.12(c)) be stamped or otherwise imprinted with a legend substantially in the following form:

THE SECURITIES REPRESENTED HEREBY HAVE BEEN ACQUIRED FOR INVESTMENT AND HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933. SUCH SHARES MAY NOT BE SOLD, PLEDGED, OR TRANSFERRED IN THE ABSENCE OF SUCH REGISTRATION OR A VALID EXEMPTION FROM THE REGISTRATION AND PROSPECTUS DELIVERY REQUIREMENTS OF SAID ACT.

THE SECURITIES REPRESENTED HEREBY MAY BE TRANSFERRED ONLY IN ACCORDANCE WITH THE TERMS OF AN AGREEMENT BETWEEN THE COMPANY AND THE STOCKHOLDER, A COPY OF WHICH IS ON FILE WITH THE SECRETARY OF THE COMPANY.

The Holders consent to the Company making a notation in its records and giving instructions to any transfer agent of the Restricted Securities in order to implement the restrictions on transfer set forth in this Section 2.12.

(c) The holder of each certificate representing Restricted Securities, by acceptance thereof, agrees to comply in all respects with the provisions of this Section 2. Before any proposed sale, pledge, or transfer of any Restricted Securities, unless there is in effect a registration statement under the Securities Act covering the proposed transaction, the Holder thereof shall give notice to the Company of such Holder's intention to effect such sale, pledge, or transfer. Each such notice shall describe the manner and circumstances of the proposed sale, pledge, or transfer in sufficient detail and, if reasonably requested by the Company, shall be

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accompanied at such Holder's expense by either (i) a written opinion of legal counsel who shall, and whose legal opinion shall, be reasonably satisfactory to the Company, addressed to the Company, to the effect that the proposed transaction may be effected without registration under the Securities Act; (ii) a "no action" letter from the SEC to the effect that the proposed sale, pledge, or transfer of such Restricted Securities without registration will not result in a recommendation by the staff of the SEC that action be taken with respect thereto; or (iii) any other evidence reasonably satisfactory to counsel to the Company to the effect that the proposed sale, pledge, or transfer of the Restricted Securities may be effected without registration under the Securities Act, whereupon the Holder of such Restricted Securities shall be entitled to sell, pledge, or transfer such Restricted Securities in accordance with the terms of the notice given by the Holder to the Company. The Company will not require such a legal opinion or "no action" letter (x) in any transaction in compliance with SEC Rule 144 or (y) in any transaction in which such Holder distributes Restricted Securities to an Affiliate of such Holder for no consideration; provided that each transferee agrees in writing to be subject to the terms of this Section 2.12. Each certificate or instrument evidencing the Restricted Securities transferred as above provided shall bear, except if such transfer is made pursuant to SEC Rule 144, the appropriate restrictive legend set forth in Section 2.12(b), except that such certificate shall not bear such restrictive legend if, in the opinion of counsel for such Holder and the Company, such legend is not required in order to establish compliance with any provisions of the Securities Act.

2.13 Termination of Registration Rights. The right of any Holder to request registration or inclusion of Registrable Securities in any registration pursuant to Section 2.1 or Section 2.2 shall terminate upon the later to occur of:

- (a) the date on which all of such Holder's Registrable Securities have been sold; or
- (b) the fifth anniversary of the Qualified Public Offering.

### 3. Information and Observer Rights.

3.1 Delivery of Financial Statements. The Company shall deliver to each Investor:

(a) as soon as practicable, but in any event within one hundred twenty (120) days after the end of each fiscal year of the Company, (i) a balance sheet as of the end of such year, (ii) statements of income and of cash flows for such year, and a comparison between (x) the actual amounts as of and for such fiscal year and (y) the comparable amounts for the prior year and as included in the Budget (as defined in Section 3.1(e)) for such year, with an explanation of any material differences between such amounts and a schedule as to the sources and applications of funds for such year, and (iii) a statement of stockholders' equity as of the end of such year, all such financial statements audited and certified by independent public accountants of regionally or nationally recognized standing selected by the Company and approved by the Board of Directors, which approval shall include the approval of the Requisite Preferred Directors.

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(b) as soon as practicable, but in any event within forty-five (45) days after the end of each of the first three (3) quarters of each fiscal year of the Company, unaudited statements of income and of cash flows for such fiscal quarter, and an unaudited balance sheet and a statement of stockholders' equity as of the end of such fiscal quarter, all prepared in accordance with GAAP (except that such financial statements may (i) be subject to normal year-end audit adjustments and (ii) not contain all notes thereto that may be required in accordance with GAAP);

(c) as soon as practicable, but in any event within forty-five (45) days after the end of each of the first three (3) quarters of each fiscal year of the Company, a statement showing the number of shares of each class and series of capital stock and securities convertible into or exercisable for shares of capital stock outstanding at the end of the period, the Common Stock issuable upon conversion or exercise of any outstanding securities convertible or exercisable for Common Stock and the exchange ratio or exercise price applicable thereto, and the number of shares of issued stock options and stock options not yet issued but reserved for issuance, if any, all in sufficient detail as to permit the Investors to calculate their respective percentage equity ownership in the Company, and certified by the chief financial officer or chief executive officer of the Company as being true, complete, and correct;

(d) as soon as practicable, but in any event within thirty (30) days of the end of each month, an unaudited income statement and statement of cash flows for such month, and an unaudited balance sheet and statement of stockholders' equity as of the end of such month, all prepared in accordance with GAAP (except that such financial statements may (i) be subject to normal year-end audit adjustments and (ii) not contain all notes thereto that may be required in accordance with GAAP); and

(e) as soon as practicable, but in any event thirty (30) days before the end of each fiscal year, a budget and business plan for the next fiscal year (collectively, the "**Budget**"), approved by the Board of Directors, which approval shall include the approval of the Requisite Preferred Directors, and prepared on a monthly basis, including balance sheets, income statements, and statements of cash flow for such months and, promptly after prepared, any other budgets or revised budgets prepared by the Company.

If, for any period, the Company has any subsidiary whose accounts are consolidated with those of the Company, then in respect of such period the financial statements delivered pursuant to the foregoing sections shall be the consolidated and consolidating financial statements of the Company and all such consolidated subsidiaries.

Notwithstanding anything else in this Section 3.1 to the contrary, the Company may cease providing the information set forth in this Section 3.1 during the period starting with the date sixty (60) days before the Company's good-faith estimate of the date of filing of a registration statement if it reasonably concludes it must do so to comply with the SEC rules applicable to such registration statement and related offering; provided that the Company's covenants under this Section 3.1 shall be reinstated at such time as the Company is no longer actively employing its commercially reasonable efforts to cause such registration statement to become effective.

**3.2 Inspection.** The Company shall permit each Investor, at such Investor's expense, to visit and inspect the Company's properties; examine its books of account and records; and discuss the Company's affairs, finances, and accounts with its officers, upon providing reasonable notice during normal business hours of the Company as may be reasonably

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requested by the Investor; provided, however, that the Company shall not be obligated pursuant to this Section 3.2 to provide access to any information that it reasonably and in good faith considers to be a trade secret or confidential information (unless covered by an enforceable confidentiality agreement, in form acceptable to the Company) or the disclosure of which would adversely affect the attorney-client privilege between the Company and its counsel.

3.3 Observer Rights. The Company shall invite a representative of (i) Genzyme Corporation, (ii) Shire plc and (iii) Third Rock Ventures, LLC to attend all meetings of its Board of Directors in a nonvoting observer capacity and shall give such representatives copies of all notices, minutes, consents, and other materials that it provides to its directors at the same time and in the same manner as provided to such directors; provided, however, that such Investor shall agree to hold (and cause its representative to hold) in confidence all information so provided; provided further, that the Company reserves the right to withhold any information and to exclude such representatives from any meeting or portion thereof if the Company's Board of Directors determines in good faith that access to such information or attendance at such meeting could adversely affect the attorney-client privilege between the Company and its counsel or result in disclosure of trade secrets or a conflict of interest. The observer rights provided by this Section 3.3, including the foregoing confidentiality obligations, shall be set forth in letter agreements in a form reasonably acceptable to the Company with each of the Investors named in this Section 3.3.

3.4 Informational Meetings. Each Investor shall be entitled to participate in semi-annual informational meetings with members of the Company's management (the "**Informational Meetings**"). The Company shall provide reasonable notice to the Investors in advance of each Informational Meeting, which shall be held at a time and place determined by the Company (which may include attendance in-person and by remote access at the option of the Investors).

3.5 Termination of Information. The covenants set forth in Sections 3.1, 3.2, 3.3 and 3.4 shall terminate and be of no further force or effect (i) immediately before but subject to the consummation of a Qualified Public Offering or (ii) when the Company first becomes subject to the periodic reporting requirements of Section 12(g) or 15(d) of the Exchange Act, whichever event occurs first.

3.6 Confidentiality. Each Investor agrees that such Investor will keep confidential and will not disclose, divulge, or use for any purpose (other than to monitor its investment in the Company) any confidential information obtained from the Company pursuant to the terms of this Agreement (including notice of the Company's intention to file a registration statement), unless such confidential information (a) is known or becomes known to the public in general (other than as a result of a breach of this Section 3.6 by such Investor), (b) is or has been independently developed or conceived by the Investor without use of the Company's confidential information, or (c) is or has been made known or disclosed to the Investor by a third party without a breach of any obligation of confidentiality such third party may have to the Company; provided, however, that an Investor may disclose confidential information (i) to its attorneys, accountants, consultants, investment advisors and other professionals to the extent necessary to obtain their services in connection with monitoring its investment in the Company; (ii) to any prospective purchaser of any Registrable Securities from such Investor, if such prospective purchaser agrees to be bound by the provisions of this Section 3.6; (iii) to any existing or

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prospective Affiliate, partner, member, stockholder, or wholly owned subsidiary of such Investor in the ordinary course of business, provided that such Investor informs such Person that such information is confidential and directs such Person to maintain the confidentiality of such information except as may be (and solely to the extent) required by reporting obligations of the Investor or its investment advisor; or (iv) as may otherwise be required by law, provided that the Investor promptly notifies the Company of such disclosure and takes reasonable steps to minimize the extent of any such required disclosure.

#### 4. Rights to Future Stock Issuances.

4.1 Right of First Offer. Unless waived by the Requisite Holders and subject to the terms and conditions of this Section 4.1 and applicable securities laws, if the Company proposes to offer or sell any New Securities, the Company shall first offer such New Securities to each Investor. An Investor shall be entitled to apportion the right of first offer hereby granted to it among itself and its Affiliates in such proportions as it deems appropriate.

(a) The Company shall give notice (the “**Offer Notice**”) to each Investor, stating (i) its bona fide intention to offer such New Securities, (ii) the number of such New Securities to be offered, and (iii) the price and terms, if any, upon which it proposes to offer such New Securities.

(b) By notification to the Company within twenty (20) days after the Offer Notice is given, each Investor may elect to purchase or otherwise acquire, at the price and on the terms specified in the Offer Notice, up to that portion of such New Securities which equals the proportion that the Common Stock issued and held, or issuable (directly or indirectly) upon conversion, exercise or exchange, as applicable, of Derivative Securities then held, by such Investor bears to the total Common Stock of the Company then outstanding (assuming full conversion, exercise or exchange, as applicable, of all Derivative Securities). At the expiration of such twenty (20) day period, the Company shall promptly notify each Investor that elects to purchase or acquire all the shares available to it (each, a “**Fully Exercising Investor**”) of any other Investor’s failure to do likewise. During the ten (10) day period commencing after the Company has given such notice, each Fully Exercising Investor may, by giving notice to the Company, elect to purchase or acquire, in addition to the number of shares specified above, up to that portion of the New Securities for which Investors were entitled to subscribe but that were not subscribed for by the Investors which is equal to the proportion that the Common Stock issued and held, or issuable (directly or indirectly) upon conversion, exercise or exchange, as applicable, of Derivative Securities then held, by such Fully Exercising Investor bears to the Common Stock issued and held, or issuable (directly or indirectly) upon conversion, exercise or exchange, as applicable, of Derivative Securities then held, by all Fully Exercising Investors who wish to purchase such unsubscribed shares. The closing of any sale pursuant to this Section 4.1(b) shall occur within the later of ninety (90) days of the date that the Offer Notice is given and the date of initial sale of New Securities pursuant to Section 4.1(c).

(c) If all New Securities referred to in the Offer Notice are not elected to be purchased or acquired as provided in Section 4.1(b), the Company may, during the ninety (90) day period following the expiration of the periods provided in Section 4.1(b), offer and sell the remaining unsubscribed portion of such New Securities to any Person or Persons at a price not less than, and upon terms no more favorable to the offeree than, those specified in the Offer



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Notice. If the Company does not enter into an agreement for the sale of the New Securities within such period, or if such agreement is not consummated within thirty (30) days of the execution thereof, the right provided hereunder shall be deemed to be revived and such New Securities shall not be offered unless first reoffered to the Investors in accordance with this Section 4.1.

(d) The right of first offer in this Section 4.1 shall not be applicable to (i) Exempted Securities (as defined in the Company's Certificate of Incorporation); (ii) shares of Common Stock issued in the IPO; and (iii) the issuance of shares of Series D Preferred Stock pursuant to the Series D Purchase Agreement.

4.2 Termination. The covenants set forth in Section 4.1 shall terminate and be of no further force or effect (i) immediately before the consummation of the Qualified Public Offering, or (ii) when the Company first becomes subject to the periodic reporting requirements of Section 12(g) or 15(d) of the Exchange Act, whichever event occurs first.

#### 5. Additional Covenants.

5.1 Insurance. The Company shall use its commercially reasonable efforts to maintain from financially sound and reputable insurers Directors and Officers liability insurance in an amount and on terms and conditions satisfactory to the Board of Directors, including the Requisite Preferred Directors, and will use commercially reasonable efforts to cause such insurance policy to be maintained until such time as the Board of Directors, including the affirmative vote of the Requisite Preferred Directors, determines that such insurance should be discontinued.

5.2 Employee Agreements. The Company will cause (i) each person now or hereafter employed by it or by any subsidiary (or engaged by the Company or any subsidiary as a consultant or independent contractor, including any member of the Company's scientific advisory board) with access to confidential information and/or trade secrets to enter into a nondisclosure and proprietary rights assignment agreement and, to the extent permitted by law, (ii) each Key Employee to enter into a one (1) year noncompetition and nonsolicitation agreement, substantially in the form approved by the Board of Directors, including the affirmative vote of the Requisite Preferred Directors, all subject to the policies of any academic or research institution with which any such person may be affiliated. In addition, the Company shall not amend, modify, terminate, waive, or otherwise alter, in whole or in part, any of the above-referenced agreements or any restricted stock agreement between the Company and any employee, without the consent of the Requisite Preferred Directors.

5.3 Employee Vesting. Unless otherwise approved by the Board of Directors, which approval must include the affirmative vote of the Requisite Preferred Directors, all future employees and consultants of the Company who purchase, receive options to purchase, or receive awards of shares of the Company's capital stock after the date hereof shall be required to execute restricted stock or option agreements, as applicable, providing for (i) vesting of shares over a four (4) year period, with the first twenty-five percent (25%) of such shares vesting following twelve (12) months of continued employment or service, and the remaining shares vesting in equal monthly installments over the following thirty-six (36) months and (ii) a one hundred eighty day (180) day lockup period in connection with the Company's IPO. In addition,

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unless otherwise approved by the Board of Directors, including the affirmative vote of the Requisite Preferred Directors, the Company shall retain a “right of first refusal” on employee transfers until the Company’s IPO and shall have the right to repurchase unvested shares at cost upon termination of employment of a holder of restricted stock.

5.4 [Reserved.]

5.5 Matters Requiring Investor Director Approval. The Company hereby covenants and agrees with each of the Investors that it shall not, nor shall it permit any subsidiary to, without approval of the Board of Directors, which approval must include the affirmative vote of the Requisite Preferred Directors:

(a) make any loan or advance to, or own any stock or other securities of, any subsidiary or other corporation, partnership, or other entity unless it is wholly owned by the Company;

(b) make any loan or advance to any Person, including, without limitation, any employee or director of the Company or any subsidiary, except advances and similar expenditures in the ordinary course of business or under the terms of an employee stock or option plan approved by the Board of Directors;

(c) guarantee, directly or indirectly, any indebtedness except for trade accounts of the Company or any subsidiary arising in the ordinary course of business;

(d) make any investment other than investments in prime commercial paper, money market funds, certificates of deposit in any United States bank having a net worth in excess of \$100,000,000, or obligations issued or guaranteed by the United States of America, in each case having a maturity not in excess of two years;

(e) otherwise enter into or be a party to any transaction with any director, officer, or employee of the Company or any “associate” (as defined in Rule 12b-2 promulgated under the Exchange Act) of any such Person, except for transactions contemplated by the Purchase Agreement;

(f) hire, terminate, or change the compensation of the executive officers, including approving any option grants or stock awards to executive officers.

(g) change the principal business of the Company, enter new lines of business, or exit the current line of business; or

(h) sell, assign, license, pledge, or encumber material technology or intellectual property, other than licenses granted in the ordinary course of business.

5.6 Meetings of the Board of Directors. Unless otherwise determined by the vote of a majority of the directors then in office, the Board of Directors shall meet at least quarterly in accordance with an agreed-upon schedule.

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5.7 Successor Indemnification. If the Company or any of its successors or assignees consolidates with or merges into any other Person and is not the continuing or surviving corporation or entity of such consolidation or merger, then to the extent necessary, proper provision shall be made so that the successors and assignees of the Company assume the obligations of the Company with respect to indemnification of members of the Board of Directors as in effect immediately before such transaction, whether such obligations are contained in the Company's Bylaws, its Certificate of Incorporation, or elsewhere, as the case may be.

5.8 Board Expenses. The Company shall reimburse nonemployee directors and the observers appointed pursuant to Section 3.3 for all reasonable out-of-pocket travel expenses incurred in connection with attending meetings of the Board of Directors.

5.9 Termination of Covenants. The covenants set forth in this Section 5, except for Section 5.8, shall terminate and be of no further force or effect (i) immediately before the consummation of the Qualified Public Offering or (ii) when the Company first becomes subject to the periodic reporting requirements of Section 12(g) or 15(d) of the Exchange Act, whichever event occurs first.

## 6. Miscellaneous.

6.1 Successors and Assigns. The rights under this Agreement may be assigned (but only with all related obligations) by a Holder to a transferee of Registrable Securities that (i) is an Affiliate, partner, member, limited partner, retired partner, retired member, or stockholder of a Holder; (ii) is a Holder's Immediate Family Member or trust for the benefit of an individual Holder or one or more of such Holder's Immediate Family Members; or (iii) after such transfer, holds at least 50,000 shares of Registrable Securities (subject to appropriate adjustment for stock splits, stock dividends, combinations, and other recapitalizations); provided, however, that (x) the Company is, within a reasonable time after such transfer, furnished with written notice of the name and address of such transferee and the Registrable Securities with respect to which such rights are being transferred; and (y) such transferee agrees in a written instrument delivered to the Company to be bound by and subject to the terms and conditions of this Agreement, including the provisions of Section 2.11. For the purposes of determining the number of shares of Registrable Securities held by a transferee, the holdings of a transferee (1) that is an Affiliate, partner, member, limited partner, retired partner, retired member, or stockholder or stockholder of a Holder; (2) who is a Holder's Immediate Family Member; or (3) that is a trust for the benefit of an individual Holder or such Holder's Immediate Family Member shall be aggregated together and with those of the transferring Holder; provided further that all transferees who would not qualify individually for assignment of rights shall have a single attorney-in-fact for the purpose of exercising any rights, receiving notices, or taking any action under this Agreement. The terms and conditions of this Agreement inure to the benefit of and are binding upon the respective successors and permitted assignees of the parties. Nothing in this Agreement, express or implied, is intended to confer upon any party other than the parties hereto or their respective successors and permitted assignees any rights, remedies, obligations or liabilities under or by reason of this Agreement, except as expressly provided herein.

6.2 Governing Law. This Agreement and any controversy arising out of or relating to this Agreement shall be governed by and construed in accordance with the General Corporation Law of the State of Delaware as to matters within the scope thereof, and as to all

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other matters shall be governed by and construed in accordance with the internal laws of The Commonwealth of Massachusetts, without regard to conflict of law principles that would result in the application of any law other than the law of The Commonwealth of Massachusetts.

6.3 Counterparts; Facsimile. This Agreement may be executed in two or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. This Agreement may also be executed and delivered by facsimile or electronic mail signature and in two or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

6.4 Titles and Subtitles. The titles and subtitles used in this Agreement are for convenience only and are not to be considered in construing or interpreting this Agreement.

6.5 Notices. All notices, requests, and other communications given or made pursuant to this Agreement shall be in writing and shall be deemed effectively given, delivered and received (i) upon personal delivery to the party to be notified; (ii) when sent by confirmed electronic mail or facsimile if sent during normal business hours of the recipient, and if not so confirmed, then on the next business day; (iii) five (5) days after having been sent by registered or certified mail, return receipt requested, postage prepaid; or (iv) one (1) business day after the business day of deposit with a nationally recognized overnight courier, specifying next-day delivery, with written verification of receipt. All communications shall be sent to the respective parties at their addresses as set forth on Schedule A hereto, or to the principal office of the Company and to the attention of the Chief Executive Officer, in the case of the Company, or to such email address, facsimile number, or address as subsequently modified by written notice given in accordance with this Section 6.5. If notice is given to the Company, a copy shall also be sent to Goodwin Procter LLP, 53 State Street, Boston, Massachusetts 02109, Attention: Michael H. Bison, Esq.

6.6 Amendments and Waivers. Any term of this Agreement, including without limitation Section 4.1, may be amended and the observance of any term of this Agreement may be waived (either generally or in a particular instance, and either retroactively or prospectively) only with the written consent of the Company and the Requisite Holders; provided that the Company may in its sole discretion waive compliance with Section 2.12(c) (and the Company's failure to object promptly in writing after notification of a proposed assignment allegedly in violation of Section 2.12(c) shall be deemed to be a waiver); and provided further that any provision hereof may be waived by any waiving party on such party's own behalf, without the consent of any other party. Notwithstanding the foregoing, this Agreement may not be amended or terminated and the observance of any term hereof may not be waived with respect to any Investor without the written consent of such Investor, unless such amendment, termination, or waiver applies to all Investors in the same fashion (it being agreed that a waiver of the provisions of Section 4 with respect to a particular transaction shall be deemed to apply to all Investors in the same fashion if such waiver does so by its terms, notwithstanding the fact that certain Investors may nonetheless, by agreement with the Company, purchase securities in such transaction); provided that (a) Section 3.3 of this Agreement shall not be amended or waived without the written consent of the Investors named therein, (b) no changes to Section 2.11 that make any provision therein more restrictive as to an Investor shall be enforceable against such Investor without its consent (and in the absence of such consent, Section 2.11 in such form as it exists as of July 23, 2012 shall remain in effect with respect to

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such Investor), and (c) no changes to Sections 3.1, 3.2 or 3.4 that reduce the rights provided to any Investor thereunder shall be enforceable against such Investor without its consent (and in the absence of such consent, the applicable Section in such form as it exists as of July 23, 2012 shall remain in effect with respect to such Investor). The Company shall give prompt notice of any amendment or termination hereof or waiver hereunder to any party hereto that did not consent in writing to such amendment, termination, or waiver. Any amendment, termination, or waiver effected in accordance with this Section 6.6 shall be binding on all parties hereto, regardless of whether any such party has consented thereto. No waivers of or exceptions to any term, condition, or provision of this Agreement, in any one or more instances, shall be deemed to be or construed as a further or continuing waiver of any such term, condition, or provision.

6.7 Severability. In case any one or more of the provisions contained in this Agreement is for any reason held to be invalid, illegal or unenforceable in any respect, such invalidity, illegality, or unenforceability shall not affect any other provision of this Agreement, and such invalid, illegal, or unenforceable provision shall be reformed and construed so that it will be valid, legal, and enforceable to the maximum extent permitted by law.

6.8 Aggregation of Stock. All shares of Registrable Securities held or acquired by Affiliates or Investors with the same or affiliated investment advisor shall be aggregated together for the purpose of determining the availability of any rights under this Agreement and such Affiliated persons may apportion such rights as among themselves in any manner they deem appropriate.

6.9 Additional Investors. Notwithstanding anything to the contrary contained herein, if the Company issues additional shares of Series D Preferred Stock after the date hereof, any purchaser of such shares of Series D Preferred Stock may become a party to this Agreement by executing and delivering an additional counterpart signature page to this Agreement, and thereafter shall be deemed an "Investor" for all purposes hereunder. No action or consent by the Investors shall be required for such joinder to this Agreement by such additional Investor, so long as such additional Investor has agreed in writing to be bound by all of the obligations as an "Investor" hereunder.

6.10 Entire Agreement. This Agreement (including the Schedule and Exhibit hereto) constitutes the full and entire understanding and agreement among the parties with respect to the subject matter hereof, and any other written or oral agreement relating to the subject matter hereof existing between the parties is expressly canceled. Upon the effectiveness of this Agreement, the Prior Agreement shall be deemed superseded and replaced in its entirety by this Agreement, and shall be of no further force or effect.

6.11 Jurisdiction; Venue. The parties (a) hereby irrevocably and unconditionally submit to the jurisdiction of the federal and state courts located within the geographic boundaries of the United States District Court for the District of Massachusetts for the purpose of any suit, action or other proceeding arising out of or based upon this Agreement, (b) agree not to commence any suit, action or other proceeding arising out of or based upon this Agreement except in the federal and state courts located within the geographic boundaries of the United States District Court for the District of Massachusetts, and (c) hereby waive, and agree not to assert, by way of motion, as a defense, or otherwise, in any such suit, action or proceeding, any claim that it is not subject personally to the jurisdiction of the above-named courts, that its

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property is exempt or immune from attachment or execution, that the suit, action or proceeding is brought in an inconvenient forum, that the venue of the suit, action or proceeding is improper or that this Agreement or the subject matter hereof may not be enforced in or by such court. The prevailing party shall be entitled to reasonable attorney's fees, costs, and necessary disbursements in addition to any other relief to which such party may be entitled. Each of the parties to this Agreement consents to personal jurisdiction for any equitable action sought in the U.S. District Court for the District of Massachusetts or any court of The Commonwealth of Massachusetts having subject matter jurisdiction.

6.12 Delays or Omissions. No delay or omission to exercise any right, power, or remedy accruing to any party under this Agreement, upon any breach or default of any other party under this Agreement, shall impair any such right, power, or remedy of such nonbreaching or nondefaulting party, nor shall it be construed to be a waiver of or acquiescence to any such breach or default, or to any similar breach or default thereafter occurring, nor shall any waiver of any single breach or default be deemed a waiver of any other breach or default theretofore or thereafter occurring. All remedies, whether under this Agreement or by law or otherwise afforded to any party, shall be cumulative and not alternative.

6.13 Acknowledgment. The Company acknowledges that the Investors are in the business of venture capital investing and therefore review the business plans and related proprietary information of many enterprises, including enterprises which may have products or services which compete directly or indirectly with those of the Company. Nothing in this Agreement shall preclude or in any way restrict the Investors from investing or participating in any particular enterprise whether or not such enterprise has products or services which compete with those of the Company.

6.14 Waiver of Conflicts. Each party to this Agreement acknowledges that Goodwin Procter LLP, counsel for the Company, has in the past and may continue to perform legal services for certain of the Investors in matters unrelated to the transactions described in this Agreement, including the representation of such Investors in venture capital financings and other matters. Accordingly, each party to this Agreement hereby (a) acknowledges that they have had an opportunity to ask for information relevant to this disclosure; (b) acknowledges that Goodwin Procter LLP represented the Company in the transaction contemplated by this Agreement and has not represented any individual Investor or any individual stockholder or employee of the Company in connection with such transaction; and (c) gives its informed written consent to Goodwin Procter LLP's representation of certain of the Investors in such unrelated matters and to Goodwin Procter LLP's representation of the Company in connection with this Agreement and the transactions contemplated hereby.

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IN WITNESS WHEREOF, the parties have executed this Amended and Restated Investors' Rights Agreement as of the date first written above.

COMPANY:

**BLUEBIRD BIO, INC.**

By: /s/ Nick Leschly

Name: Nick Leschly

Title: President and Chief Executive Officer

[Signature Page to Amended and Restated Investors' Rights Agreement]

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IN WITNESS WHEREOF, the parties have executed this Amended and Restated Investors' Rights Agreement as of the date first written above.

INVESTORS:

**ARCH VENTURE FUND VII, L.P.**

By: ARCH Venture Partners VII, L.P.

Its: General Partner

By: ARCH Venture Partners VII, LLC

Its: General Partner

By: /s/ Clinton W. Bybee

Name: Clinton Bybee

Title: Manager Director

[Signature Page to Amended and Restated Investors' Rights Agreement]



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INVESTORS:

**THIRD ROCK VENTURES, L.P.**

By: Third Rock Ventures GP, L.P., its general partner

By: TRV GP, LLC, its general partner

By: /s/ Robert Tepper

Name: Robert Tepper

Title: Manager

[Signature Page to Amended and Restated Investors' Rights Agreement]

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INVESTORS:

**TVM V LIFE SCIENCE VENTURES GMBH & CO. KG**

By: /s/ Axel Polack  
Name: Dr. Axel Polack  
Title: Managing Limited Partner

By: /s/ Mark G. Cipriano  
Name: Mark G. Cipriano  
Title: Managing Limited Partner

[Signature Page to Amended and Restated Investors' Rights Agreement]

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INVESTORS:

**COOPERATIVE AAC LS U.A.**

By: /s/ Bart Bergstein

Name: Bart Bergstein

Title: Managing Partner

By: /s/ Christina Takke

Name: Christina Takke

Title: Partner

[Signature Page to Amended and Restated Investors' Rights Agreement]

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INVESTORS:

**SMALLCAP WORLD FUND, INC.**

By: Capital Research and Management Company  
Its: Investment Advisor

By: /s/ Michael J. Downer

Name: Michael J. Downer

Title: Senior Vice President and Secretary

**AMERICAN FUNDS INSURANCE SERIES –  
GLOBAL SMALL CAPITALIZATION FUND**

By: Capital Research and Management Company  
Its: Investment Advisor

By: /s/ Michael J. Downer

Name: Michael J. Downer

Title: Senior Vice President and Secretary

[Signature Page to Amended and Restated Investors' Rights Agreement]

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IN WITNESS WHEREOF, the parties have executed this Amended and Restated Investors' Rights Agreement as of the date first written above.

INVESTORS:

**DEERFIELD SPECIAL SITUATIONS FUND, L.P.**

By: Deerfield Capital, L.P.  
General Partner

By: J.E. Flynn Capital, LLC  
General Partner

By: /s/ James Flynn  
Name: James Flynn  
Title: General Partner

**DEERFIELD SPECIAL SITUATIONS  
INTERNATIONAL MASTER FUND, L.P.**

By: Deerfield Capital, L.P.  
General Partner

By: J.E. Flynn Capital, LLC  
General Partner

By: /s/ James Flynn  
Name: James Flynn  
Title: General Partner

[Signature Page to Amended and Restated Investors' Rights Agreement]

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INVESTORS:

**RA CAPITAL HEALTHCARE FUND, LP**

By: /s/ Peter Kolchinsky

Name: Peter Kolchinsky

Title: Manager

[Signature Page to Amended and Restated Investors' Rights Agreement]

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INVESTORS:

**COWEN BLUEBIRD LLC**

By: Ramius Advisors, LLC, its managing member

By: /s/ Andrew Cohen

Name: Andrew Cohen

Title: Managing Director

By: /s/ Owen Littman

Name: Owen Littman

Title: Authorized Signatory

[Signature Page to Amended and Restated Investors' Rights Agreement]

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INVESTORS:

**SHIRE LLC**

By: /s/ Mike Chapman

Name: Mike Chapman

Title: President

[Signature Page to Amended and Restated Investors' Rights Agreement]



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INVESTORS:

**FIDELITY CONTRAFUND: FIDELITY  
CONTRAFUND**

By: /s/ Adrien Deberghes

Name: Adrien Deberghes

Title: Deputy Treasurer

[Signature Page to Amended and Restated Investors' Rights Agreement]

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IN WITNESS WHEREOF, the parties have executed this Amended and Restated Investors' Rights Agreement as of the date first written above.

INVESTORS:

**FIDELITY CONTRAFUND: FIDELITY ADVISOR  
NEW INSIGHTS FUND**

By: /s/ Adrien Deberghes

Name: Adrien Deberghes

Title: Deputy Treasurer

[Signature Page to Amended and Restated Investors' Rights Agreement]

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INVESTORS:

**FIDELITY MT. VERNON STREET TRUST: FIDELITY  
GROWTH COMPANY FUND**

By: /s/ Adrien Deberghes

Name: Adrien Deberghes

Title: Deputy Treasurer

[Signature Page to Amended and Restated Investors' Rights Agreement]

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INVESTORS:

**FIDELITY SELECT PORTFOLIOS:  
BIOTECHNOLOGY PORTFOLIO**

By: /s/ Adrien Deberghes

Name: Adrien Deberghes

Title: Deputy Treasurer

[Signature Page to Amended and Restated Investors' Rights Agreement]

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IN WITNESS WHEREOF, the parties have executed this Amended and Restated Investors' Rights Agreement as of the date first written above.

INVESTORS:

**FIDELITY ADVISOR SERIES VII: FIDELITY  
ADVISOR BIOTECHNOLOGY FUND**

By: /s/ Adrien Deberghes

Name: Adrien Deberghes

Title: Deputy Treasurer

[Signature Page to Amended and Restated Investors' Rights Agreement]

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INVESTORS:

**FIDELITY MAGELLAN FUND: FIDELITY  
MAGELLAN FUND**

By: /s/ Adrien Deberghes

Name: Adrien Deberghes

Title: Deputy Treasurer

[Signature Page to Amended and Restated Investors' Rights Agreement]

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**SCHEDULE A**

**Investors**

**Series A Investors**

**TVM V Life Science Ventures GmbH & Co. KG**

c/o TVM Capital  
Maximilianstr. 35  
Entrance C  
80539 Munich, Germany  
Attn: Josef Moosholzer  
Fax: 49-89-998-992-55

With copies to:

c/o TVM Capital  
470 Atlantic Ave, 4th floor  
Boston, MA 02210  
Attn: Mark G. Cipriano  
Fax: 617-345-9377

**Cooperative AAC LS U.A.**

c/o Forbion Capital Partners  
PO Box 5187  
1410 AD Naarden  
The Netherlands  
Attn: Geert-Jan Mulder  
Fax: 31.35.699.3001

**Easton Hunt Capital Partners, L.P.**

767 Third Avenue, 7<sup>th</sup> Floor  
New York, NY 10017  
Attn: John Friedman  
Fax: 212.702.0852

**Series B Investors**

**Third Rock Ventures, L.P.**

29 Newbury Street  
Boston, MA 02116  
Attn.: Kevin Starr

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**TVM V Life Science Ventures GmbH & Co. KG**

c/o TVM Capital  
Maximilianstr. 35  
Entrance C  
80539 Munich, Germany  
Attn: Josef Moosholzer  
Fax: 49-89-998-992-55

With copies to:

c/o TVM Capital  
470 Atlantic Ave, 4th floor  
Boston, MA 02210  
Attn: Mark G. Cipriano  
Fax: 617-345-9377

**Cooperative AAC LS U.A.**

c/o Forbion Capital Partners  
PO Box 5187  
1410 AD Naarden  
The Netherlands  
Attn: Geert-Jan Mulder  
Fax: 31.35.699.3001

**Easton Hunt Capital Partners, L.P.**

767 Third Avenue, 7<sup>th</sup> Floor  
New York, NY 10017  
Attn: John Friedman  
Fax: 212.702.0852

**Genzyme Corporation**

500 Kendall Street  
Cambridge, Massachusetts 02142  
Attn: Alan Walts

**Series C Investors**

**ARCH Venture Fund VII, L.P.**

c/o ARCH Venture Partners  
8725 West Higgins Road  
Suite 290  
Chicago, IL 60631  
Attn: Mark McDonnell  
Fax: 773 380 6606  
Email: [mmcdonnell@archventure.com](mailto:mmcdonnell@archventure.com)

Copy to:

ARCH Venture Partners  
1000 Second Avenue  
Suite 3700  
Seattle, WA 98104  
Attn: Steve Gillis, Ph. D.  
Fax: 206-674-3026  
Email: [sgillis@archventure.com](mailto:sgillis@archventure.com)



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With a copy (which shall not constitute notice) to:

Proskauer Rose LLP  
One International Place  
Boston, MA 02110  
Attn: Ori Solomon  
Fax: 617-526-9899  
Email: osolomon@proskauer.com

**Third Rock Ventures, L.P.**

29 Newbury Street  
Boston, MA 02116  
Attn.: Kevin Starr

**TVM V Life Science Ventures GmbH & Co. KG**

c/o TVM Capital  
Maximilianstr. 35  
Entrance C  
80539 Munich, Germany  
Attn: Josef Moosholzer  
Fax: 49-89-998-992-55

With copies to:

c/o TVM Capital  
470 Atlantic Ave, 4th floor  
Boston, MA 02210  
Attn: Mark G. Cipriano  
Fax: 617-345-9377

**Cooperative AAC LS U.A.**

c/o Forbion Capital Partners  
PO Box 5187  
1410 AD Naarden  
The Netherlands  
Attn: Geert-Jan Mulder  
Fax: 31.35.699.3001

**Easton Hunt Capital Partners, L.P.**

767 Third Avenue, 7<sup>th</sup> Floor  
New York, NY 10017  
Attn: John Friedman  
Fax: 212.702.0852

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**Series D Investors**

**ARCH Venture Fund VII, L.P.**

c/o ARCH Venture Partners  
8725 West Higgins Road  
Suite 290  
Chicago, IL 60631  
Attn: Mark McDonnell  
Fax: 773 380 6606  
Email: mmcdonnell@archventure.com

Copy to:

ARCH Venture Partners  
1000 Second Avenue  
Suite 3700  
Seattle, WA 98104  
Attn: Steve Gillis, Ph. D.  
Fax: 206-674-3026  
Email: sgillis@archventure.com

**Third Rock Ventures, L.P.**

29 Newbury Street  
Boston, MA 02116  
Attn.: Kevin Starr

**TVM V Life Science Ventures GmbH & Co. KG**

c/o TVM Capital  
Maximilianstr. 35  
Entrance C  
80539 Munich, Germany  
Attn: Josef Moosholzer  
Fax: 49-89-998-992-55

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Boston, MA 02210  
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c/o Forbion Capital Partners  
PO Box 5187  
1410 AD Naarden  
The Netherlands  
Attn: Geert-Jan Mulder  
Fax: 31.35.699.3001

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**Mag & Co fbo Fidelity Contrafund: Fidelity Contrafund**

c/o Fidelity Investments  
82 Devonshire Street, V13H  
Boston, MA 02109  
Attn: Andrew Boyd

**Mag & Co fbo Fidelity Contrafund: Fidelity Advisor New Insights Fund**

c/o Fidelity Investments  
82 Devonshire Street, V13H  
Boston, MA 02109  
Attn: Andrew Boyd

**Ball & Co fbo Fidelity Mt. Vernon Street Trust: Fidelity Growth Company Fund**

c/o Fidelity Investments  
82 Devonshire Street, V13H  
Boston, MA 02109  
Attn: Andrew Boyd

**Mag & Co fbo Fidelity Select Portfolios: Biotechnology Portfolio**

c/o Fidelity Investments  
82 Devonshire Street, V13H  
Boston, MA 02109  
Attn: Andrew Boyd

**Bangle & Co fbo Fidelity Advisor Series VII: Fidelity Advisor Biotechnology Fund**

c/o Fidelity Investments  
82 Devonshire Street, V13H  
Boston, MA 02109  
Attn: Andrew Boyd

**SAILBOAT & CO. fbo Fidelity Magellan Fund: Fidelity Magellan Fund**

c/o Fidelity Investments  
82 Devonshire Street, V13H  
Boston, MA 02109  
Attn: Andrew Boyd

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**Shire LLC**

9200 Brookfield Court  
Florence, Kentucky 41042

**SMALLCAP World Fund, Inc.**

333 S. Hope Street, 55th Floor  
Los Angeles, CA 90017  
Attn: Michael Triessl and Don Rolfe  
Email: mcjt@capgroup.com;  
dhr@capgroup.com  
Fax: 213-615-0430

**American Funds Insurance Series Global Small Capitalization Fund**

333 S. Hope Street, 55th Floor  
Los Angeles, CA 90017  
Attn: Michael Triessl and Don Rolfe  
Email: mcjt@capgroup.com;  
dhr@capgroup.com  
Fax: 213-615-0430

**Deerfield Special Situations Fund, L.P.**

780 Third Avenue, 37th Floor  
New York, New York 10017  
Attn: Howard Furst

**Deerfield Special Situations International Master Fund, L.P.**

780 Third Avenue, 37th Floor  
New York, New York 10017  
Attn: Howard Furst

**Cowen Bluebird LLC**

c/o RAMIUS  
599 Lexington Ave  
New York, NY 10022  
Tel: 212.201.4860  
Fax: 212.845.7999  
Attn: Andrew Cohen | Managing Director  
Email: acohen@ramius.com

**RA Capital Healthcare Fund, LP**

c/o RA Capital Management, LLC  
20 Park Plaza, Suite 1200  
Boston, MA 02116  
f: 617 778 2510

**AMENDMENT TO THE GENETIX PHARMACEUTICALS INC.  
2002 EMPLOYEE, DIRECTOR AND CONSULTANT STOCK PLAN**

This Amendment to the Genetix Pharmaceuticals Inc. 2002 Employee, Director and Consultant Stock Plan, attached hereto as Exhibit A, (the "Plan") is effective as of March 5, 2010.

1. Section 3(a) of the Plan is hereby amended by deleting the first sentence thereof and replacing such sentence with the following:

"The number of Shares which may be issued from time to time pursuant to this Plan shall be 33,520,333, or the equivalent of such number of Shares after the Administrator, in its sole discretion, has interpreted the effect of any stock split, stock dividend, combination, recapitalization or similar transaction in accordance with Paragraph 23 of the Plan."

2. Section 3(b) of the Plan is hereby deleted and replaced in its entirety with the following:

"If an Option ceases to be "outstanding", in whole or in part (other than by exercise), or if the Company shall reacquire (at not more than its original issuance price) any Shares issued pursuant to a Stock Grant or Stock-Based Award, or if any Stock Right expires or is forfeited, cancelled, or otherwise terminated or results in any Shares not being issued, the unissued or reacquired Shares which were subject to such Stock Right shall again be available for issuance from time to time pursuant to this Plan."

3. Except as expressly amended hereby, the Plan shall remain in full force and effect.

IN WITNESS WHEREOF, the President and Chief Executive Officer of the Company has duly executed this Amendment to be effective as the date first above written.

GENETIX PHARMACEUTICALS INC.

By: /s/ Alfred Slanetz

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Alfred Slanetz

President and Chief Executive Officer

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**Exhibit A**

**GENETIX PHARMACEUTICALS INC.  
2002 EMPLOYEE, DIRECTOR AND CONSULTANT STOCK PLAN**

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As adopted by the Board of Directors on April 19, 2002  
As approved by the shareholders on April 19, 2002  
As amended by the Board of Directors on May 11, 2004  
As approved by the shareholders on May 11, 2004  
As amended by the Board of Directors on June 2, 2004  
As approved by the shareholders on June 2, 2004

**GENETIX PHARMACEUTICALS INC.**  
**SECOND AMENDED AND RESTATED**  
**2002 EMPLOYEE, DIRECTOR AND CONSULTANT STOCK PLAN**

1. DEFINITIONS.

Unless otherwise specified or unless the context otherwise requires, the following terms, as used in this Genetix Pharmaceuticals Inc. 2002 Employee, Director and Consultant Stock Plan, have the following meanings:

Administrator means the Board of Directors, unless it has delegated power to act on its behalf to the Committee, in which case the Administrator means the Committee.

Affiliate means a corporation which, for purposes of Section 424 of the Code, is a parent or subsidiary of the Company, direct or indirect.

Board of Directors means the Board of Directors of the Company.

Code means the United States Internal Revenue Code of 1986, as amended.

Committee means the committee of the Board of Directors to which the Board of Directors has delegated power to act under or pursuant to the provisions of the Plan.

Common Stock means shares of the Company's common stock, \$.001 par value per share.

Company means Genetix Pharmaceuticals Inc., a Delaware corporation.

Disability or Disabled means permanent and total disability as defined in Section 22(e)(3) of the Code.

Employee means any employee of the Company or of an Affiliate (including, without limitation, an employee who is also serving as an officer or director of the Company or of an Affiliate), designated by the Administrator to be eligible to be granted one or more Stock Rights under the Plan.

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Fair Market Value of a Share of Common Stock means:

- (1) If the Common Stock is listed on a national securities exchange or traded in the over-the-counter market and sales prices are regularly reported for the Common Stock, the closing or last price of the Common Stock on the Composite Tape or other comparable reporting system for the trading day immediately preceding the applicable date;
- (2) If the Common Stock is not traded on a national securities exchange but is traded on the over-the-counter market, if sales prices are not regularly reported for the Common Stock for the trading day referred to in clause (1), and if bid and asked prices for the Common Stock are regularly reported, the mean between the bid and the asked price for the Common Stock at the close of trading in the over-the-counter market for the trading day on which Common Stock was traded immediately preceding the applicable date; and
- (3) If the Common Stock is neither listed on a national securities exchange nor traded in the over-the-counter market, such value as the Administrator, in good faith, shall determine.

ISO means an option meant to qualify as an incentive stock option under Section 422 of the Code.

Non-Qualified Option means an option which is not intended to qualify as an ISO.

Option means an ISO or Non-Qualified Option granted under the Plan.

Option Agreement means an agreement between the Company and a Participant delivered pursuant to the Plan, in such form as the Administrator shall approve.

Participant means an Employee, director or consultant of the Company or an Affiliate to whom one or more Stock Rights are granted under the Plan. As used herein, "Participant" shall include "Participant's Survivors" where the context requires.

Plan means this Genetix Pharmaceuticals Inc. 2002 Employee, Director and Consultant Stock Plan.

Shares means shares of the Common Stock as to which Stock Rights have been or may be granted under the Plan or any shares of capital stock into which the Shares are changed or for which they are exchanged within the provisions of Paragraph 3 of the Plan. The Shares issued under the Plan may be authorized and unissued shares or shares held by the Company in its treasury, or both.

Stock Grant means a grant by the Company of Shares under the Plan.



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Stock Grant Agreement means an agreement between the Company and a Participant delivered pursuant to the Plan, in such form as the Administrator shall approve.

Stock Right means a right to Shares of the Company granted pursuant to the Plan — an ISO, a Non-Qualified Option or a Stock Grant.

Survivor means a deceased Participant's legal representatives and/or any person or persons who acquired the Participant's rights to a Stock Right by will or by the laws of descent and distribution.

2. PURPOSES OF THE PLAN.

The Plan is intended to encourage ownership of Shares by Employees and directors of and certain consultants to the Company in order to attract such people, to induce them to work for the benefit of the Company or of an Affiliate and to provide additional incentive for them to promote the success of the Company or of an Affiliate. The Plan provides for the granting of ISOs, Non-Qualified Options and Stock Grants.

3. SHARES SUBJECT TO THE PLAN.

(a) The number of Shares which may be issued from time to time pursuant to this Plan shall be 4,446,834, or the equivalent of such number of Shares after the Administrator, in its sole discretion, has interpreted the effect of any stock split, stock dividend, combination, recapitalization or similar transaction in accordance with Paragraph 23 of the Plan.

(b) If an Option ceases to be "outstanding", in whole or in part, or if the Company shall reacquire any Shares issued pursuant to a Stock Grant, the Shares which were subject to such Option and any Shares so reacquired by the Company shall be available for the granting of other Stock Rights under the Plan. Any Option shall be treated as "outstanding" until such Option is exercised in full, or terminates or expires under the provisions of the Plan, or by agreement of the parties to the pertinent Option Agreement.

4. ADMINISTRATION OF THE PLAN.

The Administrator of the Plan will be the Board of Directors, except to the extent the Board of Directors delegates its authority to the Committee, in which case the Committee shall be the Administrator. Subject to the provisions of the Plan, the Administrator is authorized to:

- a. Interpret the provisions of the Plan or of any Option or Stock Grant and to make all rules and determinations which it deems necessary or advisable for the administration of the Plan;
- b. Determine which Employees, directors and consultants shall be granted Stock Rights;
- c. Determine the number of Shares for which a Stock Right or Stock Rights shall be granted;

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- d. Specify the terms and conditions upon which a Stock Right or Stock Rights may be granted; and
  - e. Adopt any sub-plans applicable to residents of any specified jurisdiction as it deems necessary or appropriate in order to comply with or take advantage of any tax laws applicable to the Company or to Plan Participants or to otherwise facilitate the administration of the Plan, which sub-plans may include additional restrictions or conditions applicable to Options or Shares acquired upon exercise of Options.

provided, however, that all such interpretations, rules, determinations, terms and conditions shall be made and prescribed in the context of preserving the tax status under Section 422 of the Code of those Options which are designated as ISOs. Subject to the foregoing, the interpretation and construction by the Administrator of any provisions of the Plan or of any Stock Right granted under it shall be final, unless otherwise determined by the Board of Directors, if the Administrator is the Committee. In addition, if the Administrator is the Committee, the Board of Directors may take any action under the Plan that would otherwise be the responsibility of the Committee.

If permissible under applicable law, the Board of Directors or the Committee may allocate all or any portion of its responsibilities and powers to any one or more of its members and may delegate all or any portion of its responsibilities and powers to any other person selected by it. Any such allocation or delegation may be revoked by the Board of Directors or the Committee at any time.

#### 5. ELIGIBILITY FOR PARTICIPATION.

The Administrator will, in its sole discretion, name the Participants in the Plan, provided, however, that each Participant must be an Employee, director or consultant of the Company or of an Affiliate at the time a Stock Right is granted. Notwithstanding the foregoing, the Administrator may authorize the grant of a Stock Right to a person not then an Employee, director or consultant of the Company or of an Affiliate; provided, however, that the actual grant of such Stock Right shall be conditioned upon such person becoming eligible to become a Participant at or prior to the time of the execution of the Agreement evidencing such Stock Right. ISOs may be granted only to Employees. Non-Qualified Options and Stock Grants may be granted to any Employee, director or consultant of the Company or an Affiliate. The granting of any Stock Right to any individual shall neither entitle that individual to, nor disqualify him or her from, participation in any other grant of Stock Rights.

#### 6. TERMS AND CONDITIONS OF OPTIONS.

Each Option shall be set forth in writing in an Option Agreement, duly executed by the Company and, to the extent required by law or requested by the Company, by the Participant. The Administrator may provide that Options be granted subject to such terms and conditions, consistent with the terms and conditions specifically required under this Plan, as the Administrator may deem appropriate including, without limitation, subsequent approval by the shareholders of the Company of this Plan or any amendments thereto. The Option Agreements, which may differ among recipients, shall be subject to at least the following terms and conditions:

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- A. Non-Qualified Options: Each Option intended to be a Non-Qualified Option shall be subject to the terms and conditions which the Administrator determines to be appropriate and in the best interest of the Company, subject to the following minimum standards for any such Non-Qualified Option:
- a. Option Price: Each Option Agreement shall state the option price (per share) of the Shares covered by each Option, which option price shall be determined by the Administrator but shall not be less than the par value per share of Common Stock;
  - b. Each Option Agreement shall state the number of Shares to which it pertains;
  - c. Each Option Agreement shall state the date or dates on which it first is exercisable and the date after which it may no longer be exercised, and may provide that the Option rights accrue or become exercisable in installments over a period of months or years, or upon the occurrence of certain conditions or the attainment of stated goals or events; and
  - d. Exercise of any Option may be conditioned upon the Participant's execution of a Share purchase agreement in form satisfactory to the Administrator providing for certain protections for the Company and its other shareholders, including requirements that:
    - i. The Participant's or the Participant's Survivors' right to sell or transfer the Shares may be restricted; and
    - ii. The Participant or the Participant's Survivors may be required to execute letters of investment intent and must also acknowledge that the Shares will bear legends noting any applicable restrictions.
- B. ISOs: Each Option intended to be an ISO shall be issued only to an Employee and be subject to the following terms and conditions, with such additional restrictions or changes as the Administrator determines are appropriate but not in conflict with Section 422 of the Code and relevant regulations and rulings of the Internal Revenue Service:
- a. Minimum standards: The ISO shall meet the minimum standards required of Non-Qualified Options, as described in Paragraph 6(A) above, except clause (a) thereunder.

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- b. Option Price: Immediately before the ISO is granted, if the Participant owns, directly or by reason of the applicable attribution rules in Section 424(d) of the Code:
    - i. 10% or less of the total combined voting power of all classes of stock of the Company or an Affiliate, the Option price per share of the Shares covered by each ISO shall not be less than 100% of the Fair Market Value per share of the Shares on the date of the grant of the Option; or
    - ii. More than 10% of the total combined voting power of all classes of stock of the Company or an Affiliate, the Option price per share of the Shares covered by each ISO shall not be less than 110% of the said Fair Market Value on the date of grant.
  - c. Term of Option: For Participants who own:
    - i. 10% or less of the total combined voting power of all classes of stock of the Company or an Affiliate, each ISO shall terminate not more than ten years from the date of the grant or at such earlier time as the Option Agreement may provide; or
    - ii. More than 10% of the total combined voting power of all classes of stock of the Company or an Affiliate, each ISO shall terminate not more than five years from the date of the grant or at such earlier time as the Option Agreement may provide.
  - d. Limitation on Yearly Exercise: The Option Agreements shall restrict the amount of ISOs which may become exercisable in any calendar year (under this or any other ISO plan of the Company or an Affiliate) so that the aggregate Fair Market Value (determined at the time each ISO is granted) of the stock with respect to which ISOs are exercisable for the first time by the Participant in any calendar year does not exceed \$100,000.

7. TERMS AND CONDITIONS OF STOCK GRANTS.

Each offer of a Stock Grant to a Participant shall state the date prior to which the Stock Grant must be accepted by the Participant, and the principal terms of each Stock Grant shall be set forth in a Stock Grant Agreement, duly executed by the Company and, to the extent required by law or requested by the Company, by the Participant. The Stock Grant Agreement shall be in a form approved by the Administrator and shall contain terms and conditions which the Administrator determines to be appropriate and in the best interest of the Company, subject to the following minimum standards:

- (a) Each Stock Grant Agreement shall state the purchase price (per share), if any, of the Shares covered by each Stock Grant, which purchase price shall be determined by the Administrator but shall not be less than the minimum consideration required by the Delaware General Corporation Law on the date of the grant of the Stock Grant;

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- (b) Each Stock Grant Agreement shall state the number of Shares to which the Stock Grant pertains; and
  - (c) Each Stock Grant Agreement shall include the terms of any right of the Company to restrict or reacquire the Shares subject to the Stock Grant, including the time and events upon which such reacquisition rights shall accrue and the purchase price therefor, if any.

8. EXERCISE OF OPTIONS AND ISSUE OF SHARES.

An Option (or any part or installment thereof) shall be exercised by giving written notice to the Company or its designee, together with provision for payment of the full purchase price in accordance with this Paragraph for the Shares as to which the Option is being exercised, and upon compliance with any other condition(s) set forth in the Option Agreement. Such notice shall be signed by the person exercising the Option, shall state the number of Shares with respect to which the Option is being exercised and shall contain any representation required by the Plan or the Option Agreement. Payment of the purchase price for the Shares as to which such Option is being exercised shall be made (a) in United States dollars in cash or by check, or (b) at the discretion of the Administrator, through delivery of shares of Common Stock having a Fair Market Value equal as of the date of the exercise to the cash exercise price of the Option and held for at least six months, or (c) at the discretion of the Administrator, by delivery of the grantee's personal note, for full, partial or no recourse, bearing interest payable not less than annually at market rate on the date of exercise and at no less than 100% of the applicable Federal rate, as defined in Section 1274(d) of the Code, with or without the pledge of such Shares as collateral, or (d) at the discretion of the Administrator, in accordance with a cashless exercise program established with a securities brokerage firm, and approved by the Administrator, or (e) at the discretion of the Administrator, by any combination of (a), (b), (c) and (d) above. Notwithstanding the foregoing, the Administrator shall accept only such payment on exercise of an ISO as is permitted by Section 422 of the Code.

The Company shall then reasonably promptly deliver the Shares as to which such Option was exercised to the Participant (or to the Participant's Survivors, as the case may be). In determining what constitutes "reasonably promptly," it is expressly understood that the issuance and delivery of the Shares may be delayed by the Company in order to comply with any law or regulation (including, without limitation, state securities or "blue sky" laws) which requires the Company to take any action with respect to the Shares prior to their issuance. The Shares shall, upon delivery, be fully paid, non-assessable Shares.

The Administrator shall have the right to accelerate the date of exercise of any installment of any Option; provided that the Administrator shall not accelerate the exercise date of any installment of any Option granted to an Employee as an ISO (and not previously converted into a Non-Qualified Option pursuant to Paragraph 26) if such acceleration would violate the annual vesting limitation contained in Section 422(d) of the Code, as described in Paragraph 6.B.d.

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The Administrator may, in its discretion, amend any term or condition of an outstanding Option provided (i) such term or condition as amended is permitted by the Plan, (ii) any such amendment shall be made only with the consent of the Participant to whom the Option was granted, or in the event of the death of the Participant, the Participant's Survivors, if the amendment is adverse to the Participant, and (iii) any such amendment of any ISO shall be made only after the Administrator determines whether such amendment would constitute a "modification" of any Option which is an ISO (as that term is defined in Section 424(h) of the Code) or would cause any adverse tax consequences for the holder of such ISO.

9. ACCEPTANCE OF STOCK GRANT AND ISSUE OF SHARES.

A Stock Grant (or any part or installment thereof) shall be accepted by executing the Stock Grant Agreement and delivering it to the Company or its designee, together with provision for payment of the full purchase price, if any, in accordance with this Paragraph for the Shares as to which such Stock Grant is being accepted, and upon compliance with any other conditions set forth in the Stock Grant Agreement. Payment of the purchase price for the Shares as to which such Stock Grant is being accepted shall be made (a) in United States dollars in cash or by check, or (b) at the discretion of the Administrator, through delivery of shares of Common Stock held for at least six months and having a Fair Market Value equal as of the date of acceptance of the Stock Grant to the purchase price of the Stock Grant, or (c) at the discretion of the Administrator, by delivery of the grantee's personal note, for full or partial recourse as determined by the Administrator, bearing interest payable not less than annually at no less than 100% of the applicable Federal rate, as defined in Section 1274(d) of the Code, or (d) at the discretion of the Administrator, by any combination of (a), (b) and (c) above.

The Company shall then reasonably promptly deliver the Shares as to which such Stock Grant was accepted to the Participant (or to the Participant's Survivors, as the case may be), subject to any escrow provision set forth in the Stock Grant Agreement. In determining what constitutes "reasonably promptly," it is expressly understood that the issuance and delivery of the Shares may be delayed by the Company in order to comply with any law or regulation (including, without limitation, state securities or "blue sky" laws) which requires the Company to take any action with respect to the Shares prior to their issuance.

The Administrator may, in its discretion, amend any term or condition of an outstanding Stock Grant or Stock Grant Agreement provided (i) such term or condition as amended is permitted by the Plan, and (ii) any such amendment shall be made only with the consent of the Participant to whom the Stock Grant was made, if the amendment is adverse to the Participant.

10. RIGHTS AS A SHAREHOLDER.

No Participant to whom a Stock Right has been granted shall have rights as a shareholder with respect to any Shares covered by such Stock Right, except after due exercise of the Option or acceptance of the Stock Grant and tender of the full purchase price, if any, for the Shares being purchased pursuant to such exercise or acceptance and registration of the Shares in the Company's share register in the name of the Participant.

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11. ASSIGNABILITY AND TRANSFERABILITY OF STOCK RIGHTS.

By its terms, a Stock Right granted to a Participant shall not be transferable by the Participant other than (i) by will or by the laws of descent and distribution, or (ii) as approved by the Administrator in its discretion and set forth in the applicable Option Agreement or Stock Grant Agreement. Notwithstanding the foregoing, an ISO transferred except in compliance with clause (i) above shall no longer qualify as an ISO. The designation of a beneficiary of a Stock Right by a Participant, with the prior approval of the Administrator and in such form as the Administrator shall prescribe, shall not be deemed a transfer prohibited by this Paragraph. Except as provided above, a Stock Right shall only be exercisable or may only be accepted, during the Participant's lifetime, only by such Participant (or by his or her legal representative) and shall not be assigned, pledged or hypothecated in any way (whether by operation of law or otherwise) and shall not be subject to execution, attachment or similar process. Any attempted transfer, assignment, pledge, hypothecation or other disposition of any Stock Right or of any rights granted thereunder contrary to the provisions of this Plan, or the levy of any attachment or similar process upon a Stock Right, shall be null and void.

12. EFFECT ON OPTIONS OF TERMINATION OF SERVICE OTHER THAN "FOR CAUSE" OR DEATH OR DISABILITY.

Except as otherwise provided in a Participant's Option Agreement, in the event of a termination of service (whether as an employee, director or consultant) with the Company or an Affiliate before the Participant has exercised an Option, the following rules apply:

- a. A Participant who ceases to be an employee, director or consultant of the Company or of an Affiliate (for any reason other than termination "for cause". Disability, or death for which events there are special rules in Paragraphs 13, 14, and 15, respectively), may exercise any Option granted to him or her to the extent that the Option is exercisable on the date of such termination of service, but only within such term as the Administrator has designated in a Participant's Option Agreement.
- b. Except as provided in Subparagraph (c) below, or Paragraph 14 or 15, in no event may an Option intended to be an ISO, be exercised later than three months after the Participant's termination of employment.
- c. The provisions of this Paragraph, and not the provisions of Paragraph 14 or 15, shall apply to a Participant who subsequently becomes Disabled or dies after the termination of employment, director status or consultancy, provided, however, in the case of a Participant's Disability or death within three months after the termination of employment, director status or consultancy, the Participant or the Participant's Survivors may exercise the Option within one year after the date of the Participant's termination of service, but in no event after the date of expiration of the term of the Option.
- d. Notwithstanding anything herein to the contrary, if subsequent to a Participant's termination of employment, termination of director status or termination of consultancy, but prior to the exercise of an Option, the Board of Directors determines that, either prior or subsequent to the Participant's termination, the Participant engaged in conduct which would constitute "cause", then such Participant shall forthwith cease to have any right to exercise any Option.

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- e. A Participant to whom an Option has been granted under the Plan who is absent from work with the Company or with an Affiliate because of temporary disability (any disability other than a permanent and total Disability as defined in Paragraph 1 hereof), or who is on leave of absence for any purpose, shall not, during the period of any such absence, be deemed, by virtue of such absence alone, to have terminated such Participant's employment, director status or consultancy with the Company or with an Affiliate, except as the Administrator may otherwise expressly provide.
  - f. Except as required by law or as set forth in a Participant's Option Agreement, Options granted under the Plan shall not be affected by any change of a Participant's status within or among the Company and any Affiliates, so long as the Participant continues to be an employee, director or consultant of the Company or any Affiliate.

13. EFFECT ON OPTIONS OF TERMINATION OF SERVICE "FOR CAUSE".

Except as otherwise provided in a Participant's Option Agreement, the following rules apply if the Participant's service (whether as an employee, director or consultant) with the Company or an Affiliate is terminated "for cause" prior to the time that all his or her outstanding Options have been exercised:

- a. All outstanding and unexercised Options as of the time the Participant is notified his or her service is terminated "for cause" will immediately be forfeited.
- b. For purposes of this Plan, "cause" shall include (and is not limited to) dishonesty with respect to the Company or any Affiliate, insubordination, substantial malfeasance or non-feasance of duty, unauthorized disclosure of confidential information, breach by the Participant of any provision of any employment, consulting, advisory, nondisclosure, non-competition or similar agreement between the Participant and the Company, and conduct substantially prejudicial to the business of the Company or any Affiliate. The determination of the Administrator as to the existence of "cause" will be conclusive on the Participant and the Company.
- c. "Cause" is not limited to events which have occurred prior to a Participant's termination of service, nor is it necessary that the Administrator's finding of "cause" occur prior to termination. If the Administrator determines, subsequent to a Participant's termination of service but prior to the exercise of an Option, that either prior or subsequent to the Participant's termination the Participant engaged in conduct which would constitute "cause", then the right to exercise any Option is forfeited.
- d. Any definition in an agreement between the Participant and the Company or an Affiliate, which contains a conflicting definition of "cause" for termination and which is in effect at the time of such termination, shall supersede the definition in this Plan with respect to that Participant.



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14. EFFECT ON OPTIONS OF TERMINATION OF SERVICE FOR DISABILITY.

Except as otherwise provided in a Participant's Option Agreement, a Participant who ceases to be an employee, director or consultant of the Company or of an Affiliate by reason of Disability may exercise any Option granted to such Participant:

- a. To the extent that the Option has become exercisable but has not been exercised on the date of Disability; and
- b. In the event rights to exercise the Option accrue periodically, to the extent of a pro rata portion through the date of Disability of any additional vesting rights that would have accrued on the next vesting date had the Participant not become Disabled. The proration shall be based upon the number of days accrued in the current vesting period prior to the date of Disability.

A Disabled Participant may exercise such rights only within the period ending one year after the date of the Participant's termination of employment, directorship or consultancy, as the case may be, notwithstanding that the Participant might have been able to exercise the Option as to some or all of the Shares on a later date if the Participant had not become Disabled and had continued to be an employee, director or consultant or, if earlier, within the originally prescribed term of the Option.

The Administrator shall make the determination both of whether Disability has occurred and the date of its occurrence (unless a procedure for such determination is set forth in another agreement between the Company and such Participant, in which case such procedure shall be used for such determination). If requested, the Participant shall be examined by a physician selected or approved by the Administrator, the cost of which examination shall be paid for by the Company.

15. EFFECT ON OPTIONS OF DEATH WHILE AN EMPLOYEE, DIRECTOR OR CONSULTANT.

Except as otherwise provided in a Participant's Option Agreement, in the event of the death of a Participant while the Participant is an employee, director or consultant of the Company or of an Affiliate, such Option may be exercised by the Participant's Survivors:

- a. To the extent that the Option has become exercisable but has not been exercised on the date of death; and
- b. In the event rights to exercise the Option accrue periodically, to the extent of a pro rata portion through the date of death of any additional vesting rights that would have accrued on the next vesting date had the Participant not died. The proration shall be based upon the number of days accrued in the current vesting period prior to the Participant's date of death.

If the Participant's Survivors wish to exercise the Option, they must take all necessary steps to exercise the Option within one year after the date of death of such Participant, notwithstanding that the decedent might have been able to exercise the Option as to some or all of the Shares on a later date if he or she had not died and had continued to be an employee, director or consultant or, if earlier, within the originally prescribed term of the Option.

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16. EFFECT OF TERMINATION OF SERVICE ON STOCK GRANTS.

In the event of a termination of service (whether as an employee, director or consultant) with the Company or an Affiliate for any reason before the Participant has accepted a Stock Grant, such offer shall terminate.

For purposes of this Paragraph 16 and Paragraph 17 below, a Participant to whom a Stock Grant has been offered and accepted under the Plan who is absent from work with the Company or with an Affiliate because of temporary disability (any disability other than a permanent and total Disability as defined in Paragraph 1 hereof), or who is on leave of absence for any purpose, shall not, during the period of any such absence, be deemed, by virtue of such absence alone, to have terminated such Participant's employment, director status or consultancy with the Company or with an Affiliate, except as the Administrator may otherwise expressly provide.

In addition, for purposes of this Paragraph 16 and Paragraph 17 below, any change of employment or other service within or among the Company and any Affiliates shall not be treated as a termination of employment, director status or consultancy so long as the Participant continues to be an employee, director or consultant of the Company or any Affiliate.

17. EFFECT ON STOCK GRANTS OF TERMINATION OF SERVICE OTHER THAN "FOR CAUSE" OR DEATH OR DISABILITY.

Except as otherwise provided in a Participant's Stock Grant Agreement, in the event of a termination of service (whether as an employee, director or consultant), other than termination "for cause," Disability, or death for which events there are special rules in Paragraphs 18, 19, and 20, respectively, before all Company rights of repurchase shall have lapsed, then the Company shall have the right to repurchase that number of Shares subject to a Stock Grant as to which the Company's repurchase rights have not lapsed.

18. EFFECT ON STOCK GRANTS OF TERMINATION OF SERVICE "FOR CAUSE".

Except as otherwise provided in a Participant's Stock Grant Agreement, the following rules apply if the Participant's service (whether as an employee, director or consultant) with the Company or an Affiliate is terminated "for cause":

- a. AH Shares subject to any Stock Grant shall be immediately subject to repurchase by the Company at the purchase price, if any, thereof.
- b. For purposes of this Plan, "cause" shall include (and is not limited to) dishonesty with respect to the employer, insubordination, substantial malfeasance or non-feasance of duty, unauthorized disclosure of confidential information, breach by the Participant of any provision of any employment, consulting, advisory, nondisclosure, non-competition or similar agreement between the Participant and the Company, and conduct substantially prejudicial to the business of the Company or any Affiliate. The determination of the Administrator as to the existence of "cause" will be conclusive on the Participant and the Company.

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- c. "Cause" is not limited to events which have occurred prior to a Participant's termination of service, nor is it necessary that the Administrator's finding of "cause" occur prior to termination. If the Administrator determines, subsequent to a Participant's termination of service, that either prior or subsequent to the Participant's termination the Participant engaged in conduct which would constitute "cause," then the Company's right to repurchase all of such Participant's Shares shall apply.
  - d. Any definition in an agreement between the Participant and the Company or an Affiliate, which contains a conflicting definition of "cause" for termination and which is in effect at the time of such termination, shall supersede the definition in this Plan with respect to that Participant.

19. EFFECT ON STOCK GRANTS OF TERMINATION OF SERVICE FOR DISABILITY.

Except as otherwise provided in a Participant's Stock Grant Agreement, the following rules apply if a Participant ceases to be an employee, director or consultant of the Company or of an Affiliate by reason of Disability: to the extent the Company's rights of repurchase have not lapsed on the date of Disability, they shall be exercisable; provided, however, that in the event such rights of repurchase lapse periodically, such rights shall lapse to the extent of a pro rata portion of the Shares subject to such Stock Grant through the date of Disability as would have lapsed had the Participant not become Disabled. The proration shall be based upon the number of days accrued prior to the date of Disability.

The Administrator shall make the determination both of whether Disability has occurred and the date of its occurrence (unless a procedure for such determination is set forth in another agreement between the Company and such Participant, in which case such procedure shall be used for such determination). If requested, the Participant shall be examined by a physician selected or approved by the Administrator, the cost of which examination shall be paid for by the Company.

20. EFFECT ON STOCK GRANTS OF DEATH WHILE AN EMPLOYEE, DIRECTOR OR CONSULTANT.

Except as otherwise provided in a Participant's Stock Grant Agreement, the following rules apply in the event of the death of a Participant while the Participant is an employee, director or consultant of the Company or of an Affiliate: to the extent the Company's rights of repurchase have not lapsed on the date of death, they shall be exercisable; provided, however, that in the event such rights of repurchase lapse periodically, such rights shall lapse to the extent of a pro rata portion of the Shares subject to such Stock Grant through the date of death as would have lapsed had the Participant not died. The proration shall be based upon the number of days accrued prior to the Participant's death.

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21. PURCHASE FOR INVESTMENT.

Unless the offering and sale of the Shares to be issued upon the particular exercise or acceptance of a Stock Right shall have been effectively registered under the Securities Act of 1933, as now in force or hereafter amended (the "1933 Act"), the Company shall be under no obligation to issue the Shares covered by such exercise unless and until the following conditions have been fulfilled:

- a. The person(s) who exercise(s) or accept(s) such Stock Right shall warrant to the Company, prior to the receipt of such Shares, that such person(s) are acquiring such Shares for their own respective accounts, for investment, and not with a view to, or for sale in connection with, the distribution of any such Shares, in which event the person(s) acquiring such Shares shall be bound by the provisions of the following legend which shall be endorsed upon the certificate(s) evidencing their Shares issued pursuant to such exercise or such grant:  
  
"The shares represented by this certificate have been taken for investment and they may not be sold or otherwise transferred by any person, including a pledgee, unless (1) either (a) a Registration Statement with respect to such shares shall be effective under the Securities Act of 1933, as amended, or (b) the Company shall have received an opinion of counsel satisfactory to it that an exemption from registration under such Act is then available, and (2) there shall have been compliance with all applicable state securities laws."
- b. At the discretion of the Administrator, the Company shall have received an opinion of its counsel that the Shares may be issued upon such particular exercise or acceptance in compliance with the 1933 Act without registration thereunder.

22. DISSOLUTION OR LIQUIDATION OF THE COMPANY.

Upon the dissolution or liquidation of the Company, all Options granted under this Plan which as of such date shall not have been exercised and all Stock Grants which have not been accepted will terminate and become null and void; provided, however, that if the rights of a Participant or a Participant's Survivors have not otherwise terminated and expired, the Participant or the Participant's Survivors will have the right immediately prior to such dissolution or liquidation to exercise or accept any Stock Right to the extent that the Stock Right is exercisable or subject to acceptance as of the date immediately prior to such dissolution or liquidation.

23. ADJUSTMENTS.

Upon the occurrence of any of the following events, a Participant's rights with respect to any Stock Right granted to him or her hereunder shall be adjusted as hereinafter provided, unless otherwise specifically provided in a Participant's Option Agreement or Stock Grant Agreement:

- A. Stock Dividends and Stock Splits. If (i) the shares of Common Stock shall be subdivided or combined into a greater or smaller number of shares or if the Company shall issue any shares of Common Stock as a stock dividend on its

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outstanding Common Stock, or (ii) additional shares or new or different shares or other securities of the Company or other non-cash assets are distributed with respect to such shares of Common Stock, the number of shares of Common Stock deliverable upon the exercise or acceptance of such Stock Right may be appropriately increased or decreased proportionately, and appropriate adjustments may be made including, in the purchase price per share, to reflect such events.

- B. Corporate Transactions. If the Company is to be consolidated with or acquired by another entity in a merger, sale of all or substantially all of the Company's assets other than a transaction to merely change the state of incorporation (a "Corporate Transaction"), the Administrator or the board of directors of any entity assuming the obligations of the Company hereunder (the "Successor Board"), shall, as to outstanding Options, either (i) make appropriate provision for the continuation of such Options by substituting on an equitable basis for the Shares then subject to such Options either the consideration payable with respect to the outstanding shares of Common Stock in connection with the Corporate Transaction or securities of any successor or acquiring entity; or (ii) upon written notice to the Participants, provide that all Options must be exercised (either to the extent then exercisable or, at the discretion of the Administrator, or, upon a change of control of the Company, all Options being made fully exercisable for purposes of this Subparagraph), within a specified number of days of the date of such notice, at the end of which period the Options shall terminate; or (iii) terminate all Options in exchange for a cash payment equal to the excess of the Fair Market Value of the Shares subject to such Options (either to the extent then exercisable or, at the discretion of the Administrator, all Options being made fully exercisable for purposes of this Subparagraph) over the exercise price thereof.

With respect to outstanding Stock Grants, the Administrator or the Successor Board, shall either (i) make appropriate provisions for the continuation of such Stock Grants by substituting on an equitable basis for the Shares then subject to such Stock Grants either the consideration payable with respect to the outstanding Shares of Common Stock in connection with the Corporate Transaction or securities of any successor or acquiring entity; or (ii) upon written notice to the Participants, provide that all Stock Grants must be accepted (to the extent then subject to acceptance) within a specified number of days of the date of such notice, at the end of which period the offer of the Stock Grants shall terminate; or (iii) terminate all Stock Grants in exchange for a cash payment equal to the excess of the Fair Market Value of the Shares subject to such Stock Grants over the purchase price thereof, if any. In addition, in the event of a Corporate Transaction, the Administrator may waive any or all Company repurchase rights with respect to outstanding Stock Grants.

- C. Recapitalization or Reorganization. In the event of a recapitalization or reorganization of the Company other than a Corporate Transaction pursuant to which securities of the Company or of another corporation are issued with respect to the outstanding shares of Common Stock, a Participant upon exercising or accepting a Stock Right after the recapitalization or reorganization shall be

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entitled to receive for the purchase price paid upon such exercise or acceptance the number of replacement securities which would have been received if such Stock Right had been exercised or accepted prior to such recapitalization or reorganization.

- D. Modification of ISOs. Notwithstanding the foregoing, any adjustments made pursuant to Subparagraph A, B or C above with respect to ISOs shall be made only after the Administrator determines whether such adjustments would constitute a “modification” of such ISOs (as that term is defined in Section 424(h) of the Code) or would cause any adverse tax consequences for the holders of such ISOs. If the Administrator determines that such adjustments made with respect to ISOs would constitute a modification of such ISOs, it may refrain from making such adjustments, unless the holder of an ISO specifically requests in writing that such adjustment be made and such writing indicates that the holder has full knowledge of the consequences of such “modification” on his or her income tax treatment with respect to the ISO.

24. ISSUANCES OF SECURITIES.

Except as expressly provided herein, no issuance by the Company of shares of stock of any class, or securities convertible into shares of stock of any class, shall affect, and no adjustment by reason thereof shall be made with respect to, the number or price of shares subject to Stock Rights. Except as expressly provided herein, no adjustments shall be made for dividends paid in cash or in property (including without limitation, securities) of the Company prior to any issuance of Shares pursuant to a Stock Right.

25. FRACTIONAL SHARES.

No fractional shares shall be issued under the Plan and the person exercising a Stock Right shall receive from the Company cash in lieu of such fractional shares equal to the Fair Market Value thereof.

26. CONVERSION OF ISOs INTO NON-QUALIFIED OPTIONS: TERMINATION OF ISOs.

The Administrator, at the written request of any Participant, may in its discretion take such actions as may be necessary to convert such Participant’s ISOs (or any portions thereof) that have not been exercised on the date of conversion into Non-Qualified Options at any time prior to the expiration of such ISOs, regardless of whether the Participant is an employee of the Company or an Affiliate at the time of such conversion. At the time of such conversion, the Administrator (with the consent of the Participant) may impose such conditions on the exercise of the resulting Non-Qualified Options as the Administrator in its discretion may determine, provided that such conditions shall not be inconsistent with this Plan. Nothing in the Plan shall be deemed to give any Participant the right to have such Participant’s ISOs converted into Non-Qualified Options, and no such conversion shall occur until and unless the Administrator takes appropriate action. The Administrator, with the consent of the Participant, may also terminate any portion of any ISO that has not been exercised at the time of such conversion.

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27. WITHHOLDING.

In the event that any federal, state, or local income taxes, employment taxes, Federal Insurance Contributions Act ("F.I.C.A.") withholdings or other amounts are required by applicable law or governmental regulation to be withheld from the Participant's salary, wages or other remuneration in connection with the exercise or acceptance of a Stock Right or in connection with a Disqualifying Disposition (as defined in Paragraph 28) or upon the lapsing of any right of repurchase, the Company may withhold from the Participant's compensation, if any, or may require that the Participant advance in cash to the Company, or to any Affiliate of the Company which employs or employed the Participant, the statutory minimum amount of such withholdings unless a different withholding arrangement, including the use of shares of the Company's Common Stock or a promissory note, is authorized by the Administrator (and permitted by law). For purposes hereof, the fair market value of the shares withheld for purposes of payroll withholding shall be determined in the manner provided in Paragraph 1 above, as of the most recent practicable date prior to the date of exercise. If the fair market value of the shares withheld is less than the amount of payroll withholdings required, the Participant may be required to advance the difference in cash to the Company or the Affiliate employer. The Administrator in its discretion may condition the exercise of an Option for less than the then Fair Market Value on the Participant's payment of such additional withholding.

28. NOTICE TO COMPANY OF DISQUALIFYING DISPOSITION.

Each Employee who receives an ISO must agree to notify the Company in writing immediately after the Employee makes a Disqualifying Disposition of any shares acquired pursuant to the exercise of an ISO. A Disqualifying Disposition is defined in Section 424(c) of the Code and includes any disposition (including any sale or gift) of such shares before the later of (a) two years after the date the Employee was granted the ISO, or (b) one year after the date the Employee acquired Shares by exercising the ISO, except as otherwise provided in Section 424(c) of the Code. If the Employee has died before such stock is sold, these holding period requirements do not apply and no Disqualifying Disposition can occur thereafter.

29. TERMINATION OF THE PLAN.

The Plan will terminate on April 19, 2012, the date which is ten years from the earlier of the date of its adoption by the Board of Directors and the date of its approval by the shareholders. The Plan may be terminated at an earlier date by vote of the shareholders or the Board of Directors of the Company; provided, however, that any such earlier termination shall not affect any Option Agreements or Stock Grant Agreements executed prior to the effective date of such termination.

30. AMENDMENT OF THE PLAN AND AGREEMENTS.

The Plan may be amended by the shareholders of the Company. The Plan may also be amended by the Administrator, including, without limitation, to the extent necessary to qualify any or all outstanding Stock Rights granted under the Plan or Stock Rights to be granted under the Plan for favorable federal income tax treatment (including deferral of taxation upon exercise) as may be afforded incentive stock options under Section 422 of the Code, and to the extent

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necessary to qualify the shares issuable upon exercise or acceptance of any outstanding Stock Rights granted, or Stock Rights to be granted, under the Plan for listing on any national securities exchange or quotation in any national automated quotation system of securities dealers. Any amendment approved by the Administrator which the Administrator determines is of a scope that requires shareholder approval shall be subject to obtaining such shareholder approval. Any modification or amendment of the Plan shall not, without the consent of a Participant, adversely affect his or her rights under a Stock Right previously granted to him or her. With the consent of the Participant affected, the Administrator may amend outstanding Option Agreements and Stock Grant Agreements in a manner which may be adverse to the Participant but which is not inconsistent with the Plan. In the discretion of the Administrator, outstanding Option Agreements and Stock Grant Agreements may be amended by the Administrator in a manner which is not adverse to the Participant.

31. EMPLOYMENT OR OTHER RELATIONSHIP.

Nothing in this Plan or any Option Agreement or Stock Grant Agreement shall be deemed to prevent the Company or an Affiliate from terminating the employment, consultancy or director status of a Participant, nor to prevent a Participant from terminating his or her own employment, consultancy or director status or to give any Participant a right to be retained in employment or other service by the Company or any Affiliate for any period of time.

32. GOVERNING LAW.

This Plan shall be construed and enforced in accordance with the law of the State of Delaware.



**NON-QUALIFIED STOCK OPTION AGREEMENT  
GENETIX PHARMACEUTICALS INC.**

AGREEMENT made as of the \_\_\_\_\_ day of \_\_\_\_\_, 20\_\_\_\_, between Genetix Pharmaceuticals Inc. (the "Company"), a Delaware corporation having a principal place of business in Cambridge, Massachusetts, and \_\_\_\_\_ of \_\_\_\_\_, (the "Participant"), for whom the Board of Directors has authorized that the Option granted herein shall begin vesting as of \_\_\_\_\_, 20\_\_\_\_ (the "Vesting Date").

WHEREAS, the Company desires to grant to the Participant an Option to purchase shares of its common stock, \$0.01 par value per share (the "Shares"), under and for the purposes set forth in the Company's 2002 Employee, Director and Consultant Stock Plan (the "Plan");

WHEREAS, the Company and the Participant understand and agree that any terms used and not defined herein have the same meanings as in the Plan; and

WHEREAS, the Company and the Participant each intend that the Option granted herein shall be a Non-Qualified Option.

NOW, THEREFORE, in consideration of the mutual covenants hereinafter set forth and for other good and valuable consideration, the parties hereto agree as follows:

1. GRANT OF OPTION.

The Company hereby grants to the Participant the right and option to purchase all or any part of an aggregate of \_\_\_\_\_ Shares, on the terms and conditions and subject to all the limitations set forth herein, under United States securities and tax laws, and in the Plan, which is incorporated herein by reference. The Participant acknowledges receipt of a copy of the Plan.

2. PURCHASE PRICE.

The purchase price of the Shares covered by the Option shall be \$ \_\_\_\_\_ per Share, subject to adjustment, as provided in the Plan, in the event of a stock split, reverse stock split or other events affecting the holders of Shares (the "Purchase Price"). Payment shall be made in accordance with Paragraph 8 of the Plan.

3. EXERCISABILITY OF OPTION.

Subject to the terms and conditions set forth in this Agreement and the Plan, the Option granted hereby shall become exercisable as follows:

On the first day of the first month following the Vesting Date	up to _____ Shares
On the first day of each month for _____ months thereafter	an additional _____ Shares

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The foregoing rights are cumulative and are subject to the other terms and conditions of this Agreement and the Plan.

4. TERM OF OPTION.

The Option shall terminate ten years from the date of this Agreement, but shall be subject to earlier termination as provided herein or in the Plan but shall be subject to earlier termination as provided herein or in the Plan.

If the Participant ceases to be an employee, director or consultant of the Company or of an Affiliate (for any reason other than the death or Disability of the Participant or termination of the Participant for "cause" (as defined in the Plan), the Option may be exercised, if it has not previously terminated, within three months after the date the Participant ceases to be an employee, director or consultant of the Company or an Affiliate, or within the originally prescribed term of the Option, whichever is earlier, but may not be exercised thereafter. In such event, the Option shall be exercisable only to the extent that the Option has become exercisable and is in effect at the date of such cessation of employment, directorship or consultancy.

Notwithstanding the foregoing, in the event of the Participant's Disability or death within three months after the termination of employment, directorship or consultancy, the Participant or the Participant's Survivors may exercise the Option within one year after the date of the Participant's termination of employment, directorship or consultancy, but in no event after the date of expiration of the term of the Option.

In the event the Participant's employment, directorship or consultancy is terminated by the Company or an Affiliate for "cause" (as defined in the Plan), the Participant's right to exercise any unexercised portion of this Option shall cease immediately as of the time the Participant is notified his or her employment, directorship or consultancy is terminated for "cause", and this Option shall thereupon terminate. Notwithstanding anything herein to the contrary, if subsequent to the Participant's termination, but prior to the exercise of the Option, the Board of Directors of the Company determines that, either prior or subsequent to the Participant's termination, the Participant engaged in conduct which would constitute "cause," then the Participant shall immediately cease to have any right to exercise the Option and this Option shall thereupon terminate.

In the event of the Disability of the Participant, as determined in accordance with the Plan, the Option shall be exercisable within one year after the Participant's termination of service or, if earlier, within the term originally prescribed by the Option. In such event, the Option shall be exercisable:

- (a) to the extent that the Option has become exercisable but has not been exercised as of the date of Disability; and
- (b) in the event rights to exercise the Option accrue periodically, to the extent of a pro rata portion through the date of Disability of any additional vesting rights that would have accrued on the next vesting date had the Participant not become Disabled. The proration shall be based upon the number of days accrued in the current vesting period prior to the date of Disability.

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In the event of the death of the Participant while an employee, director or consultant of the Company or of an Affiliate, the Option shall be exercisable by the Participant's Survivors within one year after the date of death of the Participant or, if earlier, within the originally prescribed term of the Option. In such event, the Option shall be exercisable:

- (x) to the extent that the Option has become exercisable but has not been exercised as of the date of death; and
- (y) in the event rights to exercise the Option accrue periodically, to the extent of a pro rata portion through the date of death of any additional vesting rights that would have accrued on the next vesting date had the Participant not died. The proration shall be based upon the number of days accrued in the current vesting period prior to the Participant's date of death.

5. METHOD OF EXERCISING OPTION.

Subject to the terms and conditions of this Agreement, the Option may be exercised by written notice to the Company or its designee, in substantially the form of Exhibit A attached hereto. Such notice shall state the number of Shares with respect to which the Option is being exercised and shall be signed by the person exercising the Option. Payment of the purchase price for such Shares shall be made in accordance with Paragraph 8 of the Plan. The Company shall deliver a certificate or certificates representing such Shares as soon as practicable after the notice shall be received, provided, however, that the Company may delay issuance of such Shares until completion of any action or obtaining of any consent, which the Company deems necessary under any applicable law (including, without limitation, state securities or "blue sky" laws). The certificate or certificates for the Shares as to which the Option shall have been so exercised shall be registered in the Company's share register in the name of the person so exercising the Option (or, if the Option shall be exercised by the Participant and if the Participant shall so request in the notice exercising the Option, shall be registered in the name of the Participant and another person jointly, with right of survivorship) and shall be delivered as provided above to or upon the written order of the person exercising the Option. In the event the Option shall be exercised, pursuant to Section 4 hereof, by any person other than the Participant, such notice shall be accompanied by appropriate proof of the right of such person to exercise the Option. All Shares that shall be purchased upon the exercise of the Option as provided herein shall be fully paid and nonassessable.

6. PARTIAL EXERCISE.

Exercise of this Option to the extent above stated may be made in part at any time and from time to time within the above limits, except that no fractional share shall be issued pursuant to this Option.

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7. NON-ASSIGNABILITY.

The Option shall not be transferable by the Participant otherwise than by will or by the laws of descent and distribution or pursuant to a qualified domestic relations order as defined by the Code or Title I of the Employee Retirement Income Security Act or the rules thereunder. However, the Participant, with the approval of the Administrator, may transfer the Option for no consideration to or for the benefit of the Participant's Immediate Family (including, without limitation, to a trust for the benefit of the Participant's Immediate Family or to a partnership or limited liability company for one or more members of the Participant's Immediate Family), subject to such limits as the Administrator may establish, and the transferee shall remain subject to all the terms and conditions applicable to the Option prior to such transfer and each such transferee shall so acknowledge in writing as a condition precedent to the effectiveness of such transfer. The term "Immediate Family" shall mean the Participant's spouse, former spouse, parents, children, stepchildren, adoptive relationships, sisters, brothers, nieces, nephews and grandchildren (and, for this purpose, shall also include the Participant). Except as provided in the previous sentence, the Option shall be exercisable, during the Participant's lifetime, only by the Participant (or, in the event of legal incapacity or incompetency, by the Participant's guardian or representative) and shall not be assigned, pledged or hypothecated in any way (whether by operation of law or otherwise) and shall not be subject to execution, attachment or similar process. Any attempted transfer, assignment, pledge, hypothecation or other disposition of the Option or of any rights granted hereunder contrary to the provisions of this Section 7, or the levy of any attachment or similar process upon the Option shall be null and void.

8. NO RIGHTS AS STOCKHOLDER UNTIL EXERCISE.

The Participant shall have no rights as a stockholder with respect to Shares subject to this Agreement until registration of the Shares in the Company's share register in the name of the Participant. Except as is expressly provided in the Plan with respect to certain changes in the capitalization of the Company, no adjustment shall be made for dividends or similar rights for which the record date is prior to the date of such registration.

9. ADJUSTMENTS.

The Plan contains provisions covering the treatment of Options in a number of contingencies such as stock splits and mergers. Provisions in the Plan for adjustment with respect to stock subject to Options and the related provisions with respect to successors to the business of the Company are hereby made applicable hereunder and are incorporated herein by reference.

10. TAXES.

The Participant acknowledges that upon exercise of the Option the Participant will be deemed to have taxable income measured by the difference between the then fair market value of the Shares received upon exercise and the price paid for such Shares pursuant to this Agreement. The Participant acknowledges that any income or other taxes due from him or her with respect to this Option or the Shares issuable pursuant to this Option shall be the Participant's responsibility.

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The Participant agrees that the Company may withhold from the Participant's remuneration, if any, the minimum statutory amount of federal, state and local withholding taxes attributable to such amount that is considered compensation includable in such person's gross income. At the Company's discretion, the amount required to be withheld may be withheld in cash from such remuneration, or in kind from the Shares otherwise deliverable to the Participant on exercise of the Option. The Participant further agrees that, if the Company does not withhold an amount from the Participant's remuneration sufficient to satisfy the Company's income tax withholding obligation, the Participant will reimburse the Company on demand, in cash, for the amount under-withheld.

11. PURCHASE FOR INVESTMENT.

Unless the offering and sale of the Shares to be issued upon the particular exercise of the Option shall have been effectively registered under the Securities Act of 1933, as now in force or hereafter amended (the "1933 Act"), the Company shall be under no obligation to issue the Shares covered by such exercise unless and until the following conditions have been fulfilled:

- (a) The person(s) who exercise the Option shall warrant to the Company, at the time of such exercise, that such person(s) are acquiring such Shares for their own respective accounts, for investment, and not with a view to, or for sale in connection with, the distribution of any such Shares, in which event the person(s) acquiring such Shares shall be bound by the provisions of the following legend which shall be endorsed upon the certificate(s) evidencing the Shares issued pursuant to such exercise:

"The shares represented by this certificate have been taken for investment and they may not be sold or otherwise transferred by any person, including a pledgee, unless (1) either (a) a Registration Statement with respect to such shares shall be effective under the Securities Act of 1933, as amended, or (b) the Company shall have received an opinion of counsel satisfactory to it that an exemption from registration under such Act is then available, and (2) there shall have been compliance with all applicable state securities laws;" and

- (b) If the Company so requires, the Company shall have received an opinion of its counsel that the Shares may be issued upon such particular exercise in compliance with the 1933 Act without registration thereunder. Without limiting the generality of the foregoing, the Company may delay issuance of the Shares until completion of any action or obtaining of any consent, which the Company deems necessary under any applicable law (including without limitation state securities or "blue sky" laws).

12. RESTRICTIONS ON TRANSFER OF SHARES.

12.1 The Shares acquired by the Participant pursuant to the exercise of the Option granted hereby shall not be transferred by the Participant except as permitted herein.

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12.2 In the event of the Participant's termination of service for any reason, the Company shall have the option, but not the obligation, to repurchase all or any part of the Shares issued pursuant to this Agreement (including, without limitation, Shares purchased after termination of employment, Disability or death in accordance with Section 4 hereof). In the event the Company does not, upon the termination of service of the Participant (as described above), exercise its option pursuant to this Section 12.2, the restrictions set forth in the balance of this Agreement shall not thereby lapse, and the Participant for himself or herself, his or her heirs, legatees, executors, administrators and other successors in interest, agrees that the Shares shall remain subject to such restrictions. The following provisions shall apply to a repurchase under this Section 12.2:

- (i) The per share repurchase price of the Shares to be sold to the Company upon exercise of its option under this Section 12.2 shall be equal to the Fair Market Value of each such Share determined in accordance with the Plan as of the date of termination of service; provided, however, in the event of a termination by the Company for "cause" (as defined in the Plan), the per share repurchase price of the Shares to be sold to the Company upon exercise of its option under this Section 12.2 shall be equal to the Purchase Price.
- (ii) The Company's option to repurchase the Participant's Shares in the event of termination of service shall be valid for a period of 18 months commencing with the date of such termination of service.
- (iii) In the event the Company shall be entitled to and shall elect to exercise its option to repurchase the Participant's Shares under this Section 12.2, the Company shall notify the Participant, or in case of death, his or her Survivor, in writing of its intent to repurchase the Shares. Such written notice may be mailed by the Company up to and including the last day of the time period provided for in Section 12.2(h) for exercise of the Company's option to repurchase.
- (iv) The written notice to the Participant shall specify the address at, and the time and date on, which payment of the repurchase price is to be made (the "Closing"). The date specified shall not be less than ten days nor more than 60 days from the date of the mailing of the notice, and the Participant or his or her successor in interest with respect to the Shares shall have no further rights as the owner thereof from and after the date specified in the notice. At the Closing, the repurchase price shall be delivered to the Participant or his or her successor in interest and the Shares being purchased, duly endorsed for transfer, shall, to the extent that they are not then in the possession of the Company, be delivered to the Company by the Participant or his or her successor in interest.

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12.3 It shall be a condition precedent to the validity of any sale or other transfer of any Shares by the Participant that the following restrictions be complied with (except as hereinafter otherwise provided):

- (i) No Shares owned by the Participant may be sold, pledged or otherwise transferred (including by gift or devise) to any person or entity, voluntarily, or by operation of law, except in accordance with the terms and conditions hereinafter set forth.
- (ii) Before selling or otherwise transferring all or part of the Shares, the Participant shall give written notice of such intention to the Company, which notice shall include the name of the proposed transferee, the proposed purchase price per share, the terms of payment of such purchase price and all other matters relating to such sale or transfer and shall be accompanied by a copy of the binding written agreement of the proposed transferee to purchase the Shares of the Participant. Such notice shall constitute a binding offer by the Participant to sell to the Company such number of the Shares then held by the Participant as are proposed to be sold in the notice at the monetary price per share designated in such notice, payable on the terms offered to the Participant by the proposed transferee (provided, however, that the Company shall not be required to meet any non-monetary terms of the proposed transfer, including, without limitation, delivery of other securities in exchange for the Shares proposed to be sold). The Company shall give written notice to the Participant as to whether such offer has been accepted in whole by the Company within sixty days after its receipt of written notice from the Participant. The Company may only accept such offer in whole and may not accept such offer in part. Such acceptance notice shall fix a time, location and date for the closing on such purchase ("Closing Date") which shall not be less than ten nor more than sixty days after the giving of the acceptance notice. The place for such closing shall be at the Company's principal office. At such closing, the Participant shall accept payment as set forth herein and shall deliver to the Company in exchange therefor certificates for the number of Shares stated in the notice accompanied by duly executed instruments of transfer.
- (iii) If the Company shall fail to accept any such offer, the Participant shall be free to sell all, but not less than all, of the Shares set forth in his or her notice to the designated transferee at the price and terms designated in the Participant's notice, provided that (i) such sale is consummated within six months after the giving of notice by the Participant to the Company as aforesaid, and (ii) the transferee first agrees in writing to be bound by the provisions of this Section 12 so that such transferee (and all subsequent transferees) shall thereafter only be permitted to sell or transfer the Shares in accordance with the terms hereof. After the expiration of such six months, the provisions of this Section 12.3 shall again apply with respect to any proposed voluntary transfer of the Participant's Shares.

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- (iv) The restrictions on transfer contained in this Section 12.3 shall not apply to (a) transfers by the Participant to his or her spouse or children or to a trust for the benefit of his or her spouse or children, (b) transfers by the Participant to his or her guardian or conservator, and (c) or transfers by the Participant, in the event of his or her death, to his or her executor(s) or administrator(s) or to trustee(s) under his or her will (collectively, "Permitted Transferees"); provided however, that in any such event the Shares so transferred in the hands of each such Permitted Transferee shall remain subject to this Agreement, and each such Permitted Transferee shall so acknowledge in writing as a condition precedent to the effectiveness of such transfer.
  - (v) The provisions of this Section 12.3 may be waived by the Company. Any such waiver may be unconditional or based upon such conditions as the Company may impose.

12.4 In the event that the Participant or his or her successor in interest fails to deliver the Shares to be repurchased by the Company under this Agreement, the Company may elect (a) to establish a segregated account in the amount of the repurchase price, such account to be turned over to the Participant or his or her successor in interest upon delivery of such Shares, and (b) immediately to take such action as is appropriate to transfer record title of such Shares from the Participant to the Company and to treat the Participant and such Shares in all respects as if delivery of such Shares had been made as required by this Agreement. The Participant hereby irrevocably grants the Company a power of attorney which shall be coupled with an interest for the purpose of effectuating the preceding sentence.

12.5 If the Company shall pay a stock dividend or declare a stock split on or with respect to any of its Common Stock, or otherwise distribute securities of the Company to the holders of its Common Stock, the number of shares of stock or other securities of the Company issued with respect to the shares then subject to the restrictions contained in this Agreement shall be added to the Shares subject to the Company's rights to repurchase pursuant to this Agreement. If the Company shall distribute to its stockholders shares of stock of another corporation, the shares of stock of such other corporation, distributed with respect to the Shares then subject to the restrictions contained in this Agreement, shall be added to the Shares subject to the Company's rights to repurchase pursuant to this Agreement.

12.6 If the outstanding shares of Common Stock of the Company shall be subdivided into a greater number of shares or combined into a smaller number of shares, or in the event of a reclassification of the outstanding shares of Common Stock of the Company, or if the Company shall be a party to a merger, consolidation or capital reorganization, there shall be substituted for the Shares then subject to the restrictions contained in this Agreement such amount and kind of securities as are issued in such subdivision, combination, reclassification, merger, consolidation or capital reorganization in respect of the Shares subject immediately prior thereto to the Company's rights to repurchase pursuant to this Agreement.

12.7 The Company shall not be required to transfer any Shares on its books which shall have been sold, assigned or otherwise transferred in violation of this Agreement, or to treat as owner of such Shares, or to accord the right to vote as such owner or to pay dividends to, any person or organization to which any such Shares shall have been so sold, assigned or otherwise transferred, in violation of this Agreement.



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12.8 The provisions of Sections 12.1, 12.2 and 12.3 shall terminate upon the effective date of the registration of the Shares pursuant to the Securities Exchange Act of 1934.

12.9 If, in connection with a registration statement filed by the Company pursuant to the 1933 Act, the Company or its underwriter so requests, the Participant will agree not to sell any Shares for a period not to exceed 180 days following the effectiveness of such registration.

12.10 The Participant acknowledges and agrees that neither the Company, its shareholders nor its directors and officers, has any duty or obligation to disclose to the Participant any material information regarding the business of the Company or affecting the value of the Shares before, at the time of, or following a termination of the employment of the Participant by the Company, including, without limitation, any information concerning plans for the Company to make a public offering of its securities or to be acquired by or merged with or into another firm or entity.

12.11 All certificates representing the Shares to be issued to the Participant pursuant to this Agreement shall have endorsed thereon a legend substantially as follows: "The shares represented by this certificate are subject to restrictions set forth in a Non-Qualified Stock Option Agreement dated \_\_\_\_\_, 20\_\_\_\_ with this Company, a copy of which Agreement is available for inspection at the offices of the Company or will be made available upon request."

13. NO OBLIGATION TO MAINTAIN RELATIONSHIP.

The Company is not by the Plan or this Option obligated to continue the Participant as an employee, director or consultant of the Company or an Affiliate. The Participant acknowledges: (i) that the Plan is discretionary in nature and may be suspended or terminated by the Company at any time; (ii) that the grant of the Option is a one-time benefit which does not create any contractual or other right to receive future grants of options, or benefits in lieu of options; (iii) that all determinations with respect to any such future grants, including, but not limited to, the times when options shall be granted, the number of shares subject to each option, the option price, and the time or times when each option shall be exercisable, will be at the sole discretion of the Company; (iv) that the Participant's participation in the Plan is voluntary; (v) that the value of the Option is an extraordinary item of compensation which is outside the scope of the Participant's employment contract, if any; and (vi) that the Option is not part of normal or expected compensation for purposes of calculating any severance, resignation, redundancy, end of service payments, bonuses, long-service awards, pension or retirement benefits or similar payments.

14. NOTICES.

Any notices required or permitted by the terms of this Agreement or the Plan shall be given by recognized courier service, facsimile, registered or certified mail, return receipt requested, addressed as follows:

If to the Company:

President  
Genetix Pharmaceuticals Inc.  
840 Memorial Drive, 5th floor  
Cambridge, MA 02139

If to the Participant:

At the address set forth on the first page of this Agreement.

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or to such other address or addresses of which notice in the same manner has previously been given. Any such notice shall be deemed to have been given upon the earlier of receipt, one business day following delivery to a recognized courier service or three business days following mailing by registered or certified mail.

15. GOVERNING LAW.

This Agreement shall be construed and enforced in accordance with the law of the State of Delaware, without giving effect to the conflict of law principles thereof. Any controversy, dispute or claim arising out of or in connection with this Agreement, or the breach, termination or validity hereof, shall be settled by final and binding arbitration to be conducted by an arbitration tribunal in Boston, Massachusetts, pursuant to the rules of the American Arbitration Association. The arbitration tribunal shall consist of three arbitrators. The party initiating arbitration shall nominate one arbitrator in the request for arbitration and the other party shall nominate a second in the answer thereto within thirty (30) days of receipt of the request. The two arbitrators so named will then jointly appoint the third arbitrator. If the answering party fails to nominate its arbitrator within the thirty (30) day period, or if the arbitrators named by the parties fail to agree on the third arbitrator within sixty (60) days, the office of the American Arbitration Association in Boston, Massachusetts shall make the necessary appointments of such arbitrator(s). The decision or award of the arbitration tribunal (by a majority determination, or if there is no majority, then by the determination of the third arbitrator, if any) shall be final, and judgment upon such decision or award may be entered in any competent court or application may be made to any competent court for judicial acceptance of such decision or award and an order of enforcement. In the event of any procedural matter not covered by the aforesaid rules, the procedural law of Massachusetts shall govern.

16. BENEFIT OF AGREEMENT.

Subject to the provisions of the Plan and the other provisions hereof, this Agreement shall be for the benefit of and shall be binding upon the heirs, executors, administrators, successors and assigns of the parties hereto.

17. ENTIRE AGREEMENT.

This Agreement, together with the Plan, embodies the entire agreement and understanding between the parties hereto with respect to the subject matter hereof and supersedes all prior oral or written agreements and understandings relating to the subject matter hereof. No statement, representation, warranty, covenant or agreement not expressly set forth in this Agreement shall affect or be used to interpret, change or restrict, the express terms and provisions of this Agreement, provided, however, in any event, this Agreement shall be subject to and governed by the Plan.

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18. MODIFICATIONS AND AMENDMENTS.

The terms and provisions of this Agreement may be modified or amended as provided in the Plan.

19. WAIVERS AND CONSENTS.

Except as provided in the Plan, the terms and provisions of this Agreement may be waived, or consent for the departure therefrom granted, only by written document executed by the party entitled to the benefits of such terms or provisions. No such waiver or consent shall be deemed to be or shall constitute a waiver or consent with respect to any other terms or provisions of this Agreement, whether or not similar. Each such waiver or consent shall be effective only in the specific instance and for the purpose for which it was given, and shall not constitute a continuing waiver or consent.

20. DATA PRIVACY.

By entering into this Agreement, the Participant: (i) authorizes the Company and each Affiliate, and any agent of the Company or any Affiliate administering the Plan or providing Plan record keeping services, to disclose to the Company or any of its Affiliates such information and data as the Company or any such Affiliate shall request in order to facilitate the grant of options and the administration of the Plan; (ii) waives any data privacy rights he or she may have with respect to such information; and (iii) authorizes the Company and each Affiliate to store and transmit such information in electronic form.

21. CONSENT OF SPOUSE.

If the Participant is married as of the date of this Agreement, the Participant's spouse shall execute a Consent of Spouse in the form of Exhibit B hereto, effective as of the date hereof. Such consent shall not be deemed to confer or convey to the spouse any rights in the Shares that do not otherwise exist by operation of law or the agreement of the parties. If the Participant marries or remarries subsequent to the date hereof, the Participant shall, not later than 60 days thereafter, obtain his or her new spouse's acknowledgement of and consent to the existence and binding effect of Section 12.2 of this Agreement by such spouse's executing and delivering a Consent of Spouse in the form of Exhibit B.

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IN WITNESS WHEREOF, the Company has caused this Non-Qualified Stock Option Agreement to be executed by its duly authorized officer, and the Participant has hereunto set his or her hand, all as of the day and year first above written.

**GENETIX PHARMACEUTICALS INC.**

By: \_\_\_\_\_  
Name:  
Title:

\_\_\_\_\_  
Participant Name:

**NOTICE OF EXERCISE OF NON-QUALIFIED STOCK OPTION**  
**[Form for Unregistered Shares]**

To: Genetix Pharmaceuticals Inc.

Ladies and Gentlemen:

I hereby exercise my Non-Qualified Stock Option to purchase \_\_\_\_\_ shares (the "Shares") of the common stock, \$0.01 par value, of Genetix Pharmaceuticals Inc. (the "Company"), at the exercise price of \$ \_\_\_\_\_ per share, pursuant to and subject to the terms of that certain Non-Qualified Stock Option Agreement between the undersigned and the Company dated \_\_\_\_\_, 20\_\_.

I am aware that the Shares have not been registered under the Securities Act of 1933, as amended (the "1933 Act"), or any state securities laws. I understand that the reliance by the Company on exemptions under the 1933 Act is predicated in part upon the truth and accuracy of the statements by me in this Notice of Exercise.

I hereby represent and warrant that (1) I have been furnished with all information which I deem necessary to evaluate the merits and risks of the purchase of the Shares; (2) I have had the opportunity to ask questions concerning the Shares and the Company and all questions posed have been answered to my satisfaction; (3) I have been given the opportunity to obtain any additional information I deem necessary to verify the accuracy of any information obtained concerning the Shares and the Company; and (4) I have such knowledge and experience in financial and business matters that I am able to evaluate the merits and risks of purchasing the Shares and to make an informed investment decision relating thereto.

I hereby represent and warrant that I am purchasing the Shares for my own personal account for investment and not with a view to the sale or distribution of all or any part of the Shares.

I understand that because the Shares have not been registered under the 1933 Act, I must continue to bear the economic risk of the investment for an indefinite time and the Shares cannot be sold unless the Shares are subsequently registered under applicable federal and state securities laws or an exemption from such registration requirements is available.

I agree that I will in no event sell or distribute or otherwise dispose of all or any part of the Shares unless (1) there is an effective registration statement under the 1933 Act and applicable state securities laws covering any such transaction involving the Shares or (2) the Company receives an opinion of my legal counsel (concurring in by legal counsel for the Company) stating that such transaction is exempt from registration or the Company otherwise satisfies itself that such transaction is exempt from registration.

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I consent to the placing of a legend on my certificate for the Shares stating that the Shares have not been registered and setting forth the restriction on transfer contemplated hereby and to the placing of a stop transfer order on the books of the Company and with any transfer agents against the Shares until the Shares may be legally resold or distributed without restriction.

I understand that at the present time Rule 144 of the Securities and Exchange Commission (the "SEC") may not be relied on for the resale or distribution of the Shares by me. I understand that the Company has no obligation to me to register the sale of the Shares with the SEC and has not represented to me that it will register the sale of the Shares.

I understand the terms and restrictions on the right to dispose of the Shares set forth in the 2002 Employee, Director and Consultant Stock Plan and the Non-Qualified Option Agreement, both of which I have carefully reviewed. I consent to the placing of a legend on my certificate for the Shares referring to such restriction and the placing of stop transfer orders until the Shares may be transferred in accordance with the terms of such restrictions.

I have considered the Federal, state and local income tax implications of the exercise of my Option and the purchase and subsequent sale of the Shares.

I am paying the option exercise price for the Shares as follows:

Please issue the stock certificate for the Shares (check one):

to me; or

to me and \_\_\_\_\_, as joint tenants with right of survivorship and mail the certificate to me at the following address:

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My mailing address for shareholder communications, if different from the address listed above is:

Very truly yours.

\_\_\_\_\_  
Employee (signature)

\_\_\_\_\_  
Print Name

\_\_\_\_\_  
Date

\_\_\_\_\_  
Social Security Number

**(Form for Registered Shares)**  
**NOTICE OF EXERCISE OF NON-QUALIFIED STOCK OPTION**

TO: Genetix Pharmaceuticals Inc.

IMPORTANT NOTICE: This form of Notice of Exercise may only be used at such time as the Company has filed a Registration Statement with the Securities and Exchange Commission under which the issuance of the Shares for which this exercise is being made is registered and such Registration Statement remains effective.

Ladies and Gentlemen:

I hereby exercise my Non-Qualified Stock Option to purchase \_\_\_\_\_ shares (the "Shares") of the common stock, \$0.01 par value, of Genetix Pharmaceuticals Inc. (the "Company"), at the exercise price of \$ \_\_\_\_\_ per share, pursuant to and subject to the terms of that certain Non-Qualified Stock Option Agreement between the undersigned and the Company dated \_\_\_\_\_, 20\_\_.

I understand the nature of the investment I am making and the financial risks thereof. I am aware that it is my responsibility to have consulted with competent tax and legal advisors about the relevant national, state and local income tax and securities laws affecting the exercise of the Option and the purchase and subsequent sale of the Shares.

I am paying the option exercise price for the Shares as follows:

Please issue the Shares (check one):

- to me; or
- to me and \_\_\_\_\_, as joint tenants with right of survivorship

at the following address:



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My mailing address for shareholder communications, if different from the address listed above is:

Very truly yours.

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Employee (signature)

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Print Name

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Date

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Social Security Number

**CONSENT OF SPOUSE**

I, \_\_\_\_\_, spouse of \_\_\_\_\_, acknowledge that I have read the Non-Qualified Stock Option Agreement dated as of \_\_\_\_\_, 20\_\_\_\_ (the "Agreement") to which this Consent is attached as Exhibit B and that I know its contents. Capitalized terms used and not defined herein shall have the meanings assigned to such terms in the Agreement. I am aware that by its provisions the Shares granted to my spouse pursuant to the Agreement are subject to a right of repurchase in favor of Genetix Pharmaceuticals Inc. (the "Company") and that, accordingly, the Company has the right to repurchase up to all of the Shares of which I may become possessed as a result of a gift from my spouse or a court decree and/or any property settlement in any domestic litigation.

I hereby agree that my interest, if any, in the Shares subject to the Agreement shall be irrevocably bound by the Agreement and further understand and agree that any community property interest I may have in the Shares shall be similarly bound by the Agreement.

I agree to the repurchase right described in Section 12.2 of the Agreement and I hereby consent to the repurchase of the Shares by the Company and the sale of the Shares by my spouse or my spouse's legal representative in accordance with the provisions of the Agreement. Further, as part of the consideration for the Agreement, I agree that at my death, if I have not disposed of any interest of mine in the Shares by an outright bequest of the Shares to my spouse, then the Company shall have the same rights against my legal representative to exercise its rights of repurchase with respect to any interest of mine in the Shares as it would have had pursuant to the Agreement if I had acquired the Shares pursuant to a court decree in domestic litigation.

I AM AWARE THAT THE LEGAL, FINANCIAL AND RELATED MATTERS CONTAINED IN THE AGREEMENT ARE COMPLEX AND THAT I AM FREE TO SEEK INDEPENDENT PROFESSIONAL GUIDANCE OR COUNSEL WITH RESPECT TO THIS CONSENT. I HAVE EITHER SOUGHT SUCH GUIDANCE OR COUNSEL OR DETERMINED AFTER REVIEWING THE AGREEMENT CAREFULLY THAT I WILL WAIVE SUCH RIGHT.

Dated as of the \_\_\_\_\_ day of \_\_\_\_\_, 20\_\_\_\_\_.

\_\_\_\_\_  
Print name:

**NOTE: Unless approved by the Administrator (as defined in the Plan), delete Exhibit C hereto and the bold-faced and bracketed language in Section 3.**

**FORM OF  
INCENTIVE STOCK OPTION AGREEMENT  
GENETIX PHARMACEUTICALS INC.**

AGREEMENT made as of the \_\_\_\_\_ day of \_\_\_\_\_, 20\_\_, between Genetix Pharmaceuticals Inc. (the "Company"), a Delaware corporation having a principal place of business in Cambridge, Massachusetts, and \_\_\_\_\_ of \_\_\_\_\_, an employee of the Company (the "Employee"), for whom the Board of Directors has authorized that the Option granted herein shall begin vesting as of \_\_\_\_\_ (the "Vesting Date").

WHEREAS, the Company desires to grant to the Employee an Option to purchase shares of its common stock, \$0.01 par value per share (the "Shares"), under and for the purposes set forth in the Company's 2002 Employee, Director and Consultant Stock Plan (the "Plan");

WHEREAS, the Company and the Employee understand and agree that any terms used and not defined herein have the same meanings as in the Plan; and

WHEREAS, the Company and the Employee each intend that the Option granted herein qualify as an ISO.

NOW, THEREFORE, in consideration of the mutual covenants hereinafter set forth and for other good and valuable consideration, the parties hereto agree as follows:

1. GRANT OF OPTION.

The Company hereby grants to the Employee the right and option to purchase all or any part of an aggregate of \_\_\_\_\_ Shares, on the terms and conditions and subject to all the limitations set forth herein, under United States securities and tax laws, and in the Plan, which is incorporated herein by reference. The Employee acknowledges receipt of a copy of the Plan.

2. PURCHASE PRICE.

The purchase price of the Shares covered by the Option shall be \$ \_\_\_\_\_ per Share, subject to adjustment, as provided in the Plan, in the event of a stock split, reverse stock split or other events affecting the holders of Shares (the "Purchase Price"). Payment shall be made in accordance with Paragraph 8 of the Plan.

3. EXERCISABILITY OF OPTION.

Subject to the terms and conditions set forth in this Agreement and the Plan, the Option granted hereby shall become exercisable as follows:

On the first anniversary of the Vesting Date up to \_\_\_\_\_ Shares  
On the last day of each month thereafter for 36 months an additional \_\_\_\_\_ Shares

The foregoing rights are cumulative and are subject to the other terms and conditions of this Agreement and the Plan.

**[Optional:** Alternatively, at the election of the Employee, the Option may be exercised in whole or in part at any time as to Shares which have not yet vested in accordance with the above schedule; provided however, as a condition to exercising the Option for such unvested Shares, the Employee shall execute a Restricted Stock Agreement in the form attached hereto as Exhibit C.]

**[Optional:** Notwithstanding the foregoing, in the event of a Change of Control (as defined below), \_\_\_\_% of the Shares which would have vested in each vesting installment remaining under this Option will be vested for purposes of Section 23(B) of the Plan unless this Option has otherwise expired or been terminated pursuant to its terms or the terms of the Plan.

**Change of Control** means the occurrence of any of the following events:

- (i) **Ownership.** Any "Person" (as such term is used in Sections 13(d) and 14(d) of the Securities Exchange Act of 1934, as amended) becomes the "Beneficial Owner" (as defined in Rule 13d-3 under said Act), directly or indirectly, of securities of the Company representing 50% or more of the total voting power represented by the Company's then outstanding voting securities (excluding for this purpose the Company or its Affiliates or any employee benefit plan of the Company) pursuant to a transaction or a series of related transactions which the Board of Directors does not approve; or
- (ii) **Merger/Sale of Assets.** A merger or consolidation of the Company whether or not approved by the Board of Directors, other than a merger or consolidation which would result in the voting securities of the Company outstanding immediately prior thereto continuing to represent (either by remaining outstanding or by being converted into voting securities of the surviving entity or the parent of such corporation) at least 50% of the total voting power represented by the voting securities of the Company or such surviving entity or parent of such corporation outstanding immediately after such merger or consolidation, or the stockholders of the Company approve an agreement for the sale or disposition by the Company of all or substantially all of the Company's assets; or

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- (iii) **Change in Board Composition.** A change in the composition of the Board of Directors, as a result of which fewer than a majority of the directors are Incumbent Directors. “Incumbent Directors” shall mean directors who either (A) are directors of the Company as of the date of this Agreement, or (B) are elected, or nominated for election, to the Board of Directors with the affirmative votes of at least a majority of the Incumbent Directors at the time of such election or nomination (but shall not include an individual whose election or nomination is in connection with an actual or threatened proxy contest relating to the election of directors to the Company).]

4. TERM OF OPTION.

The Option shall terminate ten years from the date of this Agreement, but shall be subject to earlier termination as provided herein or in the Plan or, if the Employee owns as of the date hereof more than 10% of the total combined voting power of all classes of capital stock of the Company or an Affiliate, five years from the date of this Agreement, but shall be subject to earlier termination as provided herein or in the Plan.

If the Employee ceases to be an employee of the Company or of an Affiliate (for any reason other than the death or Disability of the Employee or termination of the Employee’s employment for “cause” (as defined in the Plan), the Option may be exercised, if it has not previously terminated, within three months after the date the Employee ceases to be an employee of the Company or an Affiliate, or within the originally prescribed term of the Option, whichever is earlier, but may not be exercised thereafter. In such event, the Option shall be exercisable only to the extent that the Option has become exercisable and is in effect at the date of such cessation of employment.

Notwithstanding the foregoing, in the event of the Employee’s Disability or death within three months after the termination of employment, the Employee or the Employee’s Survivors may exercise the Option within one year after the date of the Employee’s termination of employment, but in no event after the date of expiration of the term of the Option.

In the event the Employee’s employment is terminated by the Employee’s employer for “cause” (as defined in the Plan), the Employee’s right to exercise any unexercised portion of this Option shall cease immediately as of the time the Employee is notified his or her employment is terminated for “cause”, and this Option shall thereupon terminate. Notwithstanding anything herein to the contrary, if subsequent to the Employee’s termination as an employee, but prior to the exercise of the Option, the Board of Directors of the Company determines that, either prior or subsequent to the Employee’s termination, the Employee engaged in conduct which would constitute “cause.” then the Employee shall immediately cease to have any right to exercise the Option and this Option shall thereupon terminate.

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In the event of the Disability of the Employee, as determined in accordance with the Plan, the Option shall be exercisable within one year after the Employee's termination of employment or, if earlier, within the term originally prescribed by the Option. In such event, the Option shall be exercisable:

- (a) to the extent that the Option has become exercisable but has not been exercised as of the date of Disability; and
- (b) in the event rights to exercise the Option accrue periodically, to the extent of a pro rata portion through the date of Disability of any additional vesting rights that would have accrued on the next vesting date had the Employee not become Disabled. The proration shall be based upon the number of days accrued in the current vesting period prior to the date of Disability.

In the event of the death of the Employee while an employee of the Company or of an Affiliate, the Option shall be exercisable by the Employee's Survivors within one year after the date of death of the Employee or, if earlier, within the originally prescribed term of the Option. In such event, the Option shall be exercisable:

- (x) to the extent that the Option has become exercisable but has not been exercised as of the date of death; and
- (y) in the event rights to exercise the Option accrue periodically, to the extent of a pro rata portion through the date of death of any additional vesting rights that would have accrued on the next vesting date had the Employee not died. The proration shall be based upon the number of days accrued in the current vesting period prior to the Employee's date of death.

5. METHOD OF EXERCISING OPTION.

Subject to the terms and conditions of this Agreement, the Option may be exercised by written notice to the Company or its designee, in substantially the form of Exhibit A attached hereto. Such notice shall state the number of Shares with respect to which the Option is being exercised and shall be signed by the person exercising the Option. Payment of the purchase price for such Shares shall be made in accordance with Paragraph 8 of the Plan. The Company shall deliver a certificate or certificates representing such Shares as soon as practicable after the notice shall be received, provided, however, that the Company may delay issuance of such Shares until completion of any action or obtaining of any consent, which the Company deems necessary under any applicable law (including, without limitation, state securities or "blue sky" laws). The certificate or certificates for the Shares as to which the Option shall have been so exercised shall be registered in the Company's share register in the name of the person so exercising the Option (or, if the Option shall be exercised by the Employee and if the Employee shall so request in the notice exercising the Option, shall be registered in the name of the Employee and another person jointly, with right of survivorship) and shall be delivered as provided above to or upon the written order of the person exercising the Option. In the event the

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Option shall be exercised, pursuant to Section 4 hereof, by any person other than the Employee, such notice shall be accompanied by appropriate proof of the right of such person to exercise the Option. All Shares that shall be purchased upon the exercise of the Option as provided herein shall be fully paid and nonassessable.

6. PARTIAL EXERCISE

Exercise of this Option to the extent above stated may be made in part at any time and from time to time within the above limits, except that no fractional share shall be issued pursuant to this Option.

7. NON-ASSIGNABILITY

The Option shall not be transferable by the Employee otherwise than by will or by the laws of descent and distribution. The Option shall be exercisable, during the Employee's lifetime, only by the Employee (or, in the event of legal incapacity or incompetency, by the Employee's guardian or representative) and shall not be assigned, pledged or hypothecated in any way (whether by operation of law or otherwise) and shall not be subject to execution, attachment or similar process. Any attempted transfer, assignment, pledge, hypothecation or other disposition of the Option or of any rights granted hereunder contrary to the provisions of this Section 7, or the levy of any attachment or similar process upon the Option shall be null and void.

8. NO RIGHTS AS STOCKHOLDER UNTIL EXERCISE

The Employee shall have no rights as a stockholder with respect to Shares subject to this Agreement until registration of the Shares in the Company's share register in the name of the Employee. Except as is expressly provided in the Plan with respect to certain changes in the capitalization of the Company, no adjustment shall be made for dividends or similar rights for which the record date is prior to the date of such registration.

9. ADJUSTMENTS

The Plan contains provisions covering the treatment of Options in a number of contingencies such as stock splits and mergers. Provisions in the Plan for adjustment with respect to stock subject to Options and the related provisions with respect to successors to the business of the Company are hereby made applicable hereunder and are incorporated herein by reference.

10. TAXES

The Employee acknowledges that any income or other taxes due from him or her with respect to this Option or the Shares issuable pursuant to this Option shall be the Employee's responsibility.

In the event of a Disqualifying Disposition (as defined in Section 15 below) or if the Option is converted into a Non-Qualified Option and such Non-Qualified Option is exercised, the Employee agrees that the Company may withhold from the Employee's remuneration, if any,

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the minimum statutory amount of federal, state and local withholding taxes attributable to such amount that is considered compensation includable in such person's gross income. At the Company's discretion, the amount required to be withheld may be withheld in cash from such remuneration, or in kind from the Shares otherwise deliverable to the Employee on exercise of the Option. The Employee further agrees that, if the Company does not withhold an amount from the Employee's remuneration sufficient to satisfy the Company's income tax withholding obligation, the Employee will reimburse the Company on demand, in cash, for the amount under-withheld.

11. PURCHASE FOR INVESTMENT.

Unless the offering and sale of the Shares to be issued upon the particular exercise of the Option shall have been effectively registered under the Securities Act of 1933, as now in force or hereafter amended (the "1933 Act"), the Company shall be under no obligation to issue the Shares covered by such exercise unless and until the following conditions have been fulfilled:

- (a) The person(s) who exercise the Option shall warrant to the Company, at the time of such exercise, that such person(s) are acquiring such Shares for their own respective accounts, for investment, and not with a view to, or for sale in connection with, the distribution of any such Shares, in which event the person(s) acquiring such Shares shall be bound by the provisions of the following legend which shall be endorsed upon the certificate(s) evidencing the Shares issued pursuant to such exercise:
  - "The shares represented by this certificate have been taken for investment and they may not be sold or otherwise transferred by any person, including a pledgee, unless (1) either (a) a Registration Statement with respect to such shares shall be effective under the Securities Act of 1933, as amended, or (b) the Company shall have received an opinion of counsel satisfactory to it that an exemption from registration under such Act is then available, and (2) there shall have been compliance with all applicable state securities laws;" and
- (b) If the Company so requires, the Company shall have received an opinion of its counsel that the Shares may be issued upon such particular exercise in compliance with the 1933 Act without registration thereunder. Without limiting the generality of the foregoing, the Company may delay issuance of the Shares until completion of any action or obtaining of any consent, which the Company deems necessary under any applicable law (including without limitation state securities or "blue sky" laws).

12. RESTRICTIONS ON TRANSFER OF SHARES.

12.1 The Shares acquired by the Employee pursuant to the exercise of the Option granted hereby shall not be transferred by the Employee except as permitted herein.



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12.2 In the event of the Employee's termination of employment for any reason, the Company shall have the option, but not the obligation, to repurchase all or any part of the Shares issued pursuant to this Agreement (including, without limitation, Shares purchased after termination of employment, Disability or death in accordance with Section 4 hereof). In the event the Company does not, upon the termination of employment of the Employee (as described above), exercise its option pursuant to this Section 12.2, the restrictions set forth in the balance of this Agreement shall not thereby lapse, and the Employee for himself or herself, his or her heirs, legatees, executors, administrators and other successors in interest, agrees that the Shares shall remain subject to such restrictions. The following provisions shall apply to a repurchase under this Section 12.2:

- (i) The per share repurchase price of the Shares to be sold to the Company upon exercise of its option under this Section 12.2 shall be equal to the Fair Market Value of each such Share determined in accordance with the Plan as of the date of termination of employment; provided, however, in the event of a termination by the Company for "cause" (as defined in the Plan), the per share repurchase price of the Shares to be sold to the Company upon exercise of its option under this Section 12.2 shall be equal to the Purchase Price.
- (ii) The Company's option to repurchase the Employee's Shares in the event of termination of service shall be valid for a period of 18 months commencing with the date of such termination of service.
- (iii) In the event the Company shall be entitled to and shall elect to exercise its option to repurchase the Employee's Shares under this Section 12.2, the Company shall notify the Employee, or in case of death, his or her Survivor, in writing of its intent to repurchase the Shares. Such written notice may be mailed by the Company up to and including the last day of the time period provided for in Section 12.2(h) for exercise of the Company's option to repurchase.
- (iv) The written notice to the Employee shall specify the address at, and the time and date on, which payment of the repurchase price is to be made (the "Closing"). The date specified shall not be less than ten days nor more than 60 days from the date of the mailing of the notice, and the Employee or his or her successor in interest with respect to the Shares shall have no further rights as the owner thereof from and after the date specified in the notice. At the Closing, the repurchase price shall be delivered to the Employee or his or her successor in interest and the Shares being purchased, duly endorsed for transfer, shall, to the extent that they are not then in the possession of the Company, be delivered to the Company by the Employee or his or her successor in interest.

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12.3 It shall be a condition precedent to the validity of any sale or other transfer of any Shares by the Employee that the following restrictions be complied with (except as hereinafter otherwise provided):

- (i) No Shares owned by the Employee may be sold, pledged or otherwise transferred (including by gift or devise) to any person or entity, voluntarily, or by operation of law, except in accordance with the terms and conditions hereinafter set forth.
- (ii) Before selling or otherwise transferring all or part of the Shares, the Employee shall give written notice of such intention to the Company, which notice shall include the name of the proposed transferee, the proposed purchase price per share, the terms of payment of such purchase price and all other matters relating to such sale or transfer and shall be accompanied by a copy of the binding written agreement of the proposed transferee to purchase the Shares of the Employee. Such notice shall constitute a binding offer by the Employee to sell to the Company such number of the Shares then held by the Employee as are proposed to be sold in the notice at the monetary price per share designated in such notice, payable on the terms offered to the Employee by the proposed transferee (provided, however, that the Company shall not be required to meet any non-monetary terms of the proposed transfer, including, without limitation, delivery of other securities in exchange for the Shares proposed to be sold). The Company shall give written notice to the Employee as to whether such offer has been accepted in whole by the Company within sixty days after its receipt of written notice from the Employee. The Company may only accept such offer in whole and may not accept such offer in part. Such acceptance notice shall fix a time, location and date for the closing on such purchase ("Closing Date") which shall not be less than ten nor more than sixty days after the giving of the acceptance notice. The place for such closing shall be at the Company's principal office. At such closing, the Employee shall accept payment as set forth herein and shall deliver to the Company in exchange therefor certificates for the number of Shares stated in the notice accompanied by duly executed instruments of transfer.
- (iii) If the Company shall fail to accept any such offer, the Employee shall be free to sell all, but not less than all, of the Shares set forth in his or her notice to the designated transferee at the price and terms designated in the Employee's notice, provided that (i) such sale is consummated within six months after the giving of notice by the Employee to the Company as aforesaid, and (ii) the transferee first agrees in writing to be bound by the provisions of this Section 12 so that such transferee (and all subsequent

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transferees) shall thereafter only be permitted to sell or transfer the Shares in accordance with the terms hereof. After the expiration of such six months, the provisions of this Section 12.3 shall again apply with respect to any proposed voluntary transfer of the Employee's Shares.

- (iv) The restrictions on transfer contained in this Section 12.3 shall not apply to (a) transfers by the Employee to his or her spouse or children or to a trust for the benefit of his or her spouse or children, (b) transfers by the Employee to his or her guardian or conservator, and (c) transfers by the Employee, in the event of his or her death, to his or her executor(s) or administrator(s) or to trustee(s) under his or her will (collectively, "Permitted Transferees"); provided however, that in any such event the Shares so transferred in the hands of each such Permitted Transferee shall remain subject to this Agreement, and each such Permitted Transferee shall so acknowledge in writing as a condition precedent to the effectiveness of such transfer.
- (v) The provisions of this Section 12.3 may be waived by the Company. Any such waiver may be unconditional or based upon such conditions as the Company may impose.

12.4 In the event that the Employee or his or her successor in interest fails to deliver the Shares to be repurchased by the Company under this Agreement, the Company may elect (a) to establish a segregated account in the amount of the repurchase price, such account to be turned over to the Employee or his or her successor in interest upon delivery of such Shares, and (b) immediately to take such action as is appropriate to transfer record title of such Shares from the Employee to the Company and to treat the Employee and such Shares in all respects as if delivery of such Shares had been made as required by this Agreement. The Employee hereby irrevocably grants the Company a power of attorney which shall be coupled with an interest for the purpose of effectuating the preceding sentence.

12.5 If the Company shall pay a stock dividend or declare a stock split on or with respect to any of its Common Stock, or otherwise distribute securities of the Company to the holders of its Common Stock, the number of shares of stock or other securities of the Company issued with respect to the shares then subject to the restrictions contained in this Agreement shall be added to the Shares subject to the Company's rights to repurchase pursuant to this Agreement. If the Company shall distribute to its stockholders shares of stock of another corporation, the shares of stock of such other corporation, distributed with respect to the Shares then subject to the restrictions contained in this Agreement, shall be added to the Shares subject to the Company's rights to repurchase pursuant to this Agreement.

12.6 If the outstanding shares of Common Stock of the Company shall be subdivided into a greater number of shares or combined into a smaller number of shares, or in the event of a reclassification of the outstanding shares of Common Stock of the Company, or if the Company shall be a party to a merger, consolidation or capital reorganization, there shall be

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substituted for the Shares then subject to the restrictions contained in this Agreement such amount and kind of securities as are issued in such subdivision, combination, reclassification, merger, consolidation or capital reorganization in respect of the Shares subject immediately prior thereto to the Company's rights to repurchase pursuant to this Agreement.

12.7 The Company shall not be required to transfer any Shares on its books which shall have been sold, assigned or otherwise transferred in violation of this Agreement, or to treat as owner of such Shares, or to accord the right to vote as such owner or to pay dividends to, any person or organization to which any such Shares shall have been so sold, assigned or otherwise transferred, in violation of this Agreement.

12.8 The provisions of Sections 12.1, 12.2 and 12.3 shall terminate upon the effective date of the registration of the Shares pursuant to the Securities Exchange Act of 1934.

12.9 If, in connection with a registration statement filed by the Company pursuant to the 1933 Act, the Company or its underwriter so requests, the Employee will agree not to sell any Shares for a period not to exceed 180 days following the effectiveness of such registration.

12.10 The Employee acknowledges and agrees that neither the Company, its shareholders nor its directors and officers, has any duty or obligation to disclose to the Employee any material information regarding the business of the Company or affecting the value of the Shares before, at the time of, or following a termination of the employment of the Employee by the Company, including, without limitation, any information concerning plans for the Company to make a public offering of its securities or to be acquired by or merged with or into another firm or entity.

12.11 All certificates representing the Shares to be issued to the Employee pursuant to this Agreement shall have endorsed thereon a legend substantially as follows: "The shares represented by this certificate are subject to restrictions set forth in a Incentive Stock Option Agreement dated \_\_\_\_\_ with this Company, a copy of which Agreement is available for inspection at the offices of the Company or will be made available upon request."

### 13. NO OBLIGATION TO EMPLOY.

The Company is not by the Plan or this Option obligated to continue the Employee as an employee of the Company or an Affiliate. The Employee acknowledges: (i) that the Plan is discretionary in nature and may be suspended or terminated by the Company at any time; (ii) that the grant of the Option is a one-time benefit which does not create any contractual or other right to receive future grants of options, or benefits in lieu of options; (iii) that all determinations with respect to any such future grants, including, but not limited to, the times when options shall be granted, the number of shares subject to each option, the option price, and the time or times when each option shall be exercisable, will be at the sole discretion of the Company; (iv) that the Employee's participation in the Plan is voluntary; (v) that the value of the Option is an extraordinary item of compensation which is outside the scope of the Employee's employment contract, if any; and (vi) that the Option is not part of normal or expected compensation for purposes of calculating any severance, resignation, redundancy, end of service payments, bonuses, long-service awards, pension or retirement benefits or similar payments.

14. OPTION IS INTENDED TO BE AN ISO.

The parties each intend that the Option be an ISO so that the Employee (or the Employee's Survivors) may qualify for the favorable tax treatment provided to holders of Options that meet the standards of Section 422 of the Code. Any provision of this Agreement or the Plan which conflicts with the Code so that this Option would not be deemed an ISO is null and void and any ambiguities shall be resolved so that the Option qualifies as an ISO. Nonetheless, if the Option is determined not to be an ISO, the Employee understands that neither the Company nor any Affiliate is responsible to compensate him or her or otherwise make up for the treatment of the Option as a Non-qualified Option and not as an ISO. The Employee should consult with the Employee's own tax advisors regarding the tax effects of the Option and the requirements necessary to obtain favorable tax treatment under Section 422 of the Code, including, but not limited to, holding period requirements.

15. NOTICE TO COMPANY OF DISQUALIFYING DISPOSITION.

The Employee agrees to notify the Company in writing immediately after the Employee makes a Disqualifying Disposition of any of the Shares acquired pursuant to the exercise of the Option. A Disqualifying Disposition is defined in Section 424(c) of the Code and includes any disposition (including any sale) of such Shares before the later of (a) two years after the date the Employee was granted the Option or (b) one year after the date the Employee acquired Shares by exercising the Option, except as otherwise provided in Section 424(c) of the Code. If the Employee has died before the Shares are sold, these holding period requirements do not apply and no Disqualifying Disposition can occur thereafter.

16. NOTICES.

Any notices required or permitted by the terms of this Agreement or the Plan shall be given by recognized courier service, facsimile, registered or certified mail, return receipt requested, addressed as follows:

If to the Company:

President  
Genetix Pharmaceuticals Inc.  
840 Memorial Drive, 5th floor  
Cambridge, MA 02139

If to the Employee:

At the address set forth on the first page of this Agreement.

or to such other address or addresses of which notice in the same manner has previously been given. Any such notice shall be deemed to have been given upon the earlier of receipt, one business day following delivery to a recognized courier service or three business days following mailing by registered or certified mail.

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17. GOVERNING LAW.

This Agreement shall be construed and enforced in accordance with the law of the State of Delaware, without giving effect to the conflict of law principles thereof. For the purpose of litigating any dispute that arises under this Agreement, the parties hereby consent to exclusive jurisdiction in Massachusetts and agree that such litigation shall be conducted in the courts of Middlesex County, Massachusetts or the federal courts of the United States for the District of Massachusetts, sitting in Boston, Massachusetts.

18. BENEFIT OF AGREEMENT.

Subject to the provisions of the Plan and the other provisions hereof, this Agreement shall be for the benefit of and shall be binding upon the heirs, executors, administrators, successors and assigns of the parties hereto.

19. ENTIRE AGREEMENT.

This Agreement, together with the Plan, embodies the entire agreement and understanding between the parties hereto with respect to the subject matter hereof and supersedes all prior oral or written agreements and understandings relating to the subject matter hereof. No statement, representation, warranty, covenant or agreement not expressly set forth in this Agreement shall affect or be used to interpret, change or restrict, the express terms and provisions of this Agreement, provided, however, in any event, this Agreement shall be subject to and governed by the Plan.

20. MODIFICATIONS AND AMENDMENTS.

The terms and provisions of this Agreement may be modified or amended as provided in the Plan.

21. WAIVERS AND CONSENTS.

Except as provided in the Plan, the terms and provisions of this Agreement may be waived, or consent for the departure therefrom granted, only by written document executed by the party entitled to the benefits of such terms or provisions. No such waiver or consent shall be deemed to be or shall constitute a waiver or consent with respect to any other terms or provisions of this Agreement, whether or not similar. Each such waiver or consent shall be effective only in the specific instance and for the purpose for which it was given, and shall not constitute a continuing waiver or consent.

22. DATA PRIVACY.

By entering into this Agreement, the Employee: (i) authorizes the Company and each Affiliate, and any agent of the Company or any Affiliate administering the Plan or providing Plan record keeping services, to disclose to the Company or any of its Affiliates such information and data as the Company or any such Affiliate shall request in order to facilitate the grant of options and the administration of the Plan; (ii) waives any data privacy rights he or she may have with respect to such information; and (iii) authorizes the Company and each Affiliate to store and transmit such information in electronic form.

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23. CONSENT OF SPOUSE.

If the Employee is married as of the date of this Agreement, the Employee's spouse shall execute a Consent of Spouse in the form of Exhibit B hereto, effective as of the date hereof. Such consent shall not be deemed to confer or convey to the spouse any rights in the Shares that do not otherwise exist by operation of law or the agreement of the parties. If the Employee marries or remarries subsequent to the date hereof, the Employee shall, not later than 60 days thereafter, obtain his or her new spouse's acknowledgement of and consent to the existence and binding effect of Section 12.2 of this Agreement by such spouse's executing and delivering a Consent of Spouse in the form of Exhibit B.

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IN WITNESS WHEREOF, the Company has caused this Incentive Stock Option Agreement to be executed by its duly authorized officer, and the Employee has hereunto set his or her hand, all as of the day and year first above written.

**GENETIX PHARMACEUTICALS INC.**

By: \_\_\_\_\_  
Name:  
Title:

\_\_\_\_\_  
Employee Name:



*NOTICE OF EXERCISE OF INCENTIVE STOCK OPTION*

*[Form for Unregistered Shares]*

To: Genetix Pharmaceuticals Inc.

Ladies and Gentlemen:

I hereby exercise my Incentive Stock Option to purchase \_\_\_\_\_ shares (the "Shares") of the common stock, \$0.01 par value, of Genetix Pharmaceuticals Inc. (the "Company"), at the exercise price of \$ \_\_\_\_\_ per share, pursuant to and subject to the terms of that certain Incentive Stock Option Agreement between the undersigned and the Company dated \_\_\_\_\_, 20\_\_.

I am aware that the Shares have not been registered under the Securities Act of 1933, as amended (the "1933 Act"), or any state securities laws. I understand that the reliance by the Company on exemptions under the 1933 Act is predicated in part upon the truth and accuracy of the statements by me in this Notice of Exercise.

I hereby represent and warrant that (1) I have been furnished with all information which I deem necessary to evaluate the merits and risks of the purchase of the Shares; (2) I have had the opportunity to ask questions concerning the Shares and the Company and all questions posed have been answered to my satisfaction; (3) I have been given the opportunity to obtain any additional information I deem necessary to verify the accuracy of any information obtained concerning the Shares and the Company; and (4) I have such knowledge and experience in financial and business matters that I am able to evaluate the merits and risks of purchasing the Shares and to make an informed investment decision relating thereto.

I hereby represent and warrant that I am purchasing the Shares for my own personal account for investment and not with a view to the sale or distribution of all or any part of the Shares.

I understand that because the Shares have not been registered under the 1933 Act, I must continue to bear the economic risk of the investment for an indefinite time and the Shares cannot be sold unless the Shares are subsequently registered under applicable federal and state securities laws or an exemption from such registration requirements is available.

I agree that I will in no event sell or distribute or otherwise dispose of all or any part of the Shares unless (1) there is an effective registration statement under the 1933 Act and applicable state securities laws covering any such transaction involving the Shares or (2) the Company receives an opinion of my legal counsel (concurring in by legal counsel for the Company) stating that such transaction is exempt from registration or the Company otherwise satisfies itself that such transaction is exempt from registration.

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I consent to the placing of a legend on my certificate for the Shares stating that the Shares have not been registered and setting forth the restriction on transfer contemplated hereby and to the placing of a stop transfer order on the books of the Company and with any transfer agents against the Shares until the Shares may be legally resold or distributed without restriction.

I understand that at the present time Rule 144 of the Securities and Exchange Commission (the "SEC") may not be relied on for the resale or distribution of the Shares by me. I understand that the Company has no obligation to me to register the sale of the Shares with the SEC and has not represented to me that it will register the sale of the Shares.

I understand the terms and restrictions on the right to dispose of the Shares set forth in the 2002 Employee, Director and Consultant Stock Plan and the Incentive Stock Option Agreement, both of which I have carefully reviewed. I consent to the placing of a legend on my certificate for the Shares referring to such restriction and the placing of stop transfer orders until the Shares may be transferred in accordance with the terms of such restrictions.

I have considered the Federal, state and local income tax implications of the exercise of my Option and the purchase and subsequent sale of the Shares.

I am paying the option exercise price for the Shares as follows:

Please issue the stock certificate for the Shares (check one):

to me; or

to me and \_\_\_\_\_, as joint tenants with right of survivorship and mail the certificate to me at the following address:

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My mailing address for shareholder communications, if different from the address listed above is:

Very truly yours.

\_\_\_\_\_  
Employee (signature)

\_\_\_\_\_  
Print Name

\_\_\_\_\_  
Date

\_\_\_\_\_  
Social Security Number

**Form for Registered Shares**

NOTICE OF EXERCISE OF INCENTIVE STOCK OPTION

TO: Genetix Pharmaceuticals Inc.

IMPORTANT NOTICE: This form of Notice of Exercise may only be used at such time as the Company has filed a Registration Statement with the Securities and Exchange Commission under which the issuance of the Shares for which this exercise is being made is registered and such Registration Statement remains effective.

Ladies and Gentlemen:

I hereby exercise my Incentive Stock Option to purchase \_\_\_\_\_ shares (the "Shares") of the common stock, \$0.01 par value, of Genetix Pharmaceuticals Inc. (the "Company"), at the exercise price of \$ \_\_\_\_\_ per share, pursuant to and subject to the terms of that certain Incentive Stock Option Agreement between the undersigned and the Company dated \_\_\_\_\_, 20\_\_.

I understand the nature of the investment I am making and the financial risks thereof. I am aware that it is my responsibility to have consulted with competent tax and legal advisors about the relevant national, state and local income tax and securities laws affecting the exercise of the Option and the purchase and subsequent sale of the Shares.

I am paying the option exercise price for the Shares as follows:

Please issue the Shares (check one):

- to me; or
- to me and \_\_\_\_\_, as joint tenants with right of survivorship

at the following address:

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My mailing address for shareholder communications, if different from the address listed above is:

Very truly yours.

\_\_\_\_\_  
Employee (signature)

\_\_\_\_\_  
Print Name

\_\_\_\_\_  
Date

\_\_\_\_\_  
Social Security Number

**CONSENT OF SPOUSE**

I, \_\_\_\_\_, spouse of \_\_\_\_\_, acknowledge that I have read the Incentive Stock Option Agreement dated as of \_\_\_\_\_, 20\_\_\_\_ (the "Agreement") to which this Consent is attached as Exhibit B and that I know its contents. Capitalized terms used and not defined herein shall have the meanings assigned to such terms in the Agreement. I am aware that by its provisions the Shares granted to my spouse pursuant to the Agreement are subject to a right of repurchase in favor of Genetix Pharmaceuticals Inc. (the "Company") and that, accordingly, the Company has the right to repurchase up to all of the Shares of which I may become possessed as a result of a gift from my spouse or a court decree and/or any property settlement in any domestic litigation.

I hereby agree that my interest, if any, in the Shares subject to the Agreement shall be irrevocably bound by the Agreement and further understand and agree that any community property interest I may have in the Shares shall be similarly bound by the Agreement.

I agree to the repurchase right described in Section 12.2 of the Agreement and I hereby consent to the repurchase of the Shares by the Company and the sale of the Shares by my spouse or my spouse's legal representative in accordance with the provisions of the Agreement. Further, as part of the consideration for the Agreement, I agree that at my death, if I have not disposed of any interest of mine in the Shares by an outright bequest of the Shares to my spouse, then the Company shall have the same rights against my legal representative to exercise its rights of repurchase with respect to any interest of mine in the Shares as it would have had pursuant to the Agreement if I had acquired the Shares pursuant to a court decree in domestic litigation.

I AM AWARE THAT THE LEGAL, FINANCIAL AND RELATED MATTERS CONTAINED IN THE AGREEMENT ARE COMPLEX AND THAT I AM FREE TO SEEK INDEPENDENT PROFESSIONAL GUIDANCE OR COUNSEL WITH RESPECT TO THIS CONSENT. I HAVE EITHER SOUGHT SUCH GUIDANCE OR COUNSEL OR DETERMINED AFTER REVIEWING THE AGREEMENT CAREFULLY THAT I WILL WAIVE SUCH RIGHT.

Dated as of the \_\_\_\_\_ day of \_\_\_\_\_, 20\_\_\_\_.

\_\_\_\_\_  
Print name:

**RESTRICTED STOCK AGREEMENT  
GENETIX PHARMACEUTICALS INC.**

**AGREEMENT** made as of the \_\_\_\_\_ day of \_\_\_\_\_, 200\_ (the "**Grant Date**"), between Genetix Pharmaceuticals Inc. (the "**Company**"), a Delaware corporation having its principal place of business in Cambridge, Massachusetts, and \_\_\_\_\_ of \_\_\_\_\_, (the "**Employee**").

**WHEREAS**, the Company has adopted the Genetix Pharmaceuticals Inc. 2002 Employee, Director and Consultant Stock Plan (the "**Plan**") to promote the interests of the Company by providing an incentive for employees, directors and consultants of the Company or its Affiliates;

**WHEREAS**, the parties hereto understand and agree that any terms used and not defined herein have the meanings ascribed to such terms in the Plan and that any and all references herein to employment of the Employee by the Company shall include the Employee's employment of the Company or any Affiliate;

**WHEREAS**, pursuant to the exercise of an option granted to the Employee pursuant to a Incentive Stock Option Agreement dated \_\_\_\_\_ by and between the Company and the Employee issued under the Plan (the "**Option Agreement**"), which Option Agreement and Plan are hereby incorporated herein by reference, the Employee has elected to purchase \_\_\_\_\_ shares of the Company's common stock, \$.001 par value per share ("**Common Stock**"), which have not become vested under the vesting schedule set forth in the Option Agreement (the "**Unvested Shares**"). The Unvested Shares and the Shares subject to the Option Agreement that have become vested are sometimes collectively referred to herein as the "**Shares**."

**WHEREAS**, as required by the Option Agreement, as a condition to the Employee's election to exercise the option, the Employee must execute this Agreement, which sets forth the rights and obligations of the parties with respect to the Unvested Shares acquired upon exercise of the option pursuant to the Option Agreement; and

**WHEREAS**, Employee wishes to accept said offer in accordance with the provisions of the Plan, all on the terms and conditions hereinafter set forth.

**NOW, THEREFORE**, in consideration of the premises and the mutual covenants contained herein and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties hereto hereby agree as follows:

1. Company's Lapsing Repurchase Right.

(a) Lapsing Repurchase Right. Except as set forth in Subsection 1(b) hereof, in the event that for any reason the Employee no longer is an employee of the Company, the Company (or its designee) shall have the option, but not the obligation, to purchase from the Employee (or the Employee's Survivor), and, in the event the Company exercises such option,

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the Employee (or the Employee's Survivor) shall be obligated to sell to the Company (or its designee), at a price per share equal to the Purchase Price, all or any part of the Unvested Shares determined as of the date of such termination of employment (the "**Lapsing Repurchase Right**"). The Lapsing Repurchase Right with respect to the Unvested Shares shall terminate as to such Unvested Shares in accordance with the vesting schedule set forth in Section 3 of the Option Agreement. The Company's Lapsing Repurchase Right shall be valid for a period of one year commencing with the date of such termination of employment or service. Notwithstanding any other provision hereof, in the event the Company is prohibited during such one year period from exercising its Lapsing Repurchase Right by applicable law, then the time period during which such Lapsing Repurchase Right may be exercised shall be extended until 30 days after the Company is first not so prohibited.

(b) Effect of Termination for Cause. Notwithstanding anything to the contrary contained in this Agreement, in the event the Company terminates the Employee's employment for "**cause**" (as defined in the Plan) or in the event the Administrator determines, within 90 days after the Employee's termination, that either prior or subsequent to the Employee's termination the Employee engaged in conduct that would constitute "cause," the Company shall have the option to repurchase all of the Unvested Shares acquired by the Employee hereunder at the Purchase Price irrespective of whether the Lapsing Repurchase Right has lapsed in accordance with the vesting schedule set forth in Section 3 of the Option Agreement.

(c) Effect of Termination for Disability or upon Death. Except as otherwise provided in Subsection 1(b) above, the following rules apply if the Employee ceases to be an employee of the Company by reason of Disability or death: to the extent the Company's Lapsing Repurchase Right has not lapsed as of the date of Disability or death, as case may be, the Company may exercise such Lapsing Repurchase Right; provided, however, that the Company's Lapsing Repurchase Right shall be deemed to have lapsed to the extent of a pro rata portion of the Unvested Shares through the date of Disability or death, as would have lapsed had the Employee not become Disabled or died, as the case may be. The proration shall be based upon the number of days accrued in such current vesting period prior to the Employee's date of Disability or death, as the case may be.

(d) Closing. In the event that the Company exercises the Lapsing Repurchase Right, the Company shall notify the Employee, or, in the case of the Employee's death, his or her Survivor, in writing of its intent to repurchase the Unvested Shares. Such notice may be mailed by the Company up to and including the last day of the time period provided for above for exercise of the Lapsing Repurchase Right. The notice shall specify the place, time and date for payment of the repurchase price (the "**Closing**") and the number of Unvested Shares with respect to which the Company is exercising the Lapsing Repurchase Right. The Closing shall be not less than ten days nor



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more than 60 days from the date of mailing of the notice, and the Employee or the Employee's Survivor with respect to the Unvested Shares which the Company elects to repurchase shall have no further rights as the owner thereof from and after the date specified in the notice. At the Closing, the repurchase price shall be delivered to the Employee or the Employee's Survivor and the Unvested Shares being repurchased, duly endorsed for transfer, shall, to the extent that they are not then in the possession of the Company, be delivered to the Company by the Employee or the Employee's Survivor.

(e) Escrow. The certificates representing all Unvested Shares acquired by the Employee hereunder which from time to time are subject to the Lapsing Repurchase Right shall be delivered to the Company and the Company shall hold such Unvested Shares in escrow as provided in this Subsection 1(e). Promptly following receipt by the Company of a written request from the Employee, the Company shall release from escrow and deliver to the Employee a certificate for the whole number of Unvested Shares, if any, as to which the Company's Lapsing Repurchase Right has lapsed. In the event of a repurchase by the Company of Unvested Shares subject to the Lapsing Repurchase Right, the Company shall release from escrow and cancel a certificate for the number of Unvested Shares so repurchased. Any securities distributed in respect of the Unvested Shares held in escrow, including, without limitation, shares issued as a result of stock splits, stock dividends or other recapitalizations, shall also be held in escrow in the same manner as the Unvested Shares.

(f) Prohibition on Transfer. The Employee recognizes and agrees that all Unvested Shares which are subject to the Lapsing Repurchase Right may not be sold, transferred, assigned, hypothecated, pledged, encumbered or otherwise disposed of, whether voluntarily or by operation of law, other than to the Company (or its designee). However, the Employee, with the approval of the Administrator, may transfer the Unvested Shares for no consideration to or for the benefit of the Employee's Immediate Family (including, without limitation, to a trust for the benefit of the Employee's Immediate Family or to a partnership or limited liability company for one or more members of the Employee's Immediate Family), subject to such limits as the Administrator may establish, and the transferee shall remain subject to all the terms and conditions applicable to this Agreement prior to such transfer and each such transferee shall so acknowledge in writing as a condition precedent to the effectiveness of such transfer. The term "**Immediate Family**" shall mean the Employee's spouse, former spouse, parents, children, stepchildren, adoptive relationships, sisters, brothers, nieces and nephews and grandchildren (and, for this purpose, shall also include the Employee. The Company shall not be required to transfer any Unvested Shares on its books which shall have been sold, assigned or otherwise transferred in violation of this Subsection 1(f), or to treat as the owner of such Unvested Shares, or to accord the right to vote as such owner or to pay dividends to, any person or organization to which any such Unvested Shares shall have been so sold,

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assigned or otherwise transferred, in violation of this Subsection 1(f). The Employee further recognizes and agrees that all Shares remain subject to Section 12 of the Option Agreement, except that with respect to the Unvested Shares subject to the Lapsing Repurchase Right, this Agreement will supercede Section 12.2 of the Option Agreement.

(g) Failure to Deliver Unvested Shares to be Repurchased. In the event that the Unvested Shares to be repurchased by the Company under this Agreement are not in the Company's possession pursuant to Subsection 1(e) above or otherwise and the Employee or the Employee's Survivor fails to deliver such Unvested Shares to the Company (or its designee), the Company may elect (i) to establish a segregated account in the amount of the repurchase price, such account to be turned over to the Employee or the Employee's Survivor upon delivery of such Unvested Shares, and (ii) immediately to take such action as is appropriate to transfer record title of such Unvested Shares from the Employee to the Company (or its designee) and to treat the Employee and such Unvested Shares in all respects as if delivery of such Unvested Shares had been made as required by this Agreement. The Employee hereby irrevocably grants the Company a power of attorney which shall be coupled with an interest for the purpose of effectuating the preceding sentence.

(h) Additional Securities. If the Company shall pay a stock dividend or declare a stock split on or with respect to any of its Common Stock, or otherwise distribute securities of the Company to the holders of its Common Stock, the number of shares of stock or other securities of the Company issued with respect to the Unvested Shares then subject to the restrictions contained in this Agreement shall be added to the Unvested Shares subject to the Company's rights of repurchase pursuant to this Agreement. If the Company shall distribute to its stockholders securities of another corporation, the securities of such other corporation, distributed with respect to the Unvested Shares then subject to the restrictions contained in this Agreement, shall be added to the Unvested Shares subject to the Company's rights of repurchase pursuant to this Agreement.

If the outstanding shares of the Company's Common Stock shall be subdivided into a greater number of shares or combined into a smaller number of shares, or in the event of a reclassification of the outstanding shares of the Company's Common Stock, or if the Company shall be a party to a merger, consolidation or capital reorganization, there shall be substituted for the Unvested Shares then subject to the restrictions contained in this Agreement such amount and kind of securities as are issued in such subdivision, combination, reclassification, merger, consolidation or capital reorganization in respect of the Unvested Shares subject immediately prior thereto to the Company's rights of repurchase pursuant to this Agreement.

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2. Legend. In addition to any legend required pursuant to the Plan, all certificates representing the Unvested Shares to be issued to the Employee pursuant to this Agreement shall have endorsed thereon a legend substantially as follows:

“The shares represented by this certificate are subject to restrictions set forth in a Restricted Stock Agreement dated as of \_\_\_\_\_, 200\_ with this Company, a copy of which Agreement is available for inspection at the offices of the Company or will be made available upon request.”

3. Purchase for Investment. If the offering and sale of the Unvested Shares have not been effectively registered under the Securities Act of 1933, as amended, the Employee hereby represents and warrants that he or she is acquiring the Unvested Shares for his or her own account, for investment, and not with a view to, or for sale in connection with, the distribution of any such Unvested Shares.

4. Provisions of Agreement Controlling. The Employee specifically understands and agrees that the Unvested Shares issued under the Plan are being sold to the Employee pursuant to the Plan and the Option Agreement, a copy of which Plan and Option Agreement the Employee acknowledges he or she has read and understands and by which Plan and Option Agreement he or she agrees to be bound. The provisions of the Plan and the Option Agreement are incorporated herein by reference. In the event of a conflict between the terms and conditions of the Plan, this Agreement and the Option Agreement, the provisions of this Agreement will control.

5. Tax Liability of the Employee and Payment of Taxes. The Employee acknowledges and agrees that any income or other taxes due from the Employee with respect to the Unvested Shares issued pursuant to this Agreement, including, without limitation, the Lapsing Repurchase Right, shall be the Employee's responsibility. Without limiting the foregoing, the Employee agrees that, to the extent that the lapsing of restrictions on disposition of any of the Unvested Shares or the declaration of dividends on any such shares before the lapse of such restrictions on disposition results in the Employee's being deemed to be in receipt of earned income under the provisions of the Code, the Company shall be entitled to immediate payment from the Employee of the amount of any tax required to be withheld by the Company.

Upon execution of this Agreement, the Employee may file an election under Section 83 of the Code in substantially the form attached as Attachment I. The Employee acknowledges that if she does not file such an election within 30 days of the date of this Agreement, as the Unvested Shares are released from the Lapsing Repurchase Right in accordance with Section 1, the Employee will have income for tax purposes equal to the fair market value of the Unvested Shares at such date, less the price paid for the Unvested Shares by the Employee.

6. Securities Law Compliance. The Employee specifically acknowledges and agrees that any sales of Unvested Shares shall be made in accordance with the requirements of the Securities Act of 1933, as amended, in a transaction as to which the Company shall have received an opinion of counsel satisfactory to it confirming such compliance.

7. Equitable Relief. The Employee specifically acknowledges and agrees that in the event of a breach or threatened breach of the provisions of this Agreement, the Option Agreement or the Plan, including the attempted transfer of the Unvested Shares by the Employee in violation of this Agreement or the Option Agreement, monetary damages may not be adequate

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to compensate the Company, and, therefore, in the event of such a breach or threatened breach, in addition to any right to damages, the Company shall be entitled to equitable relief in any court having competent jurisdiction. Nothing herein shall be construed as prohibiting the Company from pursuing any other remedies available to it for any such breach or threatened breach.

8. No Obligation to Employ. The Company is not by the Plan, the Option Agreement or this Agreement obligated to continue the Employee as an employee of the Company or an Affiliate.

9. Notices. Any notices required or permitted by the terms of this Agreement, the Option Agreement or the Plan shall be given by recognized courier service, facsimile, registered or certified mail, return receipt requested, addressed as follows:

If to the Company:                President  
   Genetix Pharmaceuticals Inc.  
   840 Memorial Drive, 5<sup>th</sup> floor  
   Cambridge, MA 02139

If to the Employee:                At the address set forth on the first page of this Agreement

or to such other address or addresses of which notice in the same manner has previously been given. Any such notice shall be deemed to have been given on the earliest of receipt, one business day following delivery by the sender to a recognized courier service, or three business days following mailing by registered or certified mail.

10. Binding Effect. This Agreement shall be for the benefit of and shall be binding upon the parties hereto, upon their respective successors and assigns and upon the Employee's heirs, executors, administrators.

11. Governing Law. This Agreement shall be construed and enforced in accordance with the law of the State of Delaware, without giving effect to the conflict of law principles thereof. For the purpose of litigating any dispute that arises under this Agreement, the parties hereby consent to exclusive jurisdiction in Massachusetts and agree that such litigation shall be conducted in the courts of Middlesex County, Massachusetts or the federal courts of the United States for the District of Massachusetts, sitting in Boston, Massachusetts.

12. Severability. If any provision of this Agreement is held to be invalid or unenforceable by a court of competent jurisdiction, then such provision or provisions shall be modified to the extent necessary to make such provision valid and enforceable, and to the extent that this is impossible, then such provision shall be deemed to be excised from this Agreement, and the validity, legality and enforceability of the rest of this Agreement shall not be affected thereby.

13. Entire Agreement. This Agreement, together with the Plan and the Option Agreement, constitutes the entire agreement and understanding between the parties hereto with respect to the subject matter hereof and supersedes all prior oral or written agreements and understandings relating to the subject matter hereof. No statement, representation, warranty, covenant or agreement not expressly set forth in this Agreement, the Option Agreement or the Plan shall affect or be used to interpret, change or restrict the express terms and provisions of this Agreement provided, however, in any event, this Agreement shall be subject to and governed by the Plan.

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14. Modifications and Amendments; Waivers and Consents. The terms and provisions of this Agreement may be modified or amended as provided in the Plan. Except as provided in the Plan, the terms and provisions of this Agreement may be waived, or consent for the departure therefrom granted, only by written document executed by the party entitled to the benefits of such terms or provisions. No such waiver or consent shall be deemed to be or shall constitute a waiver or consent with respect to any other terms or provisions of this Agreement, whether or not similar. Each such waiver or consent shall be effective only in the specific instance and for the purpose for which it was given, and shall not constitute a continuing waiver or consent.

15. Consent of Spouse. If the Employee is married as of the date of this Agreement, the Employee's spouse shall execute a Consent of Spouse in the form of Attachment II hereto, effective as of the date hereof. Such consent shall not be deemed to confer or convey to the spouse any rights in the Unvested Shares that do not otherwise exist by operation of law or the agreement of the parties. If the Employee marries or remarries subsequent to the date hereof, the Employee shall, not later than 60 days thereafter, obtain his or her new spouse's acknowledgement of and consent to the existence and binding effect of all restrictions contained in this Agreement by such spouse's executing and delivering a Consent of Spouse in the form of Exhibit A.

16. Counterparts. This Agreement may be executed in one or more counterparts, and by different parties hereto on separate counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

[THE NEXT PAGE IS THE SIGNATURE PAGE]

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**IN WITNESS WHEREOF**, the parties hereto have executed this Restricted Stock Agreement as of the day and year first above written.

**GENETIX PHARMACEUTICALS INC.**

By: \_\_\_\_\_  
Name:  
Title:

**EMPLOYEE:**

\_\_\_\_\_  
Print name:

**Election to Include Gross Income in Year  
of Transfer Pursuant to Section 83(b)  
of the Internal Revenue Code of 1986, as amended**

In accordance with Section 83(b) of the Internal Revenue Code of 1986, as amended (the “ Code”), the undersigned hereby elects to include in his gross income as compensation for services the excess, if any, of the fair market value of the property (described below) at the time of transfer over the amount paid for such property.

The following sets for the information required in accordance with the Code and the regulations promulgated hereunder:

1. The name, address and social security number of the undersigned are:

Name:

Address:

Social Security No.:

2. The description of the property with respect to which the election is being made is as follows:

\_\_\_\_\_(\_\_\_\_\_) shares (the “**Shares**”) of Common Stock, \$.001 par value per share, of Genetix Pharmaceuticals Inc., a Delaware corporation (the “**Company**”).

3. This election is made for the calendar year \_\_\_\_\_, with respect to the transfer of the property to the Taxpayer on \_\_\_\_\_.

4. Description of restrictions: The property is subject to the following restrictions:

In the event taxpayer’s employment with the Company or an Affiliate is terminated, the Company may repurchase all or any portion of the Shares determined as set forth below at the acquisition price paid by the taxpayer:

A. If the termination takes place on or prior to \_\_\_\_\_, the Purchase Option will apply to all of the Shares.

B. If the termination takes place after \_\_\_\_\_, 200\_, the number of Shares to which the Purchase Option applies shall be \_\_\_\_\_(\_\_\_\_\_) Shares less \_\_\_\_\_(\_\_\_\_\_) Shares for each month elapsed after \_\_\_\_\_, 200\_ if the taxpayer is employed by the Company or an Affiliate.

5. The fair market value at time of transfer (determined without regard to any restrictions other than restrictions which by their terms will never lapse) of the property with respect to which this election is being made was not more than \$ \_\_\_\_\_ per Share.

6. The amount paid by taxpayer for said property was \$ \_\_\_\_\_ per Share.

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7. A copy of this statement has been furnished to the Company.

Signed this \_\_\_\_\_ day of \_\_\_\_\_, 200\_.

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Print name:



**CONSENT OF SPOUSE**

I, \_\_\_\_\_, spouse of \_\_\_\_\_, acknowledge that I have read the RESTRICTED STOCK AGREEMENT dated as of \_\_\_\_\_, 200\_ (the “**Agreement**”) to which this Consent is attached as Attachment II and that I know its contents. Capitalized terms used and not defined herein shall have the meanings assigned to such terms in the Agreement. I am aware that by its provisions the Unvested Shares granted to my spouse pursuant to the Agreement are subject to a Lapsing Repurchase Right in favor of Genetix Pharmaceuticals Inc. (the “**Company**”) and that, accordingly, the Company has the right to repurchase up to all of the Unvested Shares of which I may become possessed as a result of a gift from my spouse or a court decree and/or any property settlement in any domestic litigation.

I hereby agree that my interest, if any, in the Unvested Shares subject to the Agreement shall be irrevocably bound by the Agreement and further understand and agree that any community property interest I may have in the Invested Shares shall be similarly bound by the Agreement.

I agree to the Lapsing Repurchase Right described in the Agreement and I hereby consent to the repurchase of the Unvested Shares by the Company and the sale of the Unvested Shares by my spouse or my spouse’s legal representative in accordance with the provisions of the Agreement. Further, as part of the consideration for the Agreement, I agree that at my death, if I have not disposed of any interest of mine in the Unvested Shares by an outright bequest of the Unvested Shares to my spouse, then the Company shall have the same rights against my legal representative to exercise its rights of repurchase with respect to any interest of mine in the Unvested Shares as it would have had pursuant to the Agreement if I had acquired the Unvested Shares pursuant to a court decree in domestic litigation.

I AM AWARE THAT THE LEGAL, FINANCIAL AND RELATED MATTERS CONTAINED IN THE AGREEMENT ARE COMPLEX AND THAT I AM FREE TO SEEK INDEPENDENT PROFESSIONAL GUIDANCE OR COUNSEL WITH RESPECT TO THIS CONSENT. I HAVE EITHER SOUGHT SUCH GUIDANCE OR COUNSEL OR DETERMINED AFTER REVIEWING THE AGREEMENT CAREFULLY THAT I WILL WAIVE SUCH RIGHT.

Dated as of the \_\_\_\_\_ day of \_\_\_\_\_, 200\_.

\_\_\_\_\_  
Print name:

**BLUEBIRD BIO, INC.**  
**FOURTH AMENDMENT TO THE**  
**2010 STOCK OPTION AND GRANT PLAN**

The bluebird bio, Inc. 2010 Stock Option and Grant Plan (the "Plan") is hereby amended by the Board of Directors and stockholders of bluebird bio, Inc., a Delaware corporation, as follows:

Section 3(a) of the Plan is hereby amended by deleting the first sentence of Section 3(a) and substituting therefor the following:

"The maximum number of shares of Stock reserved and available for issuance under the Plan shall be the sum of (i) 81,953,382 shares and (ii) such number of shares as equals that number of stock options or awards returned to the Company's 2002 Employee, Director and Consultant Stock Plan, as amended and in effect from time to time, after January 16, 2013 as a result of the expiration, cancellation or termination of such stock options or awards, subject to adjustment as provided in Section 3(b)."

ADOPTED BY BOARD OF DIRECTORS: January 16, 2013

ADOPTED BY STOCKHOLDERS: January 16, 2013

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**BLUEBIRD BIO, INC.**  
**THIRD AMENDMENT TO THE**  
**2010 STOCK OPTION AND GRANT PLAN**

The bluebird bio, Inc. 2010 Stock Option and Grant Plan (the "Plan") is hereby amended by the Board of Directors and stockholders of bluebird bio, Inc., a Delaware corporation, as follows:

Section 3(a) of the Plan is hereby amended by deleting the first sentence of Section 3(a) and substituting therefor the following:

"The maximum number of shares of Stock reserved and available for issuance under the Plan shall be the sum of (i) 65,322,771 shares and (ii) such number of shares as equals that number of stock options or awards returned to the Company's 2002 Employee, Director and Consultant Stock Plan, as amended and in effect from time to time, after July 23, 2012 as a result of the expiration, cancellation or termination of such stock options or awards, subject to adjustment as provided in Section 3(b)."

ADOPTED BY BOARD OF DIRECTORS: July 23, 2012

ADOPTED BY STOCKHOLDERS: July 23, 2012

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**BLUEBIRD BIO, INC.**  
**SECOND AMENDMENT TO THE**  
**2010 STOCK OPTION AND GRANT PLAN**

The bluebird bio, Inc. 2010 Stock Option and Grant Plan, as amended (the “Plan”), is hereby amended by the Board of Directors and stockholders of bluebird bio, Inc., a Delaware corporation, as follows:

Section 3(a) of the Plan is hereby amended by deleting the first sentence of Section 3(a) and substituting therefor the following:

“The maximum number of shares of Stock reserved and available for issuance under the Plan shall be the sum of (i) 46,136,871 shares and (ii) such number of shares as equals that number of stock options or awards returned to the Company’s 2002 Employee, Director and Consultant Stock Plan, as amended and in effect from time to time, after the Effective Date (September 10, 2010) as a result of the expiration, cancellation or termination of such stock options or awards, subject to adjustment as provided in Section 3(b).”

ADOPTED BY BOARD OF DIRECTORS: April 24, 2012

ADOPTED BY STOCKHOLDERS: May 7, 2012

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**BLUEBIRD BIO, INC.**  
**FIRST AMENDMENT TO THE**  
**2010 STOCK OPTION AND GRANT PLAN**

The bluebird bio, Inc. 2010 Stock Option and Grant Plan (the "Plan") is hereby amended by the Board of Directors and stockholders of bluebird bio, Inc., a Delaware corporation, as follows:

Section 3(a) of the Plan is hereby amended by deleting the first sentence of Section 3(a) and substituting therefor the following:

"The maximum number of shares of Stock reserved and available for issuance under the Plan shall be the sum of (i) 41,636,871 shares and (ii) such number of shares as equals that number of stock options or awards returned to the Company's 2002 Employee, Director and Consultant Stock Plan, as amended and in effect from time to time, after the Effective Date as a result of the expiration, cancellation or termination of such stock options or awards, subject to adjustment as provided in Section 3(b)."

ADOPTED BY BOARD OF DIRECTORS: April 15, 2011

ADOPTED BY STOCKHOLDERS: April 15, 2011

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BLUEBIRD BIO, INC.

2010 STOCK OPTION AND GRANT PLAN

SECTION 1. GENERAL PURPOSE OF THE PLAN; DEFINITIONS

The name of the plan is the bluebird bio, Inc. 2010 Stock Option and Grant Plan (the "Plan"). The purpose of the Plan is to encourage and enable the officers, employees, directors, Consultants and other key persons (including prospective employees, but conditioned on their employment) of bluebird bio, Inc. (f/k/a Genetix Pharmaceuticals, Inc.), a Delaware corporation (including any successor entity, the "Company") and any Subsidiary, upon whose judgment, initiative and efforts the Company largely depends for the successful conduct of its business, to acquire a proprietary interest in the Company.

The following terms shall be defined as set forth below:

"*Affiliate*" of any Person means a Person that directly or indirectly, through one or more intermediaries, controls, is controlled by or is under common control with the first mentioned Person. A Person shall be deemed to control another Person if such first Person possesses directly or indirectly the power to direct, or cause the direction of, the management and policies of the second Person, whether through the ownership of voting securities, by contract or otherwise.

"*Award*" or "*Awards*," except where referring to a particular category of grant under the Plan, shall include Incentive Stock Options, Non-Qualified Stock Options, Restricted Stock Awards, Unrestricted Stock Awards, Restricted Stock Units or any combination of the foregoing.

"*Award Agreement*" means a written or electronic agreement setting forth the terms and provisions applicable to an Award granted under the Plan. Each Award Agreement may contain terms and conditions in addition to those set forth in the Plan; *provided, however*, that except to the extent explicitly provided to the contrary, in the event of any conflict in the terms of the Plan and the Award Agreement, the terms of the Plan shall govern.

"*Bankruptcy*" shall mean (i) the filing of a voluntary petition under any bankruptcy or insolvency law, or a petition for the appointment of a receiver or the making of an assignment for the benefit of creditors, with respect to the Holder, (ii) the Holder being subjected involuntarily to such a petition or assignment or to an attachment or other legal or equitable interest with respect to the Holder's assets, which involuntary petition or assignment or attachment is not discharged within 60 days after its date, or (iii) the Holder being subject to a transfer of its Issued Shares or Award(s) by operation of law (including by divorce, even if not insolvent), except by reason of death.

"*Board*" means the Board of Directors of the Company.

"*Cause*" shall have the meaning as set forth in the Award Agreement(s). In the case that any Award Agreement does not contain a definition of "*Cause*," it shall mean (i) the grantee's dishonest statements or acts with respect to the Company or any Affiliate of the Company, or

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any of the Company's current or prospective customers, suppliers vendors or other third parties with which such entity does business; (ii) the grantee's commission of (A) a felony or (B) any misdemeanor involving moral turpitude, deceit, dishonesty or fraud; (iii) the grantee's failure to perform his assigned duties and responsibilities to the reasonable satisfaction of the Company which failure continues, in the reasonable judgment of the Company, after written notice given to the grantee by the Company; (iv) the grantee's gross negligence, willful misconduct or insubordination with respect to the Company or any Affiliate of the Company; or (v) the grantee's violation of any provision of any agreement(s) between the grantee and the Company relating to noncompetition, nondisclosure and/or assignment of inventions.

"*Chief Executive Officer*" means the Chief Executive Officer of the Company or, if there is no Chief Executive Officer, then the President of the Company.

"*Code*" means the Internal Revenue Code of 1986, as amended, and any successor Code, and related rules, regulations and interpretations.

"*Committee*" means the Committee of the Board referred to in Section 2.

"*Consultant*" means any natural person that provides bona fide services to the Company (including a Subsidiary), and such services are not in connection with the offer or sale of securities in a capital-raising transaction and do not directly or indirectly promote or maintain a market for the Company's securities.

"*Effective Date*" means the date on which the Plan is adopted as set forth on the final page of the Plan.

"*Exchange Act*" means the Securities Exchange Act of 1934, as amended, and the rules and regulations thereunder.

"*Fair Market Value*" of the Stock on any given date means the fair market value of the Stock determined in good faith by the Committee based on the reasonable application of a reasonable valuation method not inconsistent with Section 409A of the Code. If the Stock is admitted to trade on a national securities exchange, the determination shall be made by reference to the closing price. If the date for which Fair Market Value is determined is the first day when trading prices for the Stock are reported on a national securities exchange, the Fair Market Value shall be the "Price to the Public" (or equivalent) set forth on the cover page for the final prospectus relating to the Company's Initial Public Offering.

"*Grant Date*" means the date that the Committee designates in its approval of an Award in accordance with applicable law as the date on which the Award is granted, which date may not precede the date of such Committee approval.

"*Holder*" means, with respect to an Award or any Issued Shares, the Person holding such Award or Issued Shares, including the initial recipient of the Award or any Permitted Transferee.

"*Incentive Stock Option*" means any Stock Option designated and qualified as an "incentive stock option" as defined in Section 422 of the Code.

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“*Incumbent Directors*” shall mean directors who either (A) are directors of the Company as of the date this Plan is first adopted by the Board, or (B) are elected, or nominated for election, to the Board with the affirmative votes of at least a majority of the Incumbent Directors at the time of such election or nomination (but shall not include an individual whose election or nomination is in connection with an actual or threatened proxy contest relating to the election of directors to the Company).

“*Initial Public Offering*” means the consummation of the first firm commitment underwritten public offering pursuant to an effective registration statement under the Securities Act covering the offer and sale by the Company of its equity securities, as a result of or following which the Stock shall be publicly held.

“*Issued Shares*” means, collectively, all outstanding Shares issued pursuant to Restricted Stock Awards, Unrestricted Stock Awards and Restricted Stock Units and all Option Shares.

“*NASDAQ*” means the NASDAQ Stock Market LLC.

“*Non-Qualified Stock Option*” means any Stock Option that is not an Incentive Stock Option.

“*Option*” or “*Stock Option*” means any option to purchase shares of Stock granted pursuant to Section 5.

“*Option Shares*” means outstanding shares of Stock that were issued to a Holder upon the exercise of a Stock Option.

“*Permitted Transferees*” shall mean any of the following to whom a Holder may transfer Issued Shares hereunder (as set forth in Section 9(a)(ii)(A)): the Holder’s child, stepchild, grandchild, parent, stepparent, grandparent, spouse, former spouse, sibling, niece, nephew, mother-in-law, father-in-law, son-in-law, daughter-in-law, brother-in-law, or sister-in-law, including adoptive relationships, any person sharing the Holder’s household (other than a tenant or employee), a trust in which these persons have more than fifty percent of the beneficial interest, a foundation in which these persons control the management of assets, and any other entity in which these persons own more than fifty percent of the voting interests ; *provided, however*, that any such trust does not require or permit distribution of any Issued Shares during the term of the Award Agreement unless subject to its terms. Upon the death of the Holder, the term Permitted Transferees shall also include such deceased Holder’s estate, executors, administrators, personal representatives, heirs, legatees and distributees, as the case may be.

“*Person*” shall mean any individual, corporation, partnership (limited or general), limited liability company, limited liability partnership, association, trust, joint venture, unincorporated organization or any similar entity.

“*Repurchase Event*” means (i) a Sale Event or (ii) the Holder’s Bankruptcy.

“*Restricted Stock Award*” means Awards granted pursuant to Section 6 and “*Restricted Stock*” means Shares granted pursuant to such Awards.



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“*Restricted Stock Unit*” means an Award of phantom stock units to a grantee, which may be settled in cash or stock as determined by the Committee, pursuant to Section 8.

“*Sale Event*” means the consummation of (i) the dissolution or liquidation of the Company, (ii) the sale of all or substantially all of the assets of the Company on a consolidated basis to an unrelated person or entity, (iii) a merger, reorganization or consolidation involving the Company in which the shares of voting stock of the Company outstanding immediately prior to such transaction represent or are converted into or exchanged for securities of the surviving or resulting entity immediately upon completion of such transaction which represent less than 50 percent of the outstanding voting power of such surviving or resulting entity, (iv) the acquisition of all or a majority of the outstanding voting stock of the Company in a single transaction or a series of related transactions by a Person or group of Persons, (v) a change in the composition of the Board, as a result of which fewer than a majority of the directors are Incumbent Directors, or (vi) any other acquisition of the business of the Company, as determined by the Board; *provided, however*, that the Company’s Initial Public Offering, any subsequent public offering or another capital raising event, or a merger effected solely to change the Company’s domicile shall not constitute a “Sale Event.”

“*Section 409A*” means Section 409A of the Code and the regulations and other guidance promulgated thereunder.

“*Securities Act*” means the Securities Act of 1933, as amended, and the rules and regulations thereunder.

“*Service Relationship*” means any relationship as a full-time employee, part-time employee, director or other key person (including Consultants) of the Company or any Subsidiary or any successor entity; provided that a change in status (e.g., from full-time employee to part-time employee or from full-time employee to Consultant or otherwise) shall, unless otherwise provided in the Award Agreement, constitute a termination of the Award recipient’s Service Relationship with the Company and its Subsidiaries.

“*Shares*” means shares of Stock.

“*Stock*” means the Common Stock, par value \$0.01 per share, of the Company.

“*Subsidiary*” means any corporation or other entity (other than the Company) in which the Company has more than a 50 percent interest, either directly or indirectly.

“*Ten Percent Owner*” means an employee who owns or is deemed to own (by reason of the attribution rules of Section 424(d) of the Code) more than 10 percent of the combined voting power of all classes of stock of the Company or any parent of the Company or any Subsidiary.

“*Termination Event*” means the termination of the Award recipient’s Service Relationship with the Company and its Subsidiaries for any reason whatsoever, regardless of the circumstances thereof, and including, without limitation, upon death, disability, retirement, discharge or resignation for any reason, whether voluntarily or involuntarily. The following shall not constitute a Termination Event: (i) a transfer to the service of the Company from a Subsidiary or from the Company to a Subsidiary, or from one Subsidiary to another Subsidiary

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or (ii) an approved leave of absence for military service or sickness, or for any other purpose approved by the Committee, if the individual's right to re-employment is guaranteed either by a statute or by contract or under the policy pursuant to which the leave of absence was granted or if the Committee otherwise so provides in writing.

*"Unrestricted Stock Award"* means any Award granted pursuant to Section 7 and *"Unrestricted Stock"* means Shares granted pursuant to such Awards.

**SECTION 2. ADMINISTRATION OF PLAN; COMMITTEE AUTHORITY TO SELECT GRANTEES AND DETERMINE AWARDS**

(a) Administration of Plan. The Plan shall be administered by the Board, or at the discretion of the Board, by a committee of the Board, comprised of not less than two Directors. All references herein to the "Committee" shall be deemed to refer to the group then responsible for administration of the Plan at the relevant time (i.e., either the Board of Directors or a committee or committees of the Board, as applicable).

(b) Powers of Committee. The Committee shall have the power and authority to grant Awards consistent with the terms of the Plan, including the power and authority:

(i) to select the individuals to whom Awards may from time to time be granted;

(ii) to determine the time or times of grant, and the amount, if any, of Incentive Stock Options, Non-Qualified Stock Options, Restricted Stock Awards, Unrestricted Stock Awards, Restricted Stock Units, or any combination of the foregoing, granted to any one or more grantees;

(iii) to determine the number of shares of Stock to be covered by any Award and, subject to the provisions of Section 5(a)(i) below, the price, exercise price, conversion ratio or other price relating thereto;

(iv) to determine and, subject to Section 12, to modify from time to time the terms and conditions, including restrictions, not inconsistent with the terms of the Plan, of any Award, which terms and conditions may differ among individual Awards and grantees, and to approve the form of Award Agreements;

(v) to accelerate at any time the exercisability or vesting of all or any portion of any Award;

(vi) to impose any limitations on Awards granted under the Plan, including limitations on transfers, repurchase provisions and the like, and to exercise repurchase rights or obligations;

(vii) subject to any restrictions imposed by Section 409A, to extend at any time the period in which Stock Options may be exercised; and

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(viii) at any time to adopt, alter and repeal such rules, guidelines and practices for administration of the Plan and for its own acts and proceedings as it shall deem advisable; to interpret the terms and provisions of the Plan and any Award (including related written instruments); to make all determinations it deems advisable for the administration of the Plan; to decide all disputes arising in connection with the Plan; and to otherwise supervise the administration of the Plan.

All decisions and interpretations of the Committee shall be binding on all persons, including the Company and Plan grantees.

(c) Award Agreement. Awards under the Plan shall be evidenced by Award Agreements that set forth the terms, conditions and limitations for each Award.

(d) Indemnification. Neither the Board nor the Committee, nor any member of either or any delegate thereof, shall be liable for any act, omission, interpretation, construction or determination made in good faith in connection with the Plan, and the members of the Board and the Committee (and any delegate thereof) shall be entitled in all cases to indemnification and reimbursement by the Company in respect of any claim, loss, damage or expense (including, without limitation, reasonable attorneys' fees) arising or resulting therefrom to the fullest extent permitted by law and/or under the Company's governing documents, including its certificate of incorporation or bylaws, or any directors' and officers' liability insurance coverage which may be in effect from time to time and/or any indemnification agreement between such individual and the Company.

(e) Foreign Award Recipients. Notwithstanding any provision of the Plan to the contrary, in order to comply with the laws in other countries in which the Company and any Subsidiary operate or have employees or other individuals eligible for Awards, the Committee, in its sole discretion, shall have the power and authority to: (i) determine which Subsidiaries, if any, shall be covered by the Plan; (ii) determine which individuals, if any, outside the United States are eligible to participate in the Plan; (iii) modify the terms and conditions of any Award granted to individuals outside the United States to comply with applicable foreign laws; (iv) establish subplans and modify exercise procedures and other terms and procedures, to the extent the Committee determines such actions to be necessary or advisable (and such subplans and/or modifications shall be attached to the Plan as appendices); provided, however, that no such subplans and/or modifications shall increase the share limitation contained in Section 3(a) hereof; and (v) take any action, before or after an Award is made, that the Committee determines to be necessary or advisable to obtain approval or comply with any local governmental regulatory exemptions or approvals.

### SECTION 3. STOCK ISSUABLE UNDER THE PLAN; MERGERS AND OTHER TRANSACTIONS; SUBSTITUTION

(a) Stock Issuable. The maximum number of shares of Stock reserved and available for issuance under the Plan shall be the sum of (i) 26,436,861 shares and (ii) such number of shares as equals that number of stock options or awards returned to the Company's 2002 Employee, Director and Consultant Stock Plan, as amended and in effect from time to time, after the Effective Date as a result of the expiration, cancellation or termination of such stock options

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or awards, subject to adjustment as provided in Section 3(b). For purposes of this limitation, the shares of Stock underlying any Awards that are forfeited, canceled, withheld upon exercise of an Option or settlement of an Award to cover the exercise price or tax withholding, reacquired by the Company prior to vesting, satisfied without the issuance of Stock or otherwise terminated (other than by exercise), in each case shall be added back to the shares of Stock available for issuance under the Plan. Subject to such overall limitations, shares of Stock may be issued up to such maximum number pursuant to any type or types of Award. The shares available for issuance under the Plan may be authorized but unissued shares of Stock or shares of Stock reacquired by the Company.

(b) Changes in Stock. Subject to Section 3(c) hereof, if, as a result of any reorganization, recapitalization, reclassification, stock dividend, stock split, reverse stock split or other similar change in the Company's capital stock, the outstanding shares of Stock are increased or decreased or are exchanged for a different number or kind of shares or other securities of the Company, or additional shares or new or different shares or other securities of the Company or other non-cash assets are distributed with respect to such shares of Stock or other securities, or, if, as a result of any merger or consolidation, or sale of all or substantially all of the assets of the Company, the outstanding shares of Stock are converted into or exchanged for securities of the Company or any successor entity (or a parent or subsidiary thereof), the Committee shall make an appropriate and equitable or proportionate adjustment in (i) the maximum number of shares reserved for issuance under the Plan, (ii) the number and kind of shares or other securities subject to any then outstanding Awards under the Plan, (iii) the repurchase price, if any, per share subject to each outstanding Award, and (iv) the exercise price for each share subject to any then outstanding Stock Options under the Plan, without changing the aggregate exercise price (i.e., the exercise price multiplied by the number of Stock Options) as to which such Stock Options remain exercisable. The adjustment by the Committee shall be final, binding and conclusive. No fractional shares of Stock shall be issued under the Plan resulting from any such adjustment, but the Committee in its discretion may make a cash payment in lieu of fractional shares.

(c) Sale Events.

(i) Options.

(A) In the case of and subject to the consummation of a Sale Event, the Plan and all Options issued hereunder shall terminate upon the effective time of any such Sale Event unless provision is made in connection with the Sale Event for the assumption or continuation of Options theretofore granted by the successor entity, or the substitution of such Options with new Options of the successor entity or parent thereof, with an equitable or proportionate adjustment as to the number and kind of shares and, if appropriate, the per share exercise prices, as such parties shall agree (after taking into account any acceleration hereunder and/or pursuant to the terms of any Award Agreement).

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(B) In the event of the termination of the Plan and all Options issued hereunder pursuant to Section 3(c), each Holder of Options shall be permitted, within a specified period of time prior to the consummation of the Sale Event as determined by the Committee, to exercise all such Options which are then exercisable or will become exercisable as of the effective time of the Sale Event; *provided, however*, that the exercise of Options not exercisable prior to the Sale Event shall be subject to the consummation of the Sale Event.

(C) Notwithstanding anything to the contrary in Section 3(c)(i)(A), in the event of a Sale Event, the Company shall have the right, but not the obligation, to make or provide for a cash payment to the grantees holding Options in exchange for the cancellation thereof, in an amount equal to the difference between (A) the value as determined by the Committee of the consideration payable per share of Stock pursuant to the Sale Event (the "Sale Price") times the number of shares of Stock subject to outstanding Options (to the extent then vested and exercisable, including by reason of acceleration in connection with such Sale Event, at prices not in excess of the Sale Price) and (B) the aggregate exercise price of all such outstanding vested Options.

(ii) Option Shares. Unless otherwise provided in an Award Agreement, in the case of and subject to the consummation of a Sale Event, Option Shares shall be subject to the repurchase right set forth in Section 9(c)(i).

(iii) Restricted Stock and Restricted Stock Unit Awards.

(A) In the case of and subject to the consummation of a Sale Event, all Restricted Stock and Restricted Stock Unit Awards issued hereunder shall be forfeited immediately prior to the effective time of any such Sale Event unless provision is made in connection with the Sale Event for the assumption or continuation of such Awards by the successor entity, or the substitution of such Awards with new Awards of the successor entity or parent thereof, with an equitable or proportionate adjustment as to the number and kind of shares subject to such Awards as such parties shall agree (after taking into account any acceleration hereunder and/or pursuant to the terms of any Award Agreement).

(B) In the event of the forfeiture of shares of Restricted Stock issued hereunder pursuant to Section 3(c)(iii)(A), such shares of Restricted Stock shall be repurchased from the Holder thereof at a price per share equal to the lower of the original per share purchase price paid by the recipient (subject to adjustment as provided in Section 3(b)) or the current Fair Market Value of such shares, determined immediately prior to the effective time of the Sale Event.

(C) Notwithstanding anything to the contrary in Section 3(c)(iii)(A), in the event of a Sale Event, the Company shall have the right, but not the obligation, to make or provide for a cash payment to the grantees holding Restricted Stock or Restricted Stock Unit Awards in exchange for the cancellation thereof, in an amount equal to the Sale Price times the number of shares of Stock subject to such Awards, to be paid at the time of such Sale Event or upon the later vesting of such Awards.

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(iv) Unrestricted Stock Awards. Unless otherwise provided in an Award Agreement, any shares of Unrestricted Stock shall be treated in a Sale Event the same as all other Shares then outstanding.

#### SECTION 4. ELIGIBILITY

Grantees under the Plan will be such full or part-time officers and other employees, directors, Consultants and key persons (including prospective employees, but conditioned on their employment) of the Company and any Subsidiary who are selected from time to time by the Committee in its sole discretion; provided, however, that an Incentive Stock Option may be granted only to a person who, at the time the Incentive Stock Option is granted, is an employee of the Company or any Subsidiary.

#### SECTION 5. STOCK OPTIONS

Upon the grant of a Stock Option, the Company and the grantee shall enter into a Stock Option Award Agreement. The terms and conditions of each such Stock Option Award Agreement shall be determined by the Committee, and such terms and conditions may differ among individual Awards and grantees.

Stock Options granted under the Plan may be either Incentive Stock Options or Non-Qualified Stock Options. Incentive Stock Options may be granted only to employees of the Company or any Subsidiary that is a "subsidiary corporation" within the meaning of Section 424(f) of the Code. To the extent that any Option does not qualify as an Incentive Stock Option, it shall be deemed a Non-Qualified Stock Option.

(a) Terms of Stock Options. The Committee in its discretion may grant Stock Options to eligible officers, employees, directors, Consultants and key persons of the Company or any Subsidiary. Stock Options granted pursuant to this Section 5(a) shall be subject to the following terms and conditions and shall contain such additional terms and conditions, not inconsistent with the terms of the Plan, as the Committee shall deem desirable.

(i) Exercise Price. The exercise price per share for the Stock covered by a Stock Option granted pursuant to Section 5(a) shall be determined by the Committee at the time of grant but shall not be less than 100 percent of the Fair Market Value on the Grant Date. In the case of an Incentive Stock Option that is granted to a Ten Percent Owner, the option price of such Incentive Stock Option shall not be less than 110 percent of the Fair Market Value on the Grant Date.

(ii) Option Term. The term of each Stock Option shall be fixed by the Committee, but no Stock Option shall be exercisable more than ten years after the date the Stock Option is granted. In the case of an Incentive Stock Option that is granted to a Ten Percent Owner, the term of such Stock Option shall be no more than five years from the Grant Date.

(iii) Exercisability; Rights of a Stockholder. Stock Options shall become exercisable and/or vested at such time or times, whether or not in installments, as shall be determined by the Committee at or after the Grant Date. The Award Agreement may permit an optionee to exercise all or a portion of a Stock Option immediately at grant; provided that the

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Option Shares issued upon such exercise shall be subject to restrictions and a vesting schedule identical to the vesting schedule of the related Stock Option and the optionee shall be required to enter into a Restricted Stock Award Agreement and any other similar documentation required by the Company as a condition to exercise of such Stock Option. An optionee shall have the rights of a stockholder only as to shares acquired upon the exercise of a Stock Option and not as to unexercised Stock Options. An optionee shall not be deemed to have acquired any such shares unless and until a Stock Option shall have been exercised pursuant to the terms hereof and the optionee's name shall have been entered on the books of the Company as a stockholder.

(iv) Method of Exercise. Stock Options may be exercised by an optionee in whole or in part, by the optionee giving written notice of exercise to the Company, specifying the number of shares to be purchased. Payment of the purchase price may be made by one or more of the following methods (or any combination thereof) to the extent provided in the Option Award Agreement:

(A) In cash, by certified or bank check, by wire transfer of immediately available funds, or other instrument acceptable to the Committee;

(B) If permitted by the Committee, by the optionee delivering to the Company a promissory note, if the Board has expressly authorized the loan of funds to the optionee for the purpose of enabling or assisting the optionee to effect the exercise of his or her Stock Option; provided, that at least so much of the exercise price as represents the par value of the Stock shall be paid other than with a promissory note if required by state law;

(C) If permitted by the Committee and the Initial Public Offering has occurred (or the Stock otherwise becomes publicly-traded), through the delivery (or attestation to the ownership) of shares of Stock that have been purchased by the optionee on the open market or that are beneficially owned by the optionee and are not then subject to restrictions under any Company plan. To the extent required to avoid variable accounting treatment under FAS 123R or other applicable accounting rules, such surrendered shares if originally purchased from the Company shall have been owned by the optionee for at least six months. Such surrendered shares shall be valued at Fair Market Value on the exercise date;

(D) If permitted by the Committee and the Initial Public Offering has occurred (or the Stock otherwise becomes publicly-traded), by the optionee delivering to the Company a properly executed exercise notice together with irrevocable instructions to a broker to promptly deliver to the Company cash or a check payable and acceptable to the Company for the purchase price; provided that in the event the optionee chooses to pay the purchase price as so provided, the optionee and the broker shall comply with such procedures and enter into such agreements of indemnity and other agreements as the Committee shall prescribe as a condition of such payment procedure; and

(E) If permitted by the Committee, with respect to Stock Options that are not Incentive Stock Options, by a "net exercise" arrangement pursuant to which the Company will reduce the number of shares of Stock issuable upon exercise by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price.

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Payment instruments will be received subject to collection. No certificates for shares of Stock so purchased will be issued to the optionee or, with respect to uncertificated Stock, no transfer to the optionee on the records of the Company will take place, until the Company has completed all steps required by law to be taken in connection with the issuance and sale of the shares, which steps may include, without limitation, (i) receipt of a representation from the optionee at the time of exercise of the Option that the optionee is purchasing the shares for the optionee's own account and not with a view to any sale or distribution thereof, (ii) the legending of any certificate (or notation on any book entry) representing the shares to evidence the foregoing restrictions, (iii) obtaining from optionee payment or provision for all withholding taxes due as a result of the exercise of the Option, and (iv) if required by the Company, the optionee shall have entered into any stockholders agreements or other agreements with the Company and/or certain other of the Company's stockholders relating to shares of the Stock. The delivery of certificates representing the shares of Stock (or the transfer to the optionee on the records of the Company with respect to uncertificated Stock) to be purchased pursuant to the exercise of a Stock Option will be contingent upon receipt from the optionee (or a purchaser acting in his or her stead in accordance with the provisions of the Stock Option) by the Company of the full purchase price for such shares and the fulfillment of any other requirements contained in the Option Award Agreement or applicable provisions of laws. In the event an optionee chooses to pay the purchase price by previously-owned shares of Stock through the attestation method, the number of shares of Stock transferred to the optionee upon the exercise of the Stock Option shall be net of the number of shares attested to.

(b) Annual Limit on Incentive Stock Options. To the extent required for "incentive stock option" treatment under Section 422 of the Code, the aggregate Fair Market Value (determined as of the time of grant) of the shares of Stock with respect to which Incentive Stock Options granted under the Plan and any other plan of the Company or its parent and any Subsidiary that become exercisable for the first time by an optionee during any calendar year shall not exceed \$100,000 or such other limit as may be in effect from time to time under Section 422 of the Code. To the extent that any Stock Option exceeds this limit, it shall constitute a Non-Qualified Stock Option.

#### SECTION 6. RESTRICTED STOCK AWARDS

(a) Nature of Restricted Stock Awards. The Committee may, in its sole discretion, grant (or sell at par value or such other purchase price determined by the Committee) to an eligible person under Section 4 hereof a Restricted Stock Award under the Plan. The Committee shall determine the restrictions and conditions applicable to each Restricted Stock Award at the time of grant. Conditions may be based on continuing employment (or other Service Relationship), achievement of pre-established performance goals and objectives and/or such other criteria as the Committee may determine. The grant of a Restricted Stock Award is contingent on the grantee executing a Restricted Stock Award Agreement. The terms and conditions of each such Award Agreement shall be determined by the Committee, and such terms and conditions may differ among individual Awards and grantees, all of whom must be eligible persons under Section 4 hereof.



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(b) Rights as a Stockholder. Upon execution of a Restricted Stock Award Agreement and payment of any applicable purchase price, a grantee of Restricted Stock shall be considered the record owner of and shall be entitled to vote the Shares of Restricted Stock if, and to the extent, such Shares are entitled to voting rights, subject to such conditions contained in the Restricted Stock Award Agreement. The grantee shall be entitled to receive all dividends and any other distributions declared on the Shares; provided, however, that the Company is under no duty to declare any such dividends or to make any such distribution. The Restricted Stock Award Agreement may require or permit the immediate payment, waiver, deferral or investment of dividends paid on the Restricted Stock. Unless the Committee shall otherwise determine, certificates evidencing the Restricted Stock shall remain in the possession of the Company until such Restricted Stock is vested as provided in subsection (d) below of this Section, and the grantee shall be required, as a condition of the grant, to deliver to the Company a stock power endorsed in blank and such other instruments of transfer as the Committee may prescribe.

(c) Restrictions. Restricted Stock may not be sold, assigned, transferred, pledged or otherwise encumbered or disposed of except as specifically provided herein or in the Restricted Stock Award Agreement. Except as may otherwise be provided by the Committee either in the Award Agreement or, subject to Section 12 below, in writing after the Award Agreement is issued, if any, if a grantee's employment (or other Service Relationship) with the Company and any Subsidiary terminates, the Company or its assigns shall have the right, as may be specified in the relevant instrument, to repurchase some or all of the Shares subject to the Award at such purchase price as is set forth in the Restricted Stock Award Agreement.

(d) Vesting of Restricted Stock. The Committee at the time of grant shall specify the date or dates and/or the attainment of pre-established performance goals, objectives and other conditions on which the substantial risk of forfeiture imposed shall lapse and the Restricted Stock shall become vested, subject to such further rights of the Company or its assigns as may be specified in the Restricted Stock Award Agreement.

#### SECTION 7. UNRESTRICTED STOCK AWARDS

The Committee may, in its sole discretion, grant (or sell at par value or such other purchase price determined by the Committee) to an eligible person under Section 4 hereof an Unrestricted Stock Award under the Plan. Unrestricted Stock Awards may be granted in respect of past services or other valid consideration, or in lieu of cash compensation due to such grantee.

#### SECTION 8. RESTRICTED STOCK UNITS

(a) Nature of Restricted Stock Units. The Committee shall determine the restrictions and conditions applicable to each Restricted Stock Unit at the time of grant. Vesting conditions may be based on continuing employment (or other Service Relationship), achievement of pre-established performance goals and objectives and/or other such criteria as the Committee may determine. The grant of Restricted Stock Unit(s) is contingent on the grantee executing a Restricted Stock Unit Award Agreement. The terms and conditions of each such Award Agreement shall be determined by the Committee and may differ among individual Awards and grantees. On or promptly following the vesting date or dates applicable to any Restricted Stock Unit, but in no event later than March 15 of the year following the year in which such vesting occurs, such Restricted Stock Unit(s), shall be settled in the form of cash or shares of Stock, as specified in the Award Agreement.

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(b) Rights as a Stockholder. A grantee shall have the rights of a stockholder only as to shares of Stock, if any, acquired upon settlement of a Restricted Stock Unit. A grantee shall not be deemed to have acquired any such shares unless and until a Restricted Stock Unit shall have been settled in Stock pursuant to the terms hereof, the Company shall have issued and delivered a certificate representing the shares to the grantee, and the grantee's name shall have been entered in the books of the Company as a stockholder.

(c) Termination. Except as may otherwise be provided by the Committee either in the Award Agreement or in writing after the Award Agreement is issued, a grantee's right in all Restricted Stock Units that have not vested shall automatically terminate upon the grantee's termination of employment (or cessation of Service Relationship) with the Company and any Subsidiary for any reason.

#### SECTION 9. TRANSFER RESTRICTIONS; COMPANY RIGHT OF FIRST REFUSAL; COMPANY REPURCHASE RIGHTS

(a) Restrictions on Transfer.

(i) Non-Transferability of Stock Options. No Stock Option shall be transferable by the optionee otherwise than by will or by the laws of descent and distribution and all Stock Options shall be exercisable, during the optionee's lifetime, only by the optionee, or by the optionee's legal representative or guardian in the event of the optionee's incapacity. Notwithstanding the foregoing, the Committee, in its sole discretion, may provide in the Award Agreement regarding a given Stock Option that the optionee may transfer, without consideration for the transfer, his or her Non-Qualified Stock Options to members of his or her immediate family, to trusts for the benefit of such family members, or to partnerships in which such family members are the only partners, provided that the transferee agrees in writing with the Company to be bound by all of the terms and conditions of this Plan and the applicable Option.

(ii) Issued Shares. No Issued Shares shall be sold, assigned, transferred, pledged, hypothecated, given away or in any other manner disposed of or encumbered, whether voluntarily or by operation of law, unless (i) such transfer is in compliance with the terms of the applicable Award Agreement, all applicable securities laws (including, without limitation, the Securities Act), and with the terms and conditions of this Section 9, (ii) such transfer does not cause the Company to become subject to the reporting requirements of the Exchange Act, and (iii) the transferee consents in writing to be bound by the provisions of the Plan, including this Section 9. In connection with any proposed transfer, the Committee may require the transferor to provide at the transferor's own expense an opinion of counsel to the transferor, satisfactory to the Committee, that such transfer is in compliance with all foreign, federal and state securities laws (including, without limitation, the Securities Act). Any attempted disposition of Issued Shares not in accordance with the terms and conditions of this Section 9 shall be null and void, and the Company shall not reflect on its records any change in record ownership of any Issued Shares as a result of any such disposition, shall otherwise refuse to recognize any such disposition and shall not in any way give effect to any such disposition of Issued Shares. Subject

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to the foregoing general provisions, and unless otherwise provided in the applicable Award Agreement, Issued Shares may be transferred pursuant to the following specific terms and conditions (provided that with respect to any transfer of Restricted Stock, all vesting and forfeiture provisions shall continue to apply only with respect to the original recipient):

(A) Transfers to Permitted Transferees. The Holder may sell, assign, transfer or give away any or all of the Issued Shares to Permitted Transferees; *provided, however*, that following such sale, assignment, transfer or gift, such Issued Shares shall continue to be subject to the terms of this Plan (including this Section 9) and such Permitted Transferee(s) shall, as a condition to any such transfer, deliver a written acknowledgment to that effect to the Company. Notwithstanding the foregoing, the Holder may not sell, assign, transfer or give any or all of the Issued Shares to any Person whom the Company reasonably determines is a direct competitor or a potential competitor of the Company or any of its Subsidiaries.

(B) Transfers Upon Death. Upon the death of the Holder, any Issued Shares then held by the Holder at the time of such death and any Issued Shares acquired thereafter by the Holder's legal representative shall be subject to the provisions of this Plan, and the Holder's estate, executors, administrators, personal representatives, heirs, legatees and distributees shall be obligated to convey such Issued Shares to the Company or its assigns under the terms contemplated hereby.

(b) Right of First Refusal. In the event that a Holder desires at any time to sell or otherwise transfer all or any part of such Holder's Issued Shares (other than shares of Restricted Stock which by their terms are not transferrable), the Holder first shall give written notice to the Company of the Holder's intention to make such transfer. Such notice shall state the number of Issued Shares which the Holder proposes to sell (the "Offered Shares"), the price and the terms at which the proposed sale is to be made and the name and address of the proposed transferee. At any time within 30 days after the receipt of such notice by the Company, the Company or its assigns may elect to purchase all or any portion of the Offered Shares at the price and on the terms offered by the proposed transferee and specified in the notice. The Company or its assigns shall exercise this right by mailing or delivering written notice to the Holder within the foregoing 30-day period. If the Company or its assigns elect to exercise its purchase rights under this Section 9(b), the closing for such purchase shall, in any event, take place within 45 days after the receipt by the Company of the initial notice from the Holder. In the event that the Company or its assigns do not elect to exercise such purchase right, or in the event that the Company or its assigns do not pay the full purchase price within such 45-day period, the Holder may, within 60 days thereafter, sell the Offered Shares to the proposed transferee and at the same price and on the same terms as specified in the Holder's notice. Any Shares purchased by such proposed transferee shall no longer be subject to the terms of the Plan. Any Shares not sold to the proposed transferee shall remain subject to the Plan. If the Holder is a party to any stockholders agreements or other agreements with the Company and/or certain other of the Company's stockholders relating to shares of the Stock, (i) the transferring Holder shall comply with the requirements of such stockholders agreements or other agreements relating to any proposed transfer of the Offered Shares, and (ii) any proposed transferee that purchases Offered Shares shall enter into such stockholders agreements or other agreements with the Company and/or certain other of the Company's stockholders relating to the Offered Shares on the same terms and in the same capacity as the transferring Holder.

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(c) Company's Right of Repurchase.

(i) Right of Repurchase for Option Shares. The Company or its assigns shall have the right and option upon a Repurchase Event to repurchase from a Holder of Option Shares some or all (as determined by the Company) of the Option Shares held or subsequently acquired upon exercise of a Stock Option by such Holder at the price per share specified below. Such repurchase right may be exercised by the Company within the later of (A) six months following the date of such Repurchase Event or (B) seven months after the acquisition of such Option Shares upon exercise of a Stock Option (the "Option Shares Repurchase Period"). The "Option Shares Repurchase Price" shall be equal to the Fair Market Value of the Option Shares, determined as of the date the Committee elects to exercise its repurchase rights in connection with a Repurchase Event.

(ii) Right of Repurchase With Respect to Restricted Stock and Shares issued pursuant to an Unrestricted Stock Award or Restricted Stock Unit Award. Unless otherwise set forth in the agreement entered into by the recipient and the Company in connection with a Restricted Stock Award, Unrestricted Stock Award or Restricted Stock Unit Award, the Company or its assigns shall have the right and option upon a Repurchase Event to repurchase from a Holder of Issued Shares received pursuant to a Restricted Stock Award, Unrestricted Stock Award or Restricted Stock Unit Award some or all (as determined by the Company) of such Issued Shares at the price per share specified below. In addition, upon a Termination Event, the Company or its assigns shall have the right and option to repurchase from a Holder of Issued Shares received pursuant to a Restricted Stock Award any Issued Shares which have not vested as of the Termination Event. Such repurchase right may be exercised by the Company within six months following the date of such Repurchase Event or Termination Event as applicable (the "Non-Option Shares Repurchase Period"). The "Non-Option Shares Repurchase Price" shall be (i) in the case of Issued Shares which are vested as of the date of the Repurchase Event, the Fair Market Value of such Issued Shares as of the date the Company elects to exercise its repurchase rights in connection with a Repurchase Event and (ii) in the case of Issued Shares which have not vested as of the date of the Repurchase Event or Termination Event (as applicable), the lower of the original per share purchase price paid by the recipient subject to adjustment as provided in Section 3(b) or the current Fair Market Value of such Issued Shares as of the date the Company elects to exercise its repurchase rights in connection with a Repurchase Event or Termination Event (as applicable).

(iii) Procedure. Any repurchase right of the Company shall be exercised by the Company or its assigns by giving the Holder written notice on or before the last day of the Option Shares Repurchase Period or Non-Option Shares Repurchase Period, as applicable, of its intention to exercise such repurchase right. Upon such notification, the Holder shall promptly surrender to the Company, free and clear of any liens or encumbrances, any certificates representing the Shares being purchased, together with a duly executed stock power for the transfer of such Shares to the Company or the Company's assignee or assignees. Upon the Company's or its assignee's receipt of the certificates from the Holder, the Company or its assignee or assignees shall deliver to him, her or them a check for the Option Shares Repurchase

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Price or the Non-Option Shares Repurchase Price, as applicable; *provided, however*, that the Company may pay the Option Shares Repurchase Price or Non-Option Shares Repurchase Price, as applicable, by offsetting and canceling any indebtedness then owed by the Holder to the Company.

(d) Drag Along Right. In the event the holders of a majority of the Company's equity securities then outstanding (the "Majority Shareholders") determine to enter into a Sale Event in a *bona fide* negotiated transaction (a "Sale"), with any non-Affiliate of the Company or any majority shareholder (in each case, the "Buyer"), a Holder of Issued Shares, including any Permitted Transferees, shall be obligated to and shall upon the written request of the Majority Shareholders: (a) sell, transfer and deliver, or cause to be sold, transferred and delivered, to the Buyer, his or her Issued Shares (including for this purpose all of such Holder's or his or her Permitted Transferee's Issued Shares that presently or as a result of any such transaction may be acquired upon the exercise of an Option (following the payment of the exercise price therefor)) on substantially the same terms applicable to the Majority Shareholders (with appropriate adjustments to reflect the conversion of convertible securities, the redemption of redeemable securities and the exercise of exercisable securities as well as the relative preferences and priorities of preferred stock); and (b) execute and deliver such instruments of conveyance and transfer and take such other action, including voting such Issued Shares in favor of any Sale proposed by the Majority Shareholders and executing any purchase agreements, merger agreements, indemnity agreements, escrow agreements or related documents as the Majority Shareholders or the Buyer may reasonably require in order to carry out the terms and provisions of this Section 9(d).

(e) Escrow Arrangement.

(i) Escrow. In order to carry out the provisions of Sections 9(b), (c), and (d) of this Agreement more effectively, the Company shall hold any Issued Shares in escrow together with separate stock powers executed by the Holder in blank for transfer, and any Permitted Transferee shall, as an additional condition to any transfer of Issued Shares, execute a like stock power as to such Issued Shares. The Company shall not dispose of the Issued Shares except as otherwise provided in this Agreement. In the event of any repurchase by the Company (or any of its assigns), the Company is hereby authorized by the Holder and any Permitted Transferee, as the Holder's and each such Permitted Transferee's attorney-in-fact, to date and complete the stock powers necessary for the transfer of the Issued Shares being purchased and to transfer such Issued Shares in accordance with the terms hereof. At such time as any Issued Shares are no longer subject to the Company's repurchase, first refusal and drag along rights, the Company shall, at the written request of the Holder, deliver to the Holder (or the relevant Permitted Transferee) a certificate representing such Issued Shares with the balance of the Issued Shares to be held in escrow pursuant to this Section 9(e).

(ii) Remedy. Without limitation of any other provision of this Agreement or other rights, in the event that a Holder, any Permitted Transferees or any other Person is required to sell a Holder's Issued Shares pursuant to the provisions of Sections 9(b), (c), or (d) hereof and in the further event that he or she refuses or for any reason fails to deliver to the Company or its designated purchaser of such Issued Shares the certificate or certificates evidencing such Issued Shares together with a related stock power, the Company or such designated purchaser may

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deposit the applicable purchase price for such Issued Shares with a bank designated by the Company, or with the Company's independent public accounting firm, as agent or trustee, or in escrow, for such Holder, any Permitted Transferees or other Person, to be held by such bank or accounting firm for the benefit of and for delivery to him, her, them or it, and/or, in its discretion, pay such purchase price by offsetting any indebtedness then owed by such Holder as provided above. Upon any such deposit and/or offset by the Company or its designated purchaser of such amount and upon notice to the Person who was required to sell the Issued Shares to be sold pursuant to the provisions of Sections 9(b), (c), or (d), such Issued Shares shall at such time be deemed to have been sold, assigned, transferred and conveyed to such purchaser, such Holder shall have no further rights thereto (other than the right to withdraw the payment thereof held in escrow, if applicable), and the Company shall record such transfer in its stock transfer book or in any appropriate manner.

(f) Lockup Provision. A Holder agrees, if requested by the Company and any underwriter engaged by the Company, not to sell or otherwise transfer or dispose of any Issued Shares (including, without limitation, pursuant to Rule 144 under the Securities Act) held by him or her for such period following the effective date of any registration statement of the Company filed under the Securities Act as the Company or such underwriter shall specify reasonably and in good faith. If requested by the underwriter engaged by the Company, each Holder shall execute a separate letter reflecting the agreement set forth in this Section 9(f).

(g) Adjustments for Changes in Capital Structure. If, as a result of any reorganization, recapitalization, reclassification, stock dividend, stock split, reverse stock split or other similar change in the Common Stock, the outstanding shares of Common Stock are increased or decreased or are exchanged for a different number or kind of shares of the Company's Stock, the restrictions contained in this Section 9 shall apply with equal force to additional and/or substitute securities, if any, received by Holder in exchange for, or by virtue of his or her ownership of, Issued Shares.

(h) Termination. The terms and provisions of Section 9(b), Section 9(c) (except for the Company's right to repurchase unvested Restricted Stock Awards upon a Termination Event) and Section 9(d) shall terminate upon the closing of the Company's Initial Public Offering or upon consummation of any Sale Event, in either case as a result of which shares of the Company (or a successor entity) of the same class as the Issued Shares are registered under Section 12 of the Exchange Act and publicly-traded on NASDAQ or any national security exchange.

#### SECTION 10. TAX WITHHOLDING

(a) Payment by Grantee. Each grantee shall, no later than the date as of which the value of an Award or of any Stock or other amounts received thereunder first becomes includable in the gross income of the grantee for Federal income tax purposes, pay to the Company, or make arrangements satisfactory to the Committee regarding payment of, any Federal, state, or local taxes of any kind required by law to be withheld by the Company with respect to such income. The Company and any Subsidiary shall, to the extent permitted by law, have the right to deduct any such taxes from any payment of any kind otherwise due to the grantee. The Company's obligation to deliver stock certificates (or evidence of book entry) to any grantee is subject to and conditioned on any such tax withholding obligations being satisfied by the grantee.

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(b) Payment in Stock. Subject to approval by the Committee, the Company's minimum required tax withholding obligation may be satisfied, in whole or in part, by the Company withholding from shares of Stock to be issued pursuant to any Award a number of shares with an aggregate Fair Market Value (as of the date the withholding is effected) that would satisfy the minimum withholding amount due.

#### SECTION 11. SECTION 409A AWARDS

To the extent that any Award is determined to constitute "nonqualified deferred compensation" within the meaning of Section 409A (a "409A Award"), the Award shall be subject to such additional rules and requirements as specified by the Committee from time to time in order to comply with Section 409A. In this regard, if any amount under a 409A Award is payable upon a "separation from service" (within the meaning of Section 409A) to a grantee who is considered a "specified employee" (within the meaning of Section 409A), then no such payment shall be made prior to the date that is the earlier of (i) six months and one day after the grantee's separation from service, or (ii) the grantee's death, but only to the extent such delay is necessary to prevent such payment from being subject to interest, penalties and/or additional tax imposed pursuant to Section 409A.

#### SECTION 12. AMENDMENTS AND TERMINATION

The Board may, at any time, amend or discontinue the Plan and the Committee may, at any time, amend or cancel any outstanding Award for the purpose of satisfying changes in law or for any other lawful purpose), but no such action shall adversely affect rights under any outstanding Award without the consent of the holder of the Award. The Committee may exercise its discretion to reduce the exercise price of outstanding Stock Options or effect repricing through cancellation of outstanding Awards and by granting such holders new Awards in replacement of the cancelled Awards. To the extent determined by the Committee to be required either by the Code to ensure that Incentive Stock Options granted under the Plan are qualified under Section 422 of the Code or otherwise, Plan amendments shall be subject to approval by the Company stockholders entitled to vote at a meeting of stockholders. Nothing in this Section 12 shall limit the Board's or Committee's authority to take any action permitted pursuant to Section 3(c).

#### SECTION 13. STATUS OF PLAN

With respect to the portion of any Award that has not been exercised and any payments in cash, Stock or other consideration not received by a grantee, a grantee shall have no rights greater than those of a general creditor of the Company unless the Committee shall otherwise expressly so determine in connection with any Award or Awards.

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## SECTION 14. GENERAL PROVISIONS

(a) No Distribution; Compliance with Legal Requirements. The Committee may require each person acquiring Stock pursuant to an Award to represent to and agree with the Company in writing that such person is acquiring the shares of Stock without a view to distribution thereof. No shares of Stock shall be issued pursuant to an Award until all applicable securities law and other legal and stock exchange or similar requirements have been satisfied. The Committee may require the placing of such stop-orders and restrictive legends on certificates for Stock and Awards as it deems appropriate.

(b) Delivery of Stock Certificates. Stock certificates to grantees under the Plan shall be deemed delivered for all purposes when the Company or a stock transfer agent of the Company shall have mailed such certificates in the United States mail, addressed to the grantee, at the grantee's last known address on file with the Company; provided that stock certificates to be held in escrow pursuant to Section 9(e) of the Plan shall be deemed delivered when the Company shall have recorded the issuance in its records. Uncertificated Stock shall be deemed delivered for all purposes when the Company or a Stock transfer agent of the Company shall have given to the grantee by electronic mail (with proof of receipt) or by United States mail, addressed to the grantee, at the grantee's last known address on file with the Company, notice of issuance and recorded the issuance in its records (which may include electronic "book entry" records).

(c) No Employment Rights. The adoption of the Plan and the grant of Awards do not confer upon any Person any right to continued employment or Service Relationship with the Company or any Subsidiary.

(d) Trading Policy Restrictions. Option exercises and other Awards under the Plan shall be subject to the Company's insider trading policy-related restrictions, terms and conditions as may be established by the Committee, or in accordance with policies set by the Committee, from time to time.

(e) Designation of Beneficiary. Each grantee to whom an Award has been made under the Plan may designate a beneficiary or beneficiaries to exercise any Award on or after the grantee's death or receive any payment under any Award payable on or after the grantee's death. Any such designation shall be on a form provided for that purpose by the Committee and shall not be effective until received by the Committee. If no beneficiary has been designated by a deceased grantee, or if the designated beneficiaries have predeceased the grantee, the beneficiary shall be the grantee's estate.

(f) Legend. Any certificate(s) representing the Issued Shares shall carry substantially the following legend (and with respect to uncertificated Stock, the book entries evidencing such shares shall contain the following notation):

The transferability of this certificate and the shares of stock represented hereby are subject to the restrictions, terms and conditions (including repurchase and restrictions against transfers) contained in the bluebird bio, Inc. 2010 Stock Option and Grant Plan and any agreement entered into thereunder by and between the company and the holder of this certificate (a copy of which is available at the offices of the company for examination).



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SECTION 15. EFFECTIVE DATE OF PLAN

The Plan shall become effective upon adoption by the Board and shall be approved by stockholders in accordance with applicable state law and the Company's articles of incorporation and bylaws within 12 months thereafter. Subject to such approval by stockholders and to the requirement that no Stock may be issued hereunder prior to such approval, Stock Options and other Awards may be granted hereunder on and after adoption of the Plan by the Board. No grants of Stock Options and other Awards may be made hereunder after the tenth anniversary of the Effective Date.

SECTION 16. GOVERNING LAW

This Plan, all Awards and any controversy arising out of or relating to this Plan and all Awards shall be governed by and construed in accordance with the General Corporation Law of the State of Delaware as to matters within the scope thereof, and as to all other matters shall be governed by and construed in accordance with the internal laws of the Commonwealth of Massachusetts, without regard to conflict of law principles that would result in the application of any law other than the law of the Commonwealth of Massachusetts.

DATE ADOPTED BY THE BOARD OF DIRECTORS: September 15, 2010

DATE APPROVED BY THE STOCKHOLDERS: October 4, 2010

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**INCENTIVE STOCK OPTION AGREEMENT  
UNDER THE BLUEBIRD BIO, INC.  
2010 STOCK OPTION AND GRANT PLAN**

Name of Optionee: \_\_\_\_\_ (the "Optionee")  
No. of Underlying Shares: \_\_\_\_\_ Shares of Common Stock  
Grant Date: \_\_\_\_\_  
Vesting Commencement Date: \_\_\_\_\_ (the "Vesting Commencement Date")  
Expiration Date: \_\_\_\_\_ (the "Expiration Date")  
Option Exercise Price/Share: \$ \_\_\_\_\_ (the "Option Exercise Price")

Pursuant to the bluebird bio, Inc. 2010 Stock Option and Grant Plan (the "Plan"), bluebird bio, Inc., a Delaware corporation (together with any successor thereto, the "Company"), hereby grants to the Optionee, who is an employee of the Company or any of its Subsidiaries, an option (the "Stock Option") to purchase on or prior to the Expiration Date, or such earlier date as is specified herein, all or any part of the number of shares of Common Stock, par value \$0.01 per share ("Common Stock"), of the Company indicated above (the "Underlying Shares," and such shares once issued shall be referred to as the "Option Shares"), at the Option Exercise Price per share, subject to the terms and conditions set forth in this Incentive Stock Option Agreement (this "Agreement") and in the Plan. This Stock Option is intended to qualify as an "incentive stock option" as defined in Section 422(b) of the Internal Revenue Code of 1986, as amended from time to time (the "Code"). To the extent that any portion of the Stock Option does not so qualify, it shall be deemed a non-qualified stock option.

All capitalized terms used herein and not otherwise defined shall have the respective meanings set forth in the Plan.

1. Vesting, Exercisability and Termination

(a) No portion of this Stock Option may be exercised until such portion shall have vested and become exercisable.

(b) Except as set forth below, and subject to the determination of the Committee in its sole discretion to accelerate the vesting schedule hereunder, this Stock Option shall be vested and exercisable with respect to the Underlying Shares on the respective dates indicated below:

(i) All Underlying Shares shall initially be unvested and unexercisable.

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(ii) [25] percent of the Underlying Shares shall vest and become exercisable on the [first] anniversary of the Vesting Commencement Date; provided that the Optionee continues to have a Service Relationship with the Company at such time.

(iii) Thereafter, the remaining [75] percent of the Underlying Shares shall vest and become exercisable in [36] equal monthly installments at the end of each month following the first anniversary of the Vesting Commencement Date, provided the Optionee continues to have a Service Relationship with the Company at such time.

Notwithstanding anything herein to the contrary in the case of a Sale Event, this Stock Option shall be treated as provided in Section 3(c) of the Plan.

(c) Termination. Except as may otherwise be provided by the Committee, if the Optionee's Service Relationship is terminated, the period within which to exercise this Stock Option will be subject to earlier termination as set forth below (and if not exercised within such period, shall thereafter terminate subject, in each case to Section 3(c) of the Plan):

(i) Termination Due to Death or Disability. If the Optionee's Service Relationship terminates by reason of such Optionee's death or disability (as defined in Section 422(c) of the Code), this Stock Option may be exercised, to the extent exercisable on the date of such termination, by the Optionee, the Optionee's legal representative or legatee for a period of 12 months from the date of death or disability or until the Expiration Date, if earlier.

(ii) Other Termination. If the Optionee's Service Relationship terminates for any reason other than death or disability (as defined in Section 422(c) of the Code), and unless otherwise determined by the Committee, this Stock Option may be exercised, to the extent exercisable on the date of termination, for a period of 90 days from the date of termination or until the Expiration Date or other termination date, if earlier; provided however, if the Optionee's Service Relationship is terminated for Cause, this Stock Option shall terminate immediately upon the date of such termination.

For purposes hereof, the Committee's determination of the reason for termination of the Optionee's Service Relationship shall be conclusive and binding on the Optionee and his or her representatives or legatees. Any portion of this Stock Option that is not exercisable on the date of termination of the Service Relationship shall terminate immediately and be null and void.

(d) It is understood and intended that this Stock Option is intended to qualify as an "incentive stock option" as defined in Section 422 of the Code to the extent permitted under applicable law. Accordingly, the Optionee understands that in order to obtain the benefits of an incentive stock option under Section 422 of the Code, no sale or other disposition may be made of Option Shares for which incentive stock option treatment is desired within the one-year period beginning on the day after the day of the transfer of such Option Shares to him or her, nor within the two-year period beginning on the day after Grant Date of this Stock Option and further that this Stock Option must be exercised within three months after termination of employment as an employee (or 12 months in the case of death or disability) to qualify as an incentive stock option. If the Optionee disposes (whether by sale, gift, transfer or otherwise) of

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any such Option Shares within either of these periods, he or she will notify the Company within 30 days after such disposition. The Optionee also agrees to provide the Company with any information concerning any such dispositions required by the Company for tax purposes. Further, to the extent the Underlying Shares and any other incentive stock options of the Optionee having an aggregate Fair Market Value in excess of \$100,000 (determined as of the Grant Date) first become exercisable in any year, such options will not qualify as incentive stock options.

2. Exercise of Stock Option.

(a) The Optionee may exercise this Stock Option only in the following manner: Prior to the Expiration Date, the Optionee may deliver a Stock Option exercise notice (an "Exercise Notice") in the form of Appendix A hereto indicating his or her election to purchase some or all of the Underlying Shares with respect to which this Stock Option is exercisable at the time of such notice. Such notice shall specify the number of Underlying Shares to be purchased. Payment of the purchase price may be made by one or more of the methods described in Sections 5(a)(iv)(A), (B), (C) or (D) of the Plan, subject to the limitations contained in such Sections of the Plan, including the requirement that the Committee specifically approve in advance certain payment methods.

(b) Notwithstanding any other provision hereof or of the Plan, no portion of this Stock Option shall be exercisable after the Expiration Date.

3. Incorporation of Plan. Notwithstanding anything herein to the contrary, this Stock Option shall be subject to and governed by all the terms and conditions of the Plan.

4. Transferability of Stock Option. This Agreement is personal to the Optionee and is not transferable by the Optionee in any manner other than by will or by the laws of descent and distribution. The Stock Option may be exercised during the Optionee's lifetime only by the Optionee (or by the Optionee's guardian or personal representative in the event of the Optionee's incapacity). The Optionee may elect to designate a beneficiary by providing written notice of the name of such beneficiary to the Company, and may revoke or change such designation at any time by filing written notice of revocation or change with the Company; such beneficiary may exercise the Optionee's Stock Option in the event of the Optionee's death to the extent provided herein. If the Optionee does not designate a beneficiary, or if the designated beneficiary predeceases the Optionee, the legal representative of the Optionee may exercise this Stock Option to the extent provided herein in the event of the Optionee's death.

5. Restrictions on Transfer of Option Shares. The Option Shares acquired upon exercise of the Stock Option shall be subject to certain transfer restrictions and other limitations including, without limitation, the provisions contained in Section 9 of the Plan.

6. Miscellaneous Provisions.

(a) Equitable Relief. The parties hereto agree and declare that legal remedies may be inadequate to enforce the provisions of this Agreement and that equitable relief, including specific performance and injunctive relief, may be used to enforce the provisions of this Agreement.

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(b) Adjustments for Changes in Capital Structure. If, as a result of any reorganization, recapitalization, reincorporation, reclassification, stock dividend, stock split, reverse stock split or other similar change in the Common Stock, the outstanding shares of Common Stock are increased or decreased or are exchanged for a different number or kind of shares of the Company's stock, the restrictions contained in this Agreement shall apply with equal force to additional and/or substitute securities, if any, received by the Optionee in exchange for, or by virtue of his or her ownership of, Option Shares.

(c) Change and Modifications. This Agreement may not be orally changed, modified or terminated, nor shall any oral waiver of any of its terms be effective. This Agreement may be changed, modified or terminated only by an agreement in writing signed by the Company and the Optionee.

(d) Governing Law. This Agreement shall be governed by and construed in accordance with the General Corporation Law of the State of Delaware as to matters within the scope hereof, and as to all other matters shall be governed by and construed in accordance with the internal laws of the Commonwealth of Massachusetts, without regard to conflict of law principles that would result in the application of any law other than the law of the Commonwealth of Massachusetts.

(e) Headings. The headings are intended only for convenience in finding the subject matter and do not constitute part of the text of this Agreement and shall not be considered in the interpretation of this Agreement.

(f) Saving Clause. If any provision(s) of this Agreement shall be determined to be illegal or unenforceable, such determination shall in no manner affect the legality or enforceability of any other provision hereof.

(g) Notices. All notices, requests, consents and other communications shall be in writing and be deemed given when delivered personally, by telex or facsimile transmission or when received if mailed by first class registered or certified mail, postage prepaid. Notices to the Company or the Optionee shall be addressed as set forth underneath their signatures below, or to such other address or addresses as may have been furnished by such party in writing to the other.

(h) Benefit and Binding Effect. This Agreement shall be binding upon and shall inure to the benefit of the parties hereto, their respective successors, permitted assigns, and legal representatives. The Company has the right to assign this Agreement, and such assignee shall become entitled to all the rights of the Company hereunder to the extent of such assignment.

(i) Counterparts. For the convenience of the parties and to facilitate execution, this Agreement may be executed in two or more counterparts, each of which shall be deemed an original, but all of which shall constitute one and the same document.

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7. Dispute Resolution.

(a) Except as provided below, any dispute arising out of or relating to the Plan or this Stock Option, this Agreement, or the breach, termination or validity of the Plan, this Stock Option or this Agreement, shall be finally settled by binding arbitration conducted expeditiously in accordance with the J.A.M.S./Endispute Comprehensive Arbitration Rules and Procedures (the "J.A.M.S. Rules"). The arbitration shall be governed by the United States Arbitration Act, 9 U.S.C. Sections 1-16, and judgment upon the award rendered by the arbitrators may be entered by any court having jurisdiction thereof. The place of arbitration shall be Boston, MA.

(b) The arbitration shall commence within 60 days of the date on which a written demand for arbitration is filed by any party hereto. In connection with the arbitration proceeding, the arbitrator shall have the power to order the production of documents by each party and any third-party witnesses. In addition, each party may take up to three depositions as of right, and the arbitrator may in his or her discretion allow additional depositions upon good cause shown by the moving party. However, the arbitrator shall not have the power to order the answering of interrogatories or the response to requests for admission. In connection with any arbitration, each party to the arbitration shall provide to the other, no later than seven business days before the date of the arbitration, the identity of all persons that may testify at the arbitration and a copy of all documents that may be introduced at the arbitration or considered or used by a party's witness or expert. The arbitrator's decision and award shall be made and delivered within six months of the selection of the arbitrator. The arbitrator's decision shall set forth a reasoned basis for any award of damages or finding of liability. The arbitrator shall not have power to award damages in excess of actual compensatory damages and shall not multiply actual damages or award punitive damages, and each party hereby irrevocably waives any claim to such damages.

(c) The Company, the Optionee, each party to the Agreement and any other holder of Stock issued pursuant to this Agreement (each, a "Party") covenants and agrees that such party will participate in the arbitration in good faith. This Section 7 applies equally to requests for temporary, preliminary or permanent injunctive relief, except that in the case of temporary or preliminary injunctive relief any party may proceed in court without prior arbitration for the limited purpose of avoiding immediate and irreparable harm.

(d) Each Party (i) hereby irrevocably submits to the jurisdiction of any United States District Court of competent jurisdiction for the purpose of enforcing the award or decision in any such proceeding, (ii) hereby waives, and agrees not to assert, by way of motion, as a defense, or otherwise, in any such suit, action or proceeding, any claim that it is not subject personally to the jurisdiction of the above named courts, that its property is exempt or immune from attachment or execution (except as protected by applicable law), that the suit, action or proceeding is brought in an inconvenient forum, that the venue of the suit, action or proceeding is improper or that this Agreement or the subject matter hereof may not be enforced in or by such court, and (iii) hereby waives and agrees not to seek any review by any court of any other jurisdiction which may be called upon to grant an enforcement of the judgment of any such court. Each Party hereby consents to service of process by registered mail at the address to which notices are to be given. Each Party agrees that its, his or her submission to jurisdiction and its, his or her consent to service of process by mail is made for the express benefit of each other Party. Final judgment against any Party in any such action, suit or proceeding may be enforced in other jurisdictions by suit, action or proceeding on the judgment, or in any other manner provided by or pursuant to the laws of such other jurisdiction.

[SIGNATURE PAGE FOLLOWS]

The foregoing Agreement is hereby accepted and the terms and conditions thereof hereby agreed to by the undersigned as of the date first above written.

**BLUEBIRD BIO, INC.**

By: \_\_\_\_\_

Name:

Title:

Address:

840 Memorial Drive  
Cambridge, MA 02139

The undersigned hereby acknowledges receiving and reviewing a copy of the Plan, including, without limitation, Section 9 thereof, and understands that the Stock Option granted hereby is subject to the terms of the Plan and of this Agreement. This Agreement is hereby accepted, and the terms and conditions of the Plan and this Agreement, SPECIFICALLY INCLUDING THE ARBITRATION PROVISIONS IN SECTION 7 OF THIS AGREEMENT, are hereby agreed to, by the undersigned as of the date first above written.

OPTIONEE:

\_\_\_\_\_  
Name:

Address:  
\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

[SPOUSE'S CONSENT

I acknowledge that I have read the  
foregoing Incentive Stock Option Agreement  
and understand the contents thereof.

\_\_\_\_\_ ]

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DESIGNATED BENEFICIARY:

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Beneficiary's Address:

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**Appendix A**

**STOCK OPTION EXERCISE NOTICE**

**BLUEBIRD BIO, INC.**

Attention: [ \_\_\_\_\_ ]  
840 Memorial Drive  
Cambridge, MA 02139

Pursuant to the terms of the stock option agreement between the undersigned and bluebird bio, Inc. (the "Company") dated \_\_\_\_\_ (the "Agreement") under the bluebird bio, Inc. 2010 Stock Option and Grant Plan, I, [Insert Name] \_\_\_\_\_, hereby [Circle One] partially/fully exercise such option by including herein payment in the amount of \$ \_\_\_\_\_ representing the purchase price for [Fill in number of Underlying Shares] \_\_\_\_\_ Underlying Shares. I have chosen the following form(s) of payment:

- 1. Cash
- 2. Certified or bank check payable to "bluebird bio, Inc."
- 3. Other (as referenced in the Agreement and described in the Plan (please describe))

\_\_\_\_\_.

In connection with my exercise of the option as set forth above, I hereby represent and warrant to the Company as follows:

- (i) I am purchasing the Underlying Shares for my own account for investment only, and not for resale or with a view to the distribution thereof.
- (ii) I have had such an opportunity as I have deemed adequate to obtain from the Company such information as is necessary to permit me to evaluate the merits and risks of my investment in the Company and have consulted with my own advisers with respect to my investment in the Company.
- (iii) I have sufficient experience in business, financial and investment matters to be able to evaluate the risks involved in the purchase of the Underlying Shares and to make an informed investment decision with respect to such purchase.
- (iv) I can afford a complete loss of the value of the Option Shares and am able to bear the economic risk of holding such Option Shares for an indefinite period of time.
- (v) I understand that the Option Shares may not be registered under the Securities Act of 1933 (it being understood that the Option Shares are being issued and sold in reliance on the exemption provided in Rule 701 thereunder) or any applicable state securities or "blue sky" laws and may not be sold or otherwise transferred or disposed of in the absence of an effective registration statement under the Securities Act

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of 1933 and under any applicable state securities or “blue sky” laws (or exemptions from the registration requirement thereof). I further acknowledge that certificates representing Option Shares will bear restrictive legends reflecting the foregoing and/or that book entries for uncertificated Option Shares will include similar restrictive notations.

Sincerely yours,

\_\_\_\_\_  
Name:

\_\_\_\_\_  
Address:  
\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

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**EARLY EXERCISE  
INCENTIVE STOCK OPTION AGREEMENT  
UNDER THE BLUEBIRD BIO, INC.  
2010 STOCK OPTION AND GRANT PLAN**

Name of Optionee: \_\_\_\_\_ (the "Optionee")  
No. of Option Shares: \_\_\_\_\_ Shares of Common Stock  
Grant Date: \_\_\_\_\_  
Vesting Commencement Date: \_\_\_\_\_ (the "Vesting Commencement Date")  
Expiration Date: \_\_\_\_\_ (the "Expiration Date")  
Option Exercise Price/Share: \$ \_\_\_\_\_ (the "Option Exercise Price")

Pursuant to the bluebird bio, Inc. 2010 Stock Option and Grant Plan (the "Plan"), bluebird bio, Inc., a Delaware corporation (together with any successor thereto, the "Company"), hereby grants to the Optionee, who is an employee of the Company or any of its Subsidiaries, an option (the "Stock Option") to purchase on or prior to the Expiration Date, or such earlier date as is specified herein, all or any part of the number of shares of Common Stock, par value \$0.01 per share ("Common Stock"), of the Company indicated above (the "Option Shares"), at the Option Exercise Price per share, subject to the terms and conditions set forth in this Early Exercise Incentive Stock Option Agreement (this "Agreement") and in the Plan. This Stock Option is intended to qualify as an "incentive stock option" as defined in Section 422(b) of the Internal Revenue Code of 1986, as amended from time to time (the "Code"). To the extent that any portion of the Stock Option does not so qualify, it shall be deemed a non-qualified stock option.

All capitalized terms used herein and not otherwise defined shall have the respective meanings set forth in the Plan.

1. Vesting, Exercisability and Termination.

(a) This Stock Option shall be immediately exercisable, regardless of whether the Option Shares are vested.

(b) Except as set forth below, and subject to the determination of the Committee in its sole discretion to accelerate the vesting schedule hereunder, the Option Shares shall be vested on the respective dates indicated below:

(i) All Option Shares shall initially be unvested.

(ii) [25] percent of the Option Shares shall vest on the [first] anniversary of the Vesting Commencement Date; provided that the Optionee continues to have a Service Relationship with the Company at such time.

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(iii) Thereafter, the remaining [75] percent of the Option Shares shall vest in [36] equal monthly installments at the end of each month following the first anniversary of the Vesting Commencement Date, provided the Optionee continues to have a Service Relationship with the Company at such time.

Notwithstanding anything herein to the contrary in the case of a Sale Event, this Stock Option and the Option Shares shall be treated as provided in Section 3(c) of the Plan.

(c) Termination. Except as may otherwise be provided by the Committee, if the Optionee's Service Relationship is terminated, the period within which to exercise this Stock Option will be subject to earlier termination as set forth below (and if not exercised within such period, shall thereafter terminate subject, in each case to Section 3(c) of the Plan):

(i) Termination Due to Death or Disability. If the Optionee's Service Relationship terminates by reason of such Optionee's death or disability (as defined in Section 422(c) of the Code), this Stock Option may continue to be exercised, to the extent the Option Shares are vested on the date of termination, by the Optionee, the Optionee's legal representative or legatee for a period of 12 months from the date of death or disability or until the Expiration Date, if earlier.

(ii) Other Termination. If the Optionee's Service Relationship terminates for any reason other than death or disability (as defined in Section 422(c) of the Code), and unless otherwise determined by the Committee, this Stock Option may continue to be exercised, to the extent the Option Shares are vested on the date of termination, for a period of 90 days from the date of termination or until the Expiration Date or other termination date, if earlier; provided however, if the Optionee's Service Relationship is terminated for Cause, this Stock Option shall terminate immediately upon the date of such termination[, **such termination shall be treated as a "Repurchase Event" and the Company shall have the right to repurchase the Shares as set forth in Section 9(c) of the Plan**].

For purposes hereof, the Committee's determination of the reason for termination of the Optionee's Service Relationship shall be conclusive and binding on the Optionee and his or her representatives or legatees. Any portion of this Stock Option with respect to Option Shares that are not vested on the date of termination of the Service Relationship shall terminate immediately and be null and void.

(d) It is understood and intended that this Stock Option is intended to qualify as an "incentive stock option" as defined in Section 422 of the Code to the extent permitted under applicable law. Accordingly, the Optionee understands that in order to obtain the benefits of an incentive stock option under Section 422 of the Code, no sale or other disposition may be made of Option Shares for which incentive stock option treatment is desired within the one-year period beginning on the day after the day of the transfer of such Option Shares to him or her, nor within the two-year period beginning on the day after Grant Date of this Stock Option and further that this Stock Option must be exercised within three months after termination of employment as an employee (or 12 months in the case of death or disability) to qualify as an incentive stock option. If the Optionee disposes (whether by sale, gift, transfer or otherwise) of

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any such Option Shares within either of these periods, he or she will notify the Company within 30 days after such disposition. The Optionee also agrees to provide the Company with any information concerning any such dispositions required by the Company for tax purposes. Further, to the extent the Option Shares and any other incentive stock options of the Optionee having an aggregate Fair Market Value in excess of \$100,000 (determined as of the Grant Date) first become exercisable in any year, such options will not qualify as incentive stock options.

2. Exercise of Stock Option.

(a) The Optionee may exercise this Stock Option only in the following manner: Prior to the Expiration Date, the Optionee may deliver a Stock Option exercise notice (an "Exercise Notice") in the form of Appendix A hereto indicating his or her election to purchase some or all of the Option Shares. Such notice shall specify the number of Option Shares to be purchased. To the extent this Stock Option is only partially exercised, such exercise shall first be with respect to the Option Shares, if any, that have previously vested, and then with respect to the Option Shares that will next vest, with the Option Shares that vest at the latest date being exercised last. Payment of the purchase price may be made by one or more of the methods described in Sections 5(a)(iv)(A), (B), (C) or (D) of the Plan, subject to the limitations contained in such Sections of the Plan, including the requirement that the Committee specifically approve in advance certain payment methods.

(b) In the event the Optionee exercises a portion of this Stock Option with respect to Option Shares that have not vested, the Optionee shall also deliver a Restricted Stock Agreement covering such unvested Option Shares in the form of Appendix B hereto (the "Restricted Stock Agreement") with the same vesting schedule for such Option Shares as set forth for such Option Shares herein.

(c) Notwithstanding any other provision hereof or of the Plan, no portion of this Stock Option shall be exercisable after the Expiration Date.

3. Incorporation of Plan. Notwithstanding anything herein to the contrary, this Stock Option shall be subject to and governed by all the terms and conditions of the Plan.

4. Transferability of Stock Option. This Agreement is personal to the Optionee and is not transferable by the Optionee in any manner other than by will or by the laws of descent and distribution. The Stock Option may be exercised during the Optionee's lifetime only by the Optionee (or by the Optionee's guardian or personal representative in the event of the Optionee's incapacity). The Optionee may elect to designate a beneficiary by providing written notice of the name of such beneficiary to the Company, and may revoke or change such designation at any time by filing written notice of revocation or change with the Company; such beneficiary may exercise the Optionee's Stock Option in the event of the Optionee's death to the extent provided herein. If the Optionee does not designate a beneficiary, or if the designated beneficiary predeceases the Optionee, the legal representative of the Optionee may exercise this Stock Option to the extent provided herein in the event of the Optionee's death.

5. Restrictions on Transfer of Option Shares. The Option Shares acquired upon exercise of the Stock Option shall be subject to certain transfer restrictions and other limitations including, without limitation, the provisions contained in Section 9 of the Plan and, if applicable, the Restricted Stock Agreement.

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6. Miscellaneous Provisions.

(a) Equitable Relief. The parties hereto agree and declare that legal remedies may be inadequate to enforce the provisions of this Agreement and that equitable relief, including specific performance and injunctive relief, may be used to enforce the provisions of this Agreement.

(b) Adjustments for Changes in Capital Structure. If, as a result of any reorganization, recapitalization, reincorporation, reclassification, stock dividend, stock split, reverse stock split or other similar change in the Common Stock, the outstanding shares of Common Stock are increased or decreased or are exchanged for a different number or kind of shares of the Company's stock, the restrictions contained in this Agreement shall apply with equal force to additional and/or substitute securities, if any, received by the Optionee in exchange for, or by virtue of his or her ownership of, Option Shares.

(c) Change and Modifications. This Agreement may not be orally changed, modified or terminated, nor shall any oral waiver of any of its terms be effective. This Agreement may be changed, modified or terminated only by an agreement in writing signed by the Company and the Optionee.

(d) Governing Law. This Agreement shall be governed by and construed in accordance with the General Corporation Law of the State of Delaware as to matters within the scope hereof, and as to all other matters shall be governed by and construed in accordance with the internal laws of the Commonwealth of Massachusetts, without regard to conflict of law principles that would result in the application of any law other than the law of the Commonwealth of Massachusetts.

(e) Headings. The headings are intended only for convenience in finding the subject matter and do not constitute part of the text of this Agreement and shall not be considered in the interpretation of this Agreement.

(f) Saving Clause. If any provision(s) of this Agreement shall be determined to be illegal or unenforceable, such determination shall in no manner affect the legality or enforceability of any other provision hereof.

(g) Notices. All notices, requests, consents and other communications shall be in writing and be deemed given when delivered personally, by telex or facsimile transmission or when received if mailed by first class registered or certified mail, postage prepaid. Notices to the Company or the Optionee shall be addressed as set forth underneath their signatures below, or to such other address or addresses as may have been furnished by such party in writing to the other.

(h) Benefit and Binding Effect. This Agreement shall be binding upon and shall inure to the benefit of the parties hereto, their respective successors, permitted assigns, and legal representatives. The Company has the right to assign this Agreement, and such assignee shall become entitled to all the rights of the Company hereunder to the extent of such assignment.

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(i) Counterparts. For the convenience of the parties and to facilitate execution, this Agreement may be executed in two or more counterparts, each of which shall be deemed an original, but all of which shall constitute one and the same document.

7. Dispute Resolution.

(a) Except as provided below, any dispute arising out of or relating to the Plan or this Stock Option, this Agreement, or the breach, termination or validity of the Plan, this Stock Option or this Agreement, shall be finally settled by binding arbitration conducted expeditiously in accordance with the J.A.M.S./Endispute Comprehensive Arbitration Rules and Procedures (the "J.A.M.S. Rules"). The arbitration shall be governed by the United States Arbitration Act, 9 U.S.C. Sections 1 16, and judgment upon the award rendered by the arbitrators may be entered by any court having jurisdiction thereof. The place of arbitration shall be Boston, MA.

(b) The arbitration shall commence within 60 days of the date on which a written demand for arbitration is filed by any party hereto. In connection with the arbitration proceeding, the arbitrator shall have the power to order the production of documents by each party and any third-party witnesses. In addition, each party may take up to three depositions as of right, and the arbitrator may in his or her discretion allow additional depositions upon good cause shown by the moving party. However, the arbitrator shall not have the power to order the answering of interrogatories or the response to requests for admission. In connection with any arbitration, each party to the arbitration shall provide to the other, no later than seven business days before the date of the arbitration, the identity of all persons that may testify at the arbitration and a copy of all documents that may be introduced at the arbitration or considered or used by a party's witness or expert. The arbitrator's decision and award shall be made and delivered within six months of the selection of the arbitrator. The arbitrator's decision shall set forth a reasoned basis for any award of damages or finding of liability. The arbitrator shall not have power to award damages in excess of actual compensatory damages and shall not multiply actual damages or award punitive damages, and each party hereby irrevocably waives any claim to such damages.

(c) The Company, the Optionee, each party to the Agreement and any other holder of Stock issued pursuant to this Agreement (each, a "Party") covenants and agrees that such party will participate in the arbitration in good faith. This Section 7 applies equally to requests for temporary, preliminary or permanent injunctive relief, except that in the case of temporary or preliminary injunctive relief any party may proceed in court without prior arbitration for the limited purpose of avoiding immediate and irreparable harm.

(d) Each Party (i) hereby irrevocably submits to the jurisdiction of any United States District Court of competent jurisdiction for the purpose of enforcing the award or decision in any such proceeding, (ii) hereby waives, and agrees not to assert, by way of motion, as a defense, or otherwise, in any such suit, action or proceeding, any claim that it is not subject personally to the jurisdiction of the above named courts, that its property is exempt or immune from attachment or execution (except as protected by applicable law), that the suit, action or

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proceeding is brought in an inconvenient forum, that the venue of the suit, action or proceeding is improper or that this Agreement or the subject matter hereof may not be enforced in or by such court, and (iii) hereby waives and agrees not to seek any review by any court of any other jurisdiction which may be called upon to grant an enforcement of the judgment of any such court. Each Party hereby consents to service of process by registered mail at the address to which notices are to be given. Each Party agrees that its, his or her submission to jurisdiction and its, his or her consent to service of process by mail is made for the express benefit of each other Party. Final judgment against any Party in any such action, suit or proceeding may be enforced in other jurisdictions by suit, action or proceeding on the judgment, or in any other manner provided by or pursuant to the laws of such other jurisdiction.

[SIGNATURE PAGE FOLLOWS]



The foregoing Agreement is hereby accepted and the terms and conditions thereof hereby agreed to by the undersigned as of the date first above written.

**BLUEBIRD BIO, INC.**

By: \_\_\_\_\_

Name:

Title:

Address:

840 Memorial Drive

Cambridge, MA 02139

The undersigned hereby acknowledges receiving and reviewing a copy of the Plan, including, without limitation, Section 9 thereof, and understands that the Stock Option granted hereby is subject to the terms of the Plan and of this Agreement. This Agreement is hereby accepted, and the terms and conditions of the Plan and this Agreement, SPECIFICALLY INCLUDING THE ARBITRATION PROVISIONS IN SECTION 7 OF THIS AGREEMENT, are hereby agreed to, by the undersigned as of the date first above written.

OPTIONEE:

\_\_\_\_\_  
Name:

Address:

\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

[SPOUSE'S CONSENT

I acknowledge that I have read the foregoing Incentive Stock Option Agreement and understand the contents thereof.

\_\_\_\_\_ ]

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DESIGNATED BENEFICIARY:

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Beneficiary's Address:

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**Appendix A**

**STOCK OPTION EXERCISE NOTICE**

**BLUEBIRD BIO, INC.**  
Attention: Office Manager  
840 Memorial Drive  
Cambridge, MA 02139

Pursuant to the terms of the stock option agreement between the undersigned and bluebird bio, Inc. (the "Company") dated \_\_\_\_\_ (the "Agreement") under the bluebird bio, Inc. 2010 Stock Option and Grant Plan, I, [Insert Name] \_\_\_\_\_, hereby [Circle One] partially/fully exercise such option by including herein payment in the amount of \$ \_\_\_\_\_ representing the purchase price for [Fill in number of Option Shares] \_\_\_\_\_ Option Shares. I have chosen the following form(s) of payment:

- 1. Cash
- 2. Certified or bank check payable to "bluebird bio, Inc."
- 3. Other (as referenced in the Agreement and described in the Plan (please describe))

\_\_\_\_\_.

In connection with my exercise of the option as set forth above, I hereby represent and warrant to the Company as follows:

- (i) I am purchasing the Option Shares for my own account for investment only, and not for resale or with a view to the distribution thereof.
- (ii) I have had such an opportunity as I have deemed adequate to obtain from the Company such information as is necessary to permit me to evaluate the merits and risks of my investment in the Company and have consulted with my own advisers with respect to my investment in the Company.
- (iii) I have sufficient experience in business, financial and investment matters to be able to evaluate the risks involved in the purchase of the Option Shares and to make an informed investment decision with respect to such purchase.
- (iv) I can afford a complete loss of the value of the Option Shares and am able to bear the economic risk of holding such Option Shares for an indefinite period of time.
- (v) I understand that the Option Shares may not be registered under the Securities Act of 1933 (it being understood that the Option Shares are being issued and sold in reliance on the exemption provided in Rule 701 thereunder) or any applicable state securities or "blue sky" laws and may not be sold or otherwise transferred or disposed of in the absence of an effective registration statement under the Securities Act

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of 1933 and under any applicable state securities or “blue sky” laws (or exemptions from the registration requirement thereof). I further acknowledge that certificates representing Option Shares will bear restrictive legends reflecting the foregoing and/or that book entries for uncertificated Option Shares will include similar restrictive notations.

(vi) To the extent required, I have executed and delivered to the Company the Restricted Stock Agreement attached as Appendix B to the Agreement.

Sincerely yours,

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Name:

Address:

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**Appendix B**

**RESTRICTED STOCK AGREEMENT**

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**NON-QUALIFIED STOCK OPTION AGREEMENT  
UNDER THE BLUEBIRD BIO, INC.  
2010 STOCK OPTION AND GRANT PLAN**

Name of Optionee: \_\_\_\_\_ (the "Optionee")  
No. of Underlying Shares: \_\_\_\_\_ Shares of Common Stock  
Grant Date: \_\_\_\_\_  
Vesting Commencement Date: \_\_\_\_\_ (the "Vesting Commencement Date")  
Expiration Date: \_\_\_\_\_ (the "Expiration Date")  
Option Exercise Price/Share: \$ \_\_\_\_\_ (the "Option Exercise Price")

Pursuant to the bluebird bio, Inc. 2010 Stock Option and Grant Plan (the "Plan"), bluebird bio, Inc., a Delaware corporation (together with any successor thereto, the "Company"), hereby grants to the Optionee, who is an officer, employee, director, Consultant or other key person of the Company or any of its Subsidiaries, an option (the "Stock Option") to purchase on or prior to the Expiration Date, or such earlier date as is specified herein, all or any part of the number of shares of Common Stock, par value \$0.01 per share ("Common Stock"), of the Company indicated above (the "Underlying Shares," and such shares once issued shall be referred to as the "Option Shares"), at the Option Exercise Price per share, subject to the terms and conditions set forth in this Non-Qualified Stock Option Agreement (this "Agreement") and in the Plan. This Stock Option is not intended to qualify as an "incentive stock option" as defined in Section 422(b) of the Internal Revenue Code of 1986, as amended from time to time (the "Code").

All capitalized terms used herein and not otherwise defined shall have the respective meanings set forth in the Plan.

1. Vesting, Exercisability and Termination.

(a) No portion of this Stock Option may be exercised until such portion shall have vested and become exercisable.

(b) Except as set forth below, and subject to the determination of the Committee in its sole discretion to accelerate the vesting schedule hereunder, this Stock Option shall be vested and exercisable with respect to the Underlying Shares on the respective dates indicated below:

(i) All Underlying Shares shall initially be unvested and unexercisable.

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(ii) [25] percent of the Underlying Shares shall vest and become exercisable on the [first] anniversary of the Vesting Commencement Date; provided that the Optionee continues to have a Service Relationship with the Company at such time.

(iii) Thereafter, the remaining [75] percent of the Underlying Shares shall vest and become exercisable in [36] equal monthly installments at the end of each month following the first anniversary of the Vesting Commencement Date, provided the Optionee continues to have a Service Relationship with the Company at such time.

Notwithstanding anything herein to the contrary in the case of a Sale Event, this Stock Option shall be treated as provided in Section 3(c) of the Plan.

(c) Termination. Except as may otherwise be provided by the Committee, if the Optionee's Service Relationship is terminated, the period within which to exercise this Stock Option will be subject to earlier termination as set forth below (and if not exercised within such period, shall thereafter terminate subject, in each case to Section 3(c) of the Plan):

(i) Termination Due to Death or Disability. If the Optionee's Service Relationship terminates by reason of such Optionee's death or disability (as defined in Section 422(c) of the Code), this Stock Option may be exercised, to the extent exercisable on the date of such termination, by the Optionee, the Optionee's legal representative or legatee for a period of 12 months from the date of death or disability or until the Expiration Date, if earlier.

(ii) Other Termination. If the Optionee's Service Relationship terminates for any reason other than death or disability (as defined in Section 422(c) of the Code), and unless otherwise determined by the Committee, this Stock Option may be exercised, to the extent exercisable on the date of termination, for a period of 90 days from the date of termination or until the Expiration Date or other termination date, if earlier; provided however, if the Optionee's Service Relationship is terminated for Cause, this Stock Option shall terminate immediately upon the date of such termination[ ], **such termination shall be treated as a "Repurchase Event" and the Company shall have the right to repurchase the Shares as set forth in Section 9(c) of the Plan**.

For purposes hereof, the Committee's determination of the reason for termination of the Optionee's Service Relationship shall be conclusive and binding on the Optionee and his or her representatives or legatees and any Permitted Transferee. Any portion of this Stock Option that is not exercisable on the date of termination of the Service Relationship shall terminate immediately and be null and void.

## 2. Exercise of Stock Option.

(a) The Optionee may exercise this Stock Option only in the following manner: Prior to the Expiration Date, the Optionee may deliver a Stock Option exercise notice (an "Exercise Notice") in the form of Appendix A hereto indicating his or her election to purchase some or all of the Underlying Shares with respect to which this Stock Option is exercisable at the time of such notice. Such notice shall specify the number of Underlying Shares to be purchased. Payment of the purchase price may be made by one or more of the methods described in Sections 5(a)(iv)(A), (B), (C), (D) or (E) of the Plan, subject to the limitations contained in such Sections of the Plan, including the requirement that the Committee specifically approve in advance certain payment methods.

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(b) Notwithstanding any other provision hereof or of the Plan, no portion of this Stock Option shall be exercisable after the Expiration Date.

3. Incorporation of Plan. Notwithstanding anything herein to the contrary, this Stock Option shall be subject to and governed by all the terms and conditions of the Plan.

4. Transferability of Stock Option. This Agreement is personal to the Optionee and is not transferable by the Optionee in any manner other than by will or by the laws of descent and distribution. The Stock Option may be exercised during the Optionee's lifetime only by the Optionee (or by the Optionee's guardian or personal representative in the event of the Optionee's incapacity). The Optionee may elect to designate a beneficiary by providing written notice of the name of such beneficiary to the Company, and may revoke or change such designation at any time by filing written notice of revocation or change with the Company; such beneficiary may exercise the Optionee's Stock Option in the event of the Optionee's death to the extent provided herein. If the Optionee does not designate a beneficiary, or if the designated beneficiary predeceases the Optionee, the legal representative of the Optionee may exercise this Stock Option to the extent provided herein in the event of the Optionee's death.

5. Restrictions on Transfer of Option Shares. The Option Shares acquired upon exercise of the Stock Option shall be subject to certain transfer restrictions and other limitations including, without limitation, the provisions contained in Section 9 of the Plan.

6. Miscellaneous Provisions.

(a) Equitable Relief. The parties hereto agree and declare that legal remedies may be inadequate to enforce the provisions of this Agreement and that equitable relief, including specific performance and injunctive relief, may be used to enforce the provisions of this Agreement.

(b) Adjustments for Changes in Capital Structure. If, as a result of any reorganization, recapitalization, reincorporation, reclassification, stock dividend, stock split, reverse stock split or other similar change in the Common Stock, the outstanding shares of Common Stock are increased or decreased or are exchanged for a different number or kind of shares of the Company's stock, the restrictions contained in this Agreement shall apply with equal force to additional and/or substitute securities, if any, received by the Optionee in exchange for, or by virtue of his or her ownership of, Option Shares.

(c) Change and Modifications. This Agreement may not be orally changed, modified or terminated, nor shall any oral waiver of any of its terms be effective. This Agreement may be changed, modified or terminated only by an agreement in writing signed by the Company and the Optionee.



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(d) Governing Law. This Agreement shall be governed by and construed in accordance with the General Corporation Law of the State of Delaware as to matters within the scope hereof, and as to all other matters shall be governed by and construed in accordance with the internal laws of the Commonwealth of Massachusetts, without regard to conflict of law principles that would result in the application of any law other than the law of the Commonwealth of Massachusetts.

(e) Headings. The headings are intended only for convenience in finding the subject matter and do not constitute part of the text of this Agreement and shall not be considered in the interpretation of this Agreement.

(f) Saving Clause. If any provision(s) of this Agreement shall be determined to be illegal or unenforceable, such determination shall in no manner affect the legality or enforceability of any other provision hereof.

(g) Notices. All notices, requests, consents and other communications shall be in writing and be deemed given when delivered personally, by telex or facsimile transmission or when received if mailed by first class registered or certified mail, postage prepaid. Notices to the Company or the Optionee shall be addressed as set forth underneath their signatures below, or to such other address or addresses as may have been furnished by such party in writing to the other.

(h) Benefit and Binding Effect. This Agreement shall be binding upon and shall inure to the benefit of the parties hereto, their respective successors, permitted assigns, and legal representatives. The Company has the right to assign this Agreement, and such assignee shall become entitled to all the rights of the Company hereunder to the extent of such assignment.

(i) Counterparts. For the convenience of the parties and to facilitate execution, this Agreement may be executed in two or more counterparts, each of which shall be deemed an original, but all of which shall constitute one and the same document.

#### 7. Dispute Resolution.

(a) Except as provided below, any dispute arising out of or relating to the Plan or this Stock Option, this Agreement, or the breach, termination or validity of the Plan, this Stock Option or this Agreement, shall be finally settled by binding arbitration conducted expeditiously in accordance with the J.A.M.S./Endispute Comprehensive Arbitration Rules and Procedures (the "J.A.M.S. Rules"). The arbitration shall be governed by the United States Arbitration Act, 9 U.S.C. Sections 1 16, and judgment upon the award rendered by the arbitrators may be entered by any court having jurisdiction thereof. The place of arbitration shall be Boston, MA.

(b) The arbitration shall commence within 60 days of the date on which a written demand for arbitration is filed by any party hereto. In connection with the arbitration proceeding, the arbitrator shall have the power to order the production of documents by each party and any third-party witnesses. In addition, each party may take up to three depositions as of right, and the arbitrator may in his or her discretion allow additional depositions upon good cause shown by the moving party. However, the arbitrator shall not have the power to order the answering of interrogatories or the response to requests for admission. In connection with any arbitration, each party to the arbitration shall provide to the other, no later than seven business

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days before the date of the arbitration, the identity of all persons that may testify at the arbitration and a copy of all documents that may be introduced at the arbitration or considered or used by a party's witness or expert. The arbitrator's decision and award shall be made and delivered within six months of the selection of the arbitrator. The arbitrator's decision shall set forth a reasoned basis for any award of damages or finding of liability. The arbitrator shall not have power to award damages in excess of actual compensatory damages and shall not multiply actual damages or award punitive damages, and each party hereby irrevocably waives any claim to such damages.

(c) The Company, the Optionee, each party to the Agreement and any other holder of Stock issued pursuant to this Agreement (each, a "Party") covenants and agrees that such party will participate in the arbitration in good faith. This Section 7 applies equally to requests for temporary, preliminary or permanent injunctive relief, except that in the case of temporary or preliminary injunctive relief any party may proceed in court without prior arbitration for the limited purpose of avoiding immediate and irreparable harm.

(d) Each Party (i) hereby irrevocably submits to the jurisdiction of any United States District Court of competent jurisdiction for the purpose of enforcing the award or decision in any such proceeding, (ii) hereby waives, and agrees not to assert, by way of motion, as a defense, or otherwise, in any such suit, action or proceeding, any claim that it is not subject personally to the jurisdiction of the above named courts, that its property is exempt or immune from attachment or execution (except as protected by applicable law), that the suit, action or proceeding is brought in an inconvenient forum, that the venue of the suit, action or proceeding is improper or that this Agreement or the subject matter hereof may not be enforced in or by such court, and (iii) hereby waives and agrees not to seek any review by any court of any other jurisdiction which may be called upon to grant an enforcement of the judgment of any such court. Each Party hereby consents to service of process by registered mail at the address to which notices are to be given. Each Party agrees that its, his or her submission to jurisdiction and its, his or her consent to service of process by mail is made for the express benefit of each other Party. Final judgment against any Party in any such action, suit or proceeding may be enforced in other jurisdictions by suit, action or proceeding on the judgment, or in any other manner provided by or pursuant to the laws of such other jurisdiction.

[SIGNATURE PAGE FOLLOWS]

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The foregoing Agreement is hereby accepted and the terms and conditions thereof hereby agreed to by the undersigned as of the date first above written.

**BLUEBIRD BIO, INC.**

By: \_\_\_\_\_

Name:

Title:

Address:

840 Memorial Drive

Cambridge, MA 02139

The undersigned hereby acknowledges receiving and reviewing a copy of the Plan, including, without limitation, Section 9 thereof, and understands that the Stock Option granted hereby is subject to the terms of the Plan and of this Agreement. This Agreement is hereby accepted, and the terms and conditions of the Plan and this Agreement, SPECIFICALLY INCLUDING THE ARBITRATION PROVISIONS IN SECTION 7 OF THIS AGREEMENT, are hereby agreed to, by the undersigned as of the date first above written.

OPTIONEE:

\_\_\_\_\_  
Name:

Address:  
  
\_\_\_\_\_  
  
\_\_\_\_\_  
  
\_\_\_\_\_

[SPOUSE'S CONSENT

I acknowledge that I have read the foregoing Non-Qualified Stock Option Agreement and understand the contents thereof.

\_\_\_\_\_ ]

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DESIGNATED BENEFICIARY:

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Beneficiary's Address:

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**Appendix A**

**STOCK OPTION EXERCISE NOTICE**

**BLUEBIRD BIO, INC.**  
Attention: Office Manager  
840 Memorial Drive  
Cambridge, MA 02139

Pursuant to the terms of the stock option agreement between the undersigned and bluebird bio, Inc. (the "Company") dated \_\_\_\_\_ (the "Agreement") under the bluebird bio, Inc. 2010 Stock Option and Grant Plan, I, [Insert Name] \_\_\_\_\_, hereby [Circle One] partially/fully exercise such option by including herein payment in the amount of \$ \_\_\_\_\_ representing the purchase price for [Fill in number of Underlying Shares] \_\_\_\_\_ Underlying Shares. I have chosen the following form(s) of payment:

- 1. Cash
  - 2. Certified or bank check payable to "bluebird bio, Inc."
  - 3. Other (as referenced in the Agreement and described in the Plan (please describe))
- 

In connection with my exercise of the option as set forth above, I hereby represent and warrant to the Company as follows:

- (i) I am purchasing the Underlying Shares for my own account for investment only, and not for resale or with a view to the distribution thereof.
- (ii) I have had such an opportunity as I have deemed adequate to obtain from the Company such information as is necessary to permit me to evaluate the merits and risks of my investment in the Company and have consulted with my own advisers with respect to my investment in the Company.
- (iii) I have sufficient experience in business, financial and investment matters to be able to evaluate the risks involved in the purchase of the Underlying Shares and to make an informed investment decision with respect to such purchase.
- (iv) I can afford a complete loss of the value of the Option Shares and am able to bear the economic risk of holding such Option Shares for an indefinite period of time.
- (v) I understand that the Option Shares may not be registered under the Securities Act of 1933 (it being understood that the Option Shares are being issued and sold in reliance on the exemption provided in Rule 701 thereunder) or any applicable state securities or "blue sky" laws and may not be sold or otherwise transferred or disposed of in the absence of an effective registration statement under the Securities Act

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of 1933 and under any applicable state securities or “blue sky” laws (or exemptions from the registration requirement thereof). I further acknowledge that certificates representing Option Shares will bear restrictive legends reflecting the foregoing and/or that book entries for uncertificated Option Shares will include similar restrictive notations.

Sincerely yours,

\_\_\_\_\_  
Name:

\_\_\_\_\_  
Address:  
\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

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**EARLY EXERCISE**  
**NON-QUALIFIED STOCK OPTION AGREEMENT**  
**UNDER THE BLUEBIRD BIO, INC.**  
**2010 STOCK OPTION AND GRANT PLAN**

Name of Optionee: \_\_\_\_\_ (the "Optionee")  
No. of Option Shares: \_\_\_\_\_ Shares of Common Stock  
Grant Date: \_\_\_\_\_  
Vesting Commencement Date: \_\_\_\_\_ (the "Vesting Commencement Date")  
Expiration Date: \_\_\_\_\_ (the "Expiration Date")  
Option Exercise Price/Share: \$ \_\_\_\_\_ (the "Option Exercise Price")

Pursuant to the bluebird bio, Inc. 2010 Stock Option and Grant Plan (the "Plan"), bluebird bio, Inc., a Delaware corporation (together with any successor thereto, the "Company"), hereby grants to the Optionee, who is an officer, employee, director, Consultant or other key person of the Company or any of its Subsidiaries, an option (the "Stock Option") to purchase on or prior to the Expiration Date, or such earlier date as is specified herein, all or any part of the number of shares of Common Stock, par value \$0.01 per share ("Common Stock"), of the Company indicated above (the "Option Shares"), at the Option Exercise Price per share, subject to the terms and conditions set forth in this Early Exercise Non-Qualified Stock Option Agreement (this "Agreement") and in the Plan. This Stock Option is not intended to qualify as an "incentive stock option" as defined in Section 422(b) of the Internal Revenue Code of 1986, as amended from time to time (the "Code").

All capitalized terms used herein and not otherwise defined shall have the respective meanings set forth in the Plan.

1. Vesting, Exercisability and Termination.

(a) This Stock Option shall be immediately exercisable, regardless of whether the Option Shares are vested.

(b) Except as set forth below, and subject to the determination of the Committee in its sole discretion to accelerate the vesting schedule hereunder, the Option Shares shall be vested on the respective dates indicated below:

(i) All Option Shares shall initially be unvested.

(ii) [25] percent of the Option Shares shall vest on the [first] anniversary of the Vesting Commencement Date; provided that the Optionee continues to have a Service Relationship with the Company at such time.

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(iii) Thereafter, the remaining [75] percent of the Option Shares shall vest in [36] equal monthly installments at the end of each month following the first anniversary of the Vesting Commencement Date, provided the Optionee continues to have a Service Relationship with the Company at such time.

Notwithstanding anything herein to the contrary, in the case of a Sale Event, this Stock Option and the Option Shares shall be treated as provided in Section 3(c) of the Plan.

(c) Termination. Except as may otherwise be provided by the Committee, if the Optionee's Service Relationship is terminated, the period within which to exercise this Stock Option will be subject to earlier termination as set forth below (and if not exercised within such period, shall thereafter terminate subject, in each case to Section 3(c) of the Plan):

(i) Termination Due to Death or Disability. If the Optionee's Service Relationship terminates by reason of such Optionee's death or disability (as defined in Section 422(c) of the Code), this Stock Option may continue to be exercised, to the extent the Option Shares are vested on the date of termination, by the Optionee, the Optionee's legal representative or legatee for a period of 12 months from the date of death or disability or until the Expiration Date, if earlier.

(ii) Other Termination. If the Optionee's Service Relationship terminates for any reason other than death or disability (as defined in Section 422(c) of the Code), and unless otherwise determined by the Committee, this Stock Option may continue to be exercised, to the extent the Option Shares are vested on the date of termination, for a period of 90 days from the date of termination or until the Expiration Date or other termination date, if earlier[; provided however, if the Optionee's Service Relationship is terminated for Cause, this Stock Option shall terminate immediately upon the date of such termination].

For purposes hereof, the Committee's determination of the reason for termination of the Optionee's Service Relationship shall be conclusive and binding on the Optionee and his or her representatives or legatees and any Permitted Transferee. Any portion of this Stock Option with respect to Option Shares that are not vested on the date of termination of the Service Relationship shall terminate immediately and be null and void.

## 2. Exercise of Stock Option.

(a) The Optionee may exercise this Stock Option only in the following manner: Prior to the Expiration Date, the Optionee may deliver a Stock Option exercise notice (an "Exercise Notice") in the form of Appendix A hereto indicating his or her election to purchase some or all of the Option Shares. Such notice shall specify the number of Option Shares to be purchased. To the extent this Stock Option is only partially exercised, such exercise shall first be with respect to the Option Shares, if any, that have previously vested, and then with respect to the Option Shares that will next vest, with the Option Shares that vest at the latest date being exercised last. Payment of the purchase price may be made by one or more of the methods described in Sections 5(a)(iv)(A), (B), (C), (D) or (E) of the Plan, subject to the limitations contained in such Sections of the Plan, including the requirement that the Committee specifically approve in advance certain payment methods.



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(b) In the event the Optionee exercises a portion of this Stock Option with respect to Option Shares that have not vested, the Optionee shall also deliver a Restricted Stock Agreement covering such unvested Option Shares in the form of Appendix B hereto (the "Restricted Stock Agreement") with the same vesting schedule for such Option Shares as set forth for such Option Shares herein.

(c) Notwithstanding any other provision hereof or of the Plan, no portion of this Stock Option shall be exercisable after the Expiration Date.

3. Incorporation of Plan. Notwithstanding anything herein to the contrary, this Stock Option shall be subject to and governed by all the terms and conditions of the Plan.

4. Transferability of Stock Option. This Agreement is personal to the Optionee and is not transferable by the Optionee in any manner other than by will or by the laws of descent and distribution. The Stock Option may be exercised during the Optionee's lifetime only by the Optionee (or by the Optionee's guardian or personal representative in the event of the Optionee's incapacity). The Optionee may elect to designate a beneficiary by providing written notice of the name of such beneficiary to the Company, and may revoke or change such designation at any time by filing written notice of revocation or change with the Company; such beneficiary may exercise the Optionee's Stock Option in the event of the Optionee's death to the extent provided herein. If the Optionee does not designate a beneficiary, or if the designated beneficiary predeceases the Optionee, the legal representative of the Optionee may exercise this Stock Option to the extent provided herein in the event of the Optionee's death.

5. Restrictions on Transfer of Option Shares. The Option Shares acquired upon exercise of the Stock Option shall be subject to certain transfer restrictions and other limitations including, without limitation, the provisions contained in Section 9 of the Plan and, if applicable, the Restricted Stock Agreement.

6. Miscellaneous Provisions.

(a) Equitable Relief. The parties hereto agree and declare that legal remedies may be inadequate to enforce the provisions of this Agreement and that equitable relief, including specific performance and injunctive relief, may be used to enforce the provisions of this Agreement.

(b) Adjustments for Changes in Capital Structure. If, as a result of any reorganization, recapitalization, reincorporation, reclassification, stock dividend, stock split, reverse stock split or other similar change in the Common Stock, the outstanding shares of Common Stock are increased or decreased or are exchanged for a different number or kind of shares of the Company's stock, the restrictions contained in this Agreement shall apply with equal force to additional and/or substitute securities, if any, received by the Optionee in exchange for, or by virtue of his or her ownership of, Option Shares.

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(c) Change and Modifications. This Agreement may not be orally changed, modified or terminated, nor shall any oral waiver of any of its terms be effective. This Agreement may be changed, modified or terminated only by an agreement in writing signed by the Company and the Optionee.

(d) Governing Law. This Agreement shall be governed by and construed in accordance with the General Corporation Law of the State of Delaware as to matters within the scope hereof, and as to all other matters shall be governed by and construed in accordance with the internal laws of the Commonwealth of Massachusetts, without regard to conflict of law principles that would result in the application of any law other than the law of the Commonwealth of Massachusetts.

(e) Headings. The headings are intended only for convenience in finding the subject matter and do not constitute part of the text of this Agreement and shall not be considered in the interpretation of this Agreement.

(f) Saving Clause. If any provision(s) of this Agreement shall be determined to be illegal or unenforceable, such determination shall in no manner affect the legality or enforceability of any other provision hereof.

(g) Notices. All notices, requests, consents and other communications shall be in writing and be deemed given when delivered personally, by telex or facsimile transmission or when received if mailed by first class registered or certified mail, postage prepaid. Notices to the Company or the Optionee shall be addressed as set forth underneath their signatures below, or to such other address or addresses as may have been furnished by such party in writing to the other.

(h) Benefit and Binding Effect. This Agreement shall be binding upon and shall inure to the benefit of the parties hereto, their respective successors, permitted assigns, and legal representatives. The Company has the right to assign this Agreement, and such assignee shall become entitled to all the rights of the Company hereunder to the extent of such assignment.

(i) Counterparts. For the convenience of the parties and to facilitate execution, this Agreement may be executed in two or more counterparts, each of which shall be deemed an original, but all of which shall constitute one and the same document.

#### 7. Dispute Resolution.

(a) Except as provided below, any dispute arising out of or relating to the Plan or this Stock Option, this Agreement, or the breach, termination or validity of the Plan, this Stock Option or this Agreement, shall be finally settled by binding arbitration conducted expeditiously in accordance with the J.A.M.S./Endispute Comprehensive Arbitration Rules and Procedures (the "J.A.M.S. Rules"). The arbitration shall be governed by the United States Arbitration Act, 9 U.S.C. Sections 1 16, and judgment upon the award rendered by the arbitrators may be entered by any court having jurisdiction thereof. The place of arbitration shall be Boston, MA.

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(b) The arbitration shall commence within 60 days of the date on which a written demand for arbitration is filed by any party hereto. In connection with the arbitration proceeding, the arbitrator shall have the power to order the production of documents by each party and any third-party witnesses. In addition, each party may take up to three depositions as of right, and the arbitrator may in his or her discretion allow additional depositions upon good cause shown by the moving party. However, the arbitrator shall not have the power to order the answering of interrogatories or the response to requests for admission. In connection with any arbitration, each party to the arbitration shall provide to the other, no later than seven business days before the date of the arbitration, the identity of all persons that may testify at the arbitration and a copy of all documents that may be introduced at the arbitration or considered or used by a party's witness or expert. The arbitrator's decision and award shall be made and delivered within six months of the selection of the arbitrator. The arbitrator's decision shall set forth a reasoned basis for any award of damages or finding of liability. The arbitrator shall not have power to award damages in excess of actual compensatory damages and shall not multiply actual damages or award punitive damages, and each party hereby irrevocably waives any claim to such damages.

(c) The Company, the Optionee, each party to the Agreement and any other holder of Stock issued pursuant to this Agreement (each, a "Party") covenants and agrees that such party will participate in the arbitration in good faith. This Section 7 applies equally to requests for temporary, preliminary or permanent injunctive relief, except that in the case of temporary or preliminary injunctive relief any party may proceed in court without prior arbitration for the limited purpose of avoiding immediate and irreparable harm.

(d) Each Party (i) hereby irrevocably submits to the jurisdiction of any United States District Court of competent jurisdiction for the purpose of enforcing the award or decision in any such proceeding, (ii) hereby waives, and agrees not to assert, by way of motion, as a defense, or otherwise, in any such suit, action or proceeding, any claim that it is not subject personally to the jurisdiction of the above named courts, that its property is exempt or immune from attachment or execution (except as protected by applicable law), that the suit, action or proceeding is brought in an inconvenient forum, that the venue of the suit, action or proceeding is improper or that this Agreement or the subject matter hereof may not be enforced in or by such court, and (iii) hereby waives and agrees not to seek any review by any court of any other jurisdiction which may be called upon to grant an enforcement of the judgment of any such court. Each Party hereby consents to service of process by registered mail at the address to which notices are to be given. Each Party agrees that its, his or her submission to jurisdiction and its, his or her consent to service of process by mail is made for the express benefit of each other Party. Final judgment against any Party in any such action, suit or proceeding may be enforced in other jurisdictions by suit, action or proceeding on the judgment, or in any other manner provided by or pursuant to the laws of such other jurisdiction.

[SIGNATURE PAGE FOLLOWS]

The foregoing Agreement is hereby accepted and the terms and conditions thereof hereby agreed to by the undersigned as of the date first above written.

**BLUEBIRD BIO, INC.**

By: \_\_\_\_\_

Name:

Title:

Address:

\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

The undersigned hereby acknowledges receiving and reviewing a copy of the Plan, including, without limitation, Section 9 thereof, and understands that the Stock Option granted hereby is subject to the terms of the Plan and of this Agreement. This Agreement is hereby accepted, and the terms and conditions of the Plan and this Agreement, SPECIFICALLY INCLUDING THE ARBITRATION PROVISIONS IN SECTION 7 OF THIS AGREEMENT, are hereby agreed to, by the undersigned as of the date first above written.

**OPTIONEE:**

\_\_\_\_\_  
Name:

Address:

\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

**[SPOUSE'S CONSENT**

I acknowledge that I have read the  
foregoing Non-Qualified Stock Option Agreement  
and understand the contents thereof.

\_\_\_\_\_ ]

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DESIGNATED BENEFICIARY:

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Beneficiary's Address:

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Appendix A

**STOCK OPTION EXERCISE NOTICE**

**BLUEBIRD BIO, INC.**

Attention: [\_\_\_\_\_]

Pursuant to the terms of the stock option agreement between the undersigned and bluebird bio, Inc. (the "Company") dated \_\_\_\_\_ (the "Agreement") under the bluebird bio, Inc. 2010 Stock Option and Grant Plan, I, [Insert Name] \_\_\_\_\_, hereby [Circle One] partially/fully exercise such option by including herein payment in the amount of \$ \_\_\_\_\_ representing the purchase price for [Fill in number of Option Shares] \_\_\_\_\_ Option Shares. I have chosen the following form(s) of payment:

- 1. Cash
- 2. Certified or bank check payable to "bluebird bio, Inc."
- 3. Other (as referenced in the Agreement and described in the Plan (please describe))

\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

In connection with my exercise of the option as set forth above, I hereby represent and warrant to the Company as follows:

- (i) I am purchasing the Option Shares for my own account for investment only, and not for resale or with a view to the distribution thereof.
- (ii) I have had such an opportunity as I have deemed adequate to obtain from the Company such information as is necessary to permit me to evaluate the merits and risks of my investment in the Company and have consulted with my own advisers with respect to my investment in the Company.
- (iii) I have sufficient experience in business, financial and investment matters to be able to evaluate the risks involved in the purchase of the Option Shares and to make an informed investment decision with respect to such purchase.
- (iv) I can afford a complete loss of the value of the Option Shares and am able to bear the economic risk of holding such Option Shares for an indefinite period of time.
- (v) I understand that the Option Shares may not be registered under the Securities Act of 1933 (it being understood that the Option Shares are being issued and sold in reliance on the exemption provided in Rule 701 thereunder) or any applicable state securities or "blue sky" laws and may not be sold or otherwise transferred or disposed of in the absence of an effective registration statement under the Securities Act

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of 1933 and under any applicable state securities or “blue sky” laws (or exemptions from the registration requirement thereof). I further acknowledge that certificates representing Option Shares will bear restrictive legends reflecting the foregoing and/or that book entries for uncertificated Option Shares will include similar restrictive notations.

(vi) To the extent required, I have executed and delivered to the Company the Restricted Stock Agreement attached as Appendix B to the Agreement.

Sincerely yours,

\_\_\_\_\_  
Name:

\_\_\_\_\_  
Address:  
\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

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**Appendix B**

**RESTRICTED STOCK AGREEMENT**



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**RESTRICTED STOCK AGREEMENT  
UNDER THE BLUEBIRD BIO, INC.  
2010 STOCK OPTION AND GRANT PLAN**

Name of Grantee: \_\_\_\_\_ (the "Grantee")  
No. of Shares: \_\_\_\_\_ Shares of Common Stock (the "Shares")  
Grant Date: \_\_\_\_\_  
Vesting Commencement Date: \_\_\_\_\_ (the "Vesting Commencement Date")  
Per Share Purchase Price: \$ \_\_\_\_\_ (the "Per Share Purchase Price")

Pursuant to the bluebird bio, Inc. 2010 Stock Option and Grant Plan (the "Plan"), bluebird bio, Inc., a Delaware corporation (together with any successor entity, the "Company"), hereby grants, sells and issues to the individual named above, who is an officer, employee, director, Consultant or other key person of the Company or any of the Subsidiaries, the Shares at the Per Share Purchase Price, which represents the fair market value per share on the Grant Date, subject to the terms and conditions set forth herein and in the Plan. The Grantee agrees to the provisions set forth herein and acknowledges that each such provision is a material condition of the Company's agreement to issue and sell the Shares to him or her. The Company hereby acknowledges receipt of \$ \_\_\_\_\_ in full payment for the Shares. All references to share prices and amounts herein shall be equitably adjusted to reflect stock splits, stock dividends, recapitalizations, mergers, reorganizations and similar changes affecting the capital stock of the Company, and any shares of capital stock of the Company received on or in respect of Shares in connection with any such event (including any shares of capital stock or any right, option or warrant to receive the same or any security convertible into or exchangeable for any such shares or received upon conversion of any such shares) shall be subject to this Agreement on the same basis and extent at the relevant time as the Shares in respect of which they were issued, and shall be deemed Shares as if and to the same extent they were issued at the date hereof.

All capitalized terms used herein and not otherwise defined shall have the respective meanings set forth in the Plan.

1. Purchase and Sale of Shares; Vesting; Investment Representations.

(a) Purchase and Sale. On the date hereof, the Company hereby sells to the Grantee, and the Grantee hereby purchases from the Company, the number of Shares set forth above for the Per Share Purchase Price.

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(b) Vesting. On the date of this Agreement, all of the Shares are non-transferable and subject to a substantial risk of forfeiture and are Shares of Restricted Stock. Except as set forth below, and subject to the determination of the Committee in its sole discretion to accelerate the vesting schedule hereunder, the risk of forfeiture shall lapse with respect to the Shares on the respective dates indicated below and such Shares shall become vested as follows:

(i) All of the Shares shall be vested as of the Vesting Commencement Date.

(ii) Notwithstanding anything herein to the contrary in the case of a Sale Event, the Shares of Restricted Stock shall be treated as provided in Section 3(c) of the Plan.

(c) Investment Representations. In connection with the purchase and sale of the Shares contemplated by Section 1(a) above, the Grantee hereby represents and warrants to the Company as follows:

(i) The Grantee is purchasing the Shares for the Grantee's own account for investment only, and not for resale or with a view to the distribution thereof.

(ii) The Grantee has had such an opportunity as he or she has deemed adequate to obtain from the Company such information as is necessary to permit him or her to evaluate the merits and risks of the Grantee's investment in the Company and has consulted with the Grantee's own advisers with respect to the Grantee's investment in the Company.

(iii) The Grantee has sufficient experience in business, financial and investment matters to be able to evaluate the risks involved in the purchase of the Shares and to make an informed investment decision with respect to such purchase.

(iv) The Grantee can afford a complete loss of the value of the Shares and is able to bear the economic risk of holding such Shares for an indefinite period.

(v) The Grantee understands that the Shares are not registered under the Act (it being understood that the Shares are being issued and sold in reliance on the exemption provided in Rule 701 thereunder) or any applicable state securities or "blue sky" laws and may not be sold or otherwise transferred or disposed of in the absence of an effective registration statement under the Act and under any applicable state securities or "blue sky" laws (or exemptions from the registration requirements thereof). The Grantee further acknowledges that certificates representing the Shares will bear restrictive legends reflecting the foregoing and/or that book entries for uncertificated Shares will include similar restrictive notations.

2. Repurchase Right. Upon a Termination Event or other Repurchase Event, the Company shall have the right to repurchase the Shares as set forth in Section 9(c) of the Plan; provided, however, that in the case of a Termination Event, the Company shall only have the right to repurchase Shares of Restricted Stock which are unvested as of the date of such Termination Event.

3. Restrictions on Transfer of Shares. The Shares (whether or not vested) shall be subject to certain transfer restrictions and other limitations including, without limitation, the provisions contained in Section 9 of the Plan

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4. Incorporation of Plan. Notwithstanding anything herein to the contrary, this Restricted Stock Award shall be subject to and governed by all the terms and conditions of the Plan.

5. Miscellaneous Provisions.

(a) Record Owner; Dividends. The Grantee and any Permitted Transferees, during the duration of this Agreement, shall be considered the record owners of and shall be entitled to vote the Shares if and to the extent the Shares are entitled to voting rights. The Grantee and any Permitted Transferees shall be entitled to receive all dividends and any other distributions declared on the Shares; provided, however, that the Company is under no duty to declare any such dividends or to make any such distribution.

(b) Section 83(b) Election. The Grantee shall consult with the Grantee's tax advisor to determine whether it would be appropriate for the Grantee to make an election under Section 83(b) of the Code with respect to this Award. Any such election must be filed with the Internal Revenue Service within 30 days of the date of this Award. If the Grantee makes an election under Section 83(b) of the Code, the Grantee shall give prompt notice to the Company (and provide a copy of such election to the Company).

(c) Equitable Relief. The parties hereto agree and declare that legal remedies are inadequate to enforce the provisions of this Agreement and that equitable relief, including specific performance and injunctive relief, may be used to enforce the provisions of this Agreement.

(d) Change and Modifications. This Agreement may not be orally changed, modified or terminated, nor shall any oral waiver of any of its terms be effective. This Agreement may be changed, modified or terminated only by an agreement in writing signed by the Company and the Grantee.

(e) Governing Law. This Agreement shall be governed by and construed in accordance with the General Corporation Law of the State of Delaware as to matters within the scope thereof, and as to all other matters shall be governed by and construed in accordance with the internal laws of the Commonwealth of Massachusetts, without regard to conflict of law principles that would result in the application of any law other than the law of the Commonwealth of Massachusetts.

(f) Headings. The headings are intended only for convenience in finding the subject matter and do not constitute part of the text of this Agreement and shall not be considered in the interpretation of this Agreement.

(g) Saving Clause. If any provision(s) of this Agreement shall be determined to be illegal or unenforceable, such determination shall in no manner affect the legality or enforceability of any other provision hereof.

(h) Notices. All notices, requests, consents and other communications shall be in writing and be deemed given when delivered personally, by telex or facsimile transmission or when received if mailed by first class registered or certified mail, postage prepaid. Notices to

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the Company or the Grantee shall be addressed as set forth underneath their signatures below, or to such other address or addresses as may have been furnished by such party in writing to the other. Notices to any holder of the Shares other than the Grantee shall be addressed to the address furnished by such holder to the Company.

(i) Benefit and Binding Effect. This Agreement shall be binding upon and shall inure to the benefit of the parties hereto, their respective successors, assigns, and legal representatives. The Company has the right to assign this Agreement, and such assignee shall become entitled to all the rights of the Company hereunder to the extent of such assignment.

(j) Counterparts. For the convenience of the parties and to facilitate execution, this Agreement may be executed in two or more counterparts, each of which shall be deemed an original, but all of which shall constitute one and the same document.

#### 6. Dispute Resolution.

(a) Except as provided below, any dispute arising out of or relating to the Plan or the Shares, this Agreement, or the breach, termination or validity of the Plan, the Shares or this Agreement, shall be finally settled by binding arbitration conducted expeditiously in accordance with the J.A.M.S./Endispute Comprehensive Arbitration Rules and Procedures (the "J.A.M.S. Rules"). The arbitration shall be governed by the United States Arbitration Act, 9 U.S.C. Sections 1 16, and judgment upon the award rendered by the arbitrators may be entered by any court having jurisdiction thereof. The place of arbitration shall be Boston, MA.

(b) The arbitration shall commence within 60 days of the date on which a written demand for arbitration is filed by any party hereto. In connection with the arbitration proceeding, the arbitrator shall have the power to order the production of documents by each party and any third-party witnesses. In addition, each party may take up to three depositions as of right, and the arbitrator may in his or her discretion allow additional depositions upon good cause shown by the moving party. However, the arbitrator shall not have the power to order the answering of interrogatories or the response to requests for admission. In connection with any arbitration, each party to the arbitration shall provide to the other, no later than seven business days before the date of the arbitration, the identity of all persons that may testify at the arbitration and a copy of all documents that may be introduced at the arbitration or considered or used by a party's witness or expert. The arbitrator's decision and award shall be made and delivered within six months of the selection of the arbitrator. The arbitrator's decision shall set forth a reasoned basis for any award of damages or finding of liability. The arbitrator shall not have power to award damages in excess of actual compensatory damages and shall not multiply actual damages or award punitive damages, and each party hereby irrevocably waives any claim to such damages.

(c) The Company, the Grantee, each party to the Agreement and any other holder of Shares or Stock issued pursuant to this Agreement (each, a "Party") covenants and agrees that such party will participate in the arbitration in good faith. This Section 6 applies equally to requests for temporary, preliminary or permanent injunctive relief, except that in the case of temporary or preliminary injunctive relief any party may proceed in court without prior arbitration for the limited purpose of avoiding immediate and irreparable harm.

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(d) Each Party (i) hereby irrevocably submits to the jurisdiction of any United States District Court of competent jurisdiction for the purpose of enforcing the award or decision in any such proceeding, (ii) hereby waives, and agrees not to assert, by way of motion, as a defense, or otherwise, in any such suit, action or proceeding, any claim that it is not subject personally to the jurisdiction of the above named courts, that its property is exempt or immune from attachment or execution (except as protected by applicable law), that the suit, action or proceeding is brought in an inconvenient forum, that the venue of the suit, action or proceeding is improper or that this Agreement or the subject matter hereof may not be enforced in or by such court, and (iii) hereby waives and agrees not to seek any review by any court of any other jurisdiction which may be called upon to grant an enforcement of the judgment of any such court. Each Party hereby consents to service of process by registered mail at the address to which notices are to be given. Each Party agrees that its, his or her submission to jurisdiction and its, his or her consent to service of process by mail is made for the express benefit of each other Party. Final judgment against any Party in any such action, suit or proceeding may be enforced in other jurisdictions by suit, action or proceeding on the judgment, or in any other manner provided by or pursuant to the laws of such other jurisdiction.

[SIGNATURE PAGE FOLLOWS]

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IN WITNESS WHEREOF, the Company and the Grantee have executed this Restricted Stock Agreement as of the date first above written.

**BLUEBIRD BIO, INC.**

By: \_\_\_\_\_  
Name:  
Title:

The undersigned hereby acknowledges receiving and reviewing a copy of the Plan, including, without limitation, Section 9 thereof and understands that the Shares granted hereby are subject to the terms of the Plan and of this Agreement. This Agreement is hereby accepted, and the terms and conditions of the Plan and this Agreement, SPECIFICALLY INCLUDING THE ARBITRATION PROVISIONS IN SECTION 6 OF THIS AGREEMENT, are hereby agreed to, by the undersigned as of the date first above written.

GRANTEE:

\_\_\_\_\_  
Name:  
Address:

[SPOUSE'S CONSENT  
I acknowledge that I have read the  
foregoing Restricted Stock Agreement  
and understand the contents thereof.

\_\_\_\_\_ ]

**FORM OF INDEMNIFICATION AGREEMENT**  
**BLUEBIRD BIO, INC.**

Indemnification Agreement

This Indemnification Agreement (“**Agreement**”) is made as of [\_\_\_\_\_] by and between **bluebird bio, Inc.**, a Delaware corporation (the “**Company**”), and (“**Indemnitee**”).

RECITALS

WHEREAS, the Company desires to attract and retain the services of highly qualified individuals, such as Indemnitee, to serve the Company;

WHEREAS, in order to induce Indemnitee to provide or continue to provide services to the Company, the Company wishes to provide for the indemnification of, and advancement of expenses to, Indemnitee to the maximum extent permitted by law;

WHEREAS, the Bylaws (as the same may be amended, restated or otherwise modified from time to time, the “**Bylaws**”) of the Company require indemnification of the officers and directors of the Company, and Indemnitee may also be entitled to indemnification pursuant to the General Corporation Law of the State of Delaware (the “**DGCL**”);

WHEREAS, the Bylaws and the DGCL expressly provide that the indemnification provisions set forth therein are not exclusive, and thereby contemplate that contracts may be entered into between the Company and members of the board of directors, officers and other persons with respect to indemnification;

WHEREAS, the Board of Directors of the Company (the “**Board**”) has determined that the increased difficulty in attracting and retaining highly qualified persons such as Indemnitee is detrimental to the best interests of the Company’s stockholders;

WHEREAS, it is reasonable, prudent and necessary for the Company contractually to obligate itself to indemnify, and to advance expenses on behalf of, such persons to the fullest extent permitted by applicable law, regardless of any amendment or revocation of the Certificate of Incorporation (as the same may be amended, restated or otherwise modified from time to time, the “**Charter**”) or the Bylaws, so that they will serve or continue to serve the Company free from undue concern that they will not be so indemnified;

WHEREAS, this Agreement is a supplement to and in furtherance of the indemnification provided in the Bylaws and any resolutions adopted pursuant thereto, and shall not be deemed a substitute therefor, nor to diminish or abrogate any rights of Indemnitee thereunder; and

[WHEREAS, Indemnitee has certain rights to indemnification and/or insurance provided by [Name of Fund/Sponsor] which Indemnitee and [Name of Fund/Sponsor] intend to be secondary to the primary obligation of the Company to indemnify Indemnitee as provided in this Agreement, with the Company’s acknowledgment and agreement to the foregoing being a material condition to Indemnitee’s willingness to serve or continue to serve on the Board.]

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NOW, THEREFORE, in consideration of the premises and the covenants contained herein, the Company and Indemnitee do hereby covenant and agree as follows:

Section 1. Services to the Company. Indemnitee agrees to serve as a director or officer of the Company. Indemnitee may at any time and for any reason resign from such position (subject to any other contractual obligation or any obligation imposed by law), in which event the Company shall have no obligation under this Agreement to continue Indemnitee in such position. This Agreement shall not be deemed an employment contract between the Company (or any of its subsidiaries or any Enterprise) and Indemnitee.

Section 2. Definitions.

As used in this Agreement:

(a) **“Change in Control”** shall mean:

(i) the date any “person,” as such term is used in Sections 13(d) and 14(d) of the Securities Exchange Act of 1934, as amended (the “Act”) (other than the Company, any of its subsidiaries, or any trustee, fiduciary or other person or entity holding securities under any employee benefit plan or trust of the Company or any of its subsidiaries), together with all “affiliates” and “associates” (as such terms are defined in Rule 12b-2 under the Act) of such person, becomes the “beneficial owner” (as such term is defined in Rule 13d-3 under the Act), directly or indirectly, of securities of the Company representing 50 percent or more of the combined voting power of the Company’s then outstanding securities having the right to vote in an election of the Board (“Voting Securities”) (in such case other than as a result of an acquisition of securities directly from the Company); or

(ii) the date a majority of the members of the Board is replaced during any 12-month period by directors whose appointment or election is not endorsed by a majority of the members of the Board before the date of the appointment or election; or

(iii) the date of consummation of (A) any consolidation or merger of the Company where the stockholders of the Company, immediately prior to the consolidation or merger, would not, immediately after the consolidation or merger, beneficially own (as such term is defined in Rule 13d-3 under the Act), directly or indirectly, shares representing in the aggregate more than 50 percent of the voting shares of the Company issuing cash or securities in the consolidation or merger (or of its ultimate parent corporation, if any), or (B) any sale or other transfer (in one transaction or a series of transactions contemplated or arranged by any party as a single plan) of all or substantially all of the assets of the Company.

Notwithstanding the foregoing, a “Change in Control” will not be deemed to have occurred for purposes of the foregoing clause (i) solely as the result of an acquisition of securities by the Company which, by reducing the number of shares of Voting Securities outstanding, increases the proportionate number of Voting Securities beneficially owned by any person to 50 percent or more of the combined voting power of all of the then outstanding Voting Securities; provided, however, that if any person referred to in this sentence will thereafter



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become the beneficial owner of any additional shares of Voting Securities (other than pursuant to a stock split, stock dividend, or similar transaction or as a result of an acquisition of securities directly from the Company) and immediately thereafter beneficially owns 50 percent or more of the combined voting power of all of the then outstanding Voting Securities, then a "Change in Control" will be deemed to have occurred for purposes of the foregoing clause (i).

(b) "**Corporate Status**" describes the status of a person as a current or former director or officer of the Company or current or former director, manager, officer, employee, agent or trustee of any other Enterprise which such person is or was serving at the request of the Company.

(c) "**Enforcement Expenses**" shall include all reasonable attorneys' fees, court costs, transcript costs, fees of experts, travel expenses, duplicating costs, printing and binding costs, telephone charges, postage, delivery service fees, and all other out-of-pocket disbursements or expenses of the types customarily incurred in connection with an action to enforce indemnification or advancement rights, or an appeal from such action. Expenses, however, shall not include fees, salaries, wages or benefits owed to Indemnitee.

(d) "**Enterprise**" shall mean any corporation (other than the Company), partnership, joint venture, trust, employee benefit plan, limited liability company, or other legal entity of which Indemnitee is or was serving at the request of the Company as a director, manager, officer, employee, agent or trustee.

(e) "**Expenses**" shall include all reasonable attorneys' fees, court costs, transcript costs, fees of experts, travel expenses, duplicating costs, printing and binding costs, telephone charges, postage, delivery service fees, and all other out-of-pocket disbursements or expenses of the types customarily incurred in connection with prosecuting, defending, preparing to prosecute or defend, investigating, being or preparing to be a witness in, or otherwise participating in, a Proceeding or an appeal resulting from a Proceeding. Expenses, however, shall not include amounts paid in settlement by Indemnitee, the amount of judgments or fines against Indemnitee or fees, salaries, wages or benefits owed to Indemnitee.

(f) "**Independent Counsel**" means a law firm, or a partner (or, if applicable, member or shareholder) of such a law firm, that is experienced in matters of Delaware corporation law and neither presently is, nor in the past five (5) years has been, retained to represent: (i) the Company, any subsidiary of the Company, any Enterprise or Indemnitee in any matter material to any such party (other than with respect to matters concerning Indemnitee under this Agreement, or of other indemnitees under similar indemnification agreements); or (ii) any other party to the Proceeding giving rise to a claim for indemnification hereunder. Notwithstanding the foregoing, the term "Independent Counsel" shall not include any person who, under the applicable standards of professional conduct then prevailing, would have a conflict of interest in representing either the Company or Indemnitee in an action to determine Indemnitee's rights under this Agreement. The Company agrees to pay the reasonable fees and expenses of the Independent Counsel referred to above and to fully indemnify such counsel against any and all expenses, claims, liabilities and damages arising out of or relating to this Agreement or its engagement pursuant hereto.

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(g) The term “**Proceeding**” shall include any threatened, pending or completed action, suit, arbitration, alternate dispute resolution mechanism, investigation, inquiry, administrative hearing or any other actual, threatened or completed proceeding, whether brought in the right of the Company or otherwise and whether of a civil, criminal, administrative, regulatory or investigative nature, and whether formal or informal, in which Indemnitee was, is or will be involved as a party or otherwise by reason of the fact that Indemnitee is or was a director or officer of the Company or is or was serving at the request of the Company as a director, manager, officer, employee, agent or trustee of any Enterprise or by reason of any action taken by Indemnitee or of any action taken on his or her part while acting as a director or officer of the Company or while serving at the request of the Company as a director, manager, officer, employee, agent or trustee of any Enterprise, in each case whether or not serving in such capacity at the time any liability or expense is incurred for which indemnification, reimbursement or advancement of expenses can be provided under this Agreement; provided, however, that the term “Proceeding” shall not include any action, suit or arbitration, or part thereof, initiated by Indemnitee to enforce Indemnitee’s rights under this Agreement as provided for in Section 12(a) of this Agreement.

Section 3. Indemnity in Third-Party Proceedings. The Company shall indemnify Indemnitee to the extent set forth in this Section 3 if Indemnitee is, or is threatened to be made, a party to or a participant in any Proceeding, other than a Proceeding by or in the right of the Company to procure a judgment in its favor. Pursuant to this Section 3, Indemnitee shall be indemnified against all Expenses, judgments, fines, penalties, excise taxes, and amounts paid in settlement actually and reasonably incurred by Indemnitee or on his or her behalf in connection with such Proceeding or any claim, issue or matter therein, if Indemnitee acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the Company and, in the case of a criminal proceeding, had no reasonable cause to believe that his or her conduct was unlawful.

Section 4. Indemnity in Proceedings by or in the Right of the Company. The Company shall indemnify Indemnitee to the extent set forth in this Section 4 if Indemnitee is, or is threatened to be made, a party to or a participant in any Proceeding by or in the right of the Company to procure a judgment in its favor. Pursuant to this Section 4, Indemnitee shall be indemnified against all Expenses actually and reasonably incurred by him or her or on his or her behalf in connection with such Proceeding or any claim, issue or matter therein, if Indemnitee acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the Company. No indemnification for Expenses shall be made under this Section 4 in respect of any claim, issue or matter as to which Indemnitee shall have been finally adjudged by a court to be liable to the Company, unless and only to the extent that the Delaware Court of Chancery (the “**Delaware Court**”) shall determine upon application that, despite the adjudication of liability but in view of all the circumstances of the case, Indemnitee is fairly and reasonably entitled to indemnification for such expenses as the Delaware Court shall deem proper.

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Section 5. Indemnification for Expenses of a Party Who is Wholly or Partly Successful. Notwithstanding any other provisions of this Agreement and except as provided in Section 7, to the extent that Indemnitee is a party to or a participant in any Proceeding and is successful in such Proceeding or in defense of any claim, issue or matter therein, the Company shall indemnify Indemnitee against all Expenses actually and reasonably incurred by him or her in connection therewith. If Indemnitee is not wholly successful in such Proceeding but is successful as to one or more but less than all claims, issues or matters in such Proceeding, the Company shall indemnify Indemnitee against all Expenses actually and reasonably incurred by him or her or on his or her behalf in connection with each successfully resolved claim, issue or matter. For purposes of this Section and without limitation, the termination of any claim, issue or matter in such a Proceeding by dismissal, with or without prejudice, shall be deemed to be a successful result as to such claim, issue or matter.

Section 6. Reimbursement for Expenses of a Witness or in Response to a Subpoena. Notwithstanding any other provision of this Agreement, to the extent that Indemnitee, by reason of his or her Corporate Status, (i) is a witness in any Proceeding to which Indemnitee is not a party and is not threatened to be made a party or (ii) receives a subpoena with respect to any Proceeding to which Indemnitee is not a party and is not threatened to be made a party, the Company shall reimburse Indemnitee for all Expenses actually and reasonably incurred by him or her or on his or her behalf in connection therewith.

Section 7. Exclusions. Notwithstanding any provision in this Agreement to the contrary, the Company shall not be obligated under this Agreement:

(a) to indemnify for amounts otherwise indemnifiable hereunder (or for which advancement is provided hereunder) if and to the extent that Indemnitee has otherwise actually received such amounts under any insurance policy, contract, agreement or otherwise; provided that the foregoing shall not affect the rights of Indemnitee or the Fund Indemnitors as set forth in Section 14(c)];

(b) to indemnify for an accounting of profits made from the purchase and sale (or sale and purchase) by Indemnitee of securities of the Company within the meaning of Section 16(b) of the Securities Exchange Act of 1934, as amended, or similar provisions of state statutory law or common law;

(c) to indemnify for any reimbursement of, or payment to, the Company by Indemnitee of any bonus or other incentive-based or equity-based compensation or of any profits realized by Indemnitee from the sale of securities of the Company pursuant to Section 304 of the Sarbanes-Oxley Act of 2002 (“SOX”) or any formal policy of the Company adopted by the Board (or a committee thereof), or any other remuneration paid to Indemnitee if it shall be determined by a final judgment or other final adjudication that such remuneration was in violation of law;

(d) to indemnify with respect to any Proceeding, or part thereof, brought by Indemnitee against the Company, any legal entity which it controls, any director or officer thereof or any third party, unless (i) the Board has consented to the initiation of such Proceeding or part thereof and (ii) the Company provides the indemnification, in its sole discretion, pursuant to the powers vested in the Company under applicable law; provided, however, that this Section 7(d) shall not apply to counterclaims or affirmative defenses asserted by Indemnitee in an action brought against Indemnitee; or

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(e) to provide any indemnification or advancement of expenses that is prohibited by applicable law (as such law exists at the time payment would otherwise be required pursuant to this Agreement).

Section 8. Advancement of Expenses. Subject to Section 9(b), the Company shall advance, to the extent not prohibited by law, the Expenses incurred by Indemnitee in connection with any Proceeding, and such advancement shall be made within thirty (30) days after the receipt by the Company of a statement or statements requesting such advances (which shall include invoices received by Indemnitee in connection with such Expenses but, in the case of invoices in connection with legal services, any references to legal work performed or to expenditures made that would cause Indemnitee to waive any privilege accorded by applicable law shall not be included with the invoice) from time to time, whether prior to or after final disposition of any Proceeding. Advances shall be unsecured and interest free. Advances shall be made without regard to Indemnitee's ability to repay the expenses and without regard to Indemnitee's ultimate entitlement to indemnification under the other provisions of this Agreement. Indemnitee shall qualify for advances upon the execution and delivery to the Company of an undertaking to repay the advance if and to the extent it is ultimately determined that Indemnitee is not entitled to indemnification, in the form attached hereto as Exhibit A. The right to advances under this paragraph shall in all events continue until final disposition of any Proceeding, including any appeal therein. Nothing in this Section 8 shall limit Indemnitee's right to advancement pursuant to Section 12(e) of this Agreement.

Section 9. Procedure for Notification and Defense of Claim.

(a) To obtain indemnification under this Agreement, Indemnitee shall submit to the Company a written request therefor specifying the basis for the claim, the amounts for which Indemnitee is seeking payment under this Agreement, and all documentation related thereto as reasonably requested by the Company.

(b) In the event that the Company shall be obligated hereunder to provide indemnification for or make any advancement of Expenses with respect to any Proceeding, the Company shall be entitled to assume the defense of such Proceeding, or any claim, issue or matter therein, with counsel approved by Indemnitee (which approval shall not be unreasonably withheld or delayed) upon the delivery to Indemnitee of written notice of the Company's election to do so. After delivery of such notice, approval of such counsel by Indemnitee and the retention of such counsel by the Company, the Company will not be liable to Indemnitee under this Agreement for any fees or expenses of separate counsel subsequently employed by or on behalf of Indemnitee with respect to the same Proceeding; provided that (i) Indemnitee shall have the right to employ separate counsel in any such Proceeding at Indemnitee's expense and (ii) if (A) the employment of separate counsel by Indemnitee has been previously authorized by the Company, (B) Indemnitee shall have reasonably concluded that there may be a conflict of interest between the Company and Indemnitee in the conduct of such defense, or (C) the Company shall not continue to retain such counsel to defend such Proceeding, then the reasonable fees and expenses actually and reasonably incurred by Indemnitee with respect to his or her separate counsel shall be Expenses hereunder.

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(c) In the event that the Company does not assume the defense in a Proceeding pursuant to paragraph (b) above, then the Company will be entitled to participate in the Proceeding at its own expense.

(d) The Company shall not be liable to indemnify Indemnitee under this Agreement for any amounts paid in settlement of any Proceeding effected without its prior written consent (which consent shall not be unreasonably withheld or delayed). The Company shall not, without the prior written consent of Indemnitee (which consent shall not be unreasonably withheld or delayed), enter into any settlement which (i) includes an admission of fault of Indemnitee, any non-monetary remedy imposed on Indemnitee or any monetary damages for which Indemnitee is not wholly and actually indemnified hereunder or (ii) with respect to any Proceeding with respect to which Indemnitee may be or is made a party or may be otherwise entitled to seek indemnification hereunder, does not include the full release of Indemnitee from all liability in respect of such Proceeding.

Section 10. Procedure Upon Application for Indemnification.

(a) To the extent that Indemnitee shall have been successful on the merits in any Proceeding to which it is a party or a participant or in defense of any claim, issue or matter therein, no determination shall be required to be made with respect to Indemnitee's entitlement to indemnification hereunder. In all other cases, a determination with respect to Indemnitee's entitlement to indemnification hereunder shall be made in the specific case by one of the following methods: (x) if a Change in Control shall have occurred, (i) by Independent Counsel in a written opinion to the Board or (ii) if the Indemnitee so requests in writing, by a majority vote of the disinterested directors, even though less than a quorum; or (y) if a Change in Control shall not have occurred: (i) by a majority vote of the disinterested directors, even though less than a quorum; (ii) by a committee of disinterested directors designated by a majority vote of the disinterested directors, even though less than a quorum; (iii) if there are no disinterested directors or if the disinterested directors so direct, by Independent Counsel in a written opinion to the Board; or (iv) if so directed by the Board, by the stockholders of the Company. For purposes hereof, disinterested directors are those members of the Board who are not parties to the action, suit or proceeding in respect of which indemnification is sought. In the case that such determination is made by Independent Counsel, a copy of Independent Counsel's written opinion shall be delivered to Indemnitee and, if it is so determined that Indemnitee is entitled to indemnification, payment to Indemnitee shall be made within thirty (30) days after such determination. Indemnitee shall cooperate with the Independent Counsel or the Company, as applicable, in making such determination with respect to Indemnitee's entitlement to indemnification, including providing to such counsel or the Company, upon reasonable advance request, any documentation or information which is not privileged or otherwise protected from disclosure and which is reasonably available to Indemnitee and reasonably necessary to such determination. Any out-of-pocket costs or expenses (including reasonable attorneys' fees and disbursements) actually and reasonably incurred by Indemnitee in so cooperating with the Independent Counsel or the Company shall be borne by the Company (irrespective of the determination as to Indemnitee's entitlement to indemnification) and the Company hereby indemnifies and agrees to hold Indemnitee harmless therefrom.

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(b) If the determination of entitlement to indemnification is to be made by Independent Counsel pursuant to Section 10(a), the Independent Counsel shall be selected by the Board if a Change in Control shall not have occurred or, if a Change in Control shall have occurred, by Indemnitee. Indemnitee or the Company, as the case may be, may, within ten (10) days after written notice of such selection, deliver to the Company or Indemnitee, as the case may be, a written objection to such selection; provided, however, that such objection may be asserted only on the ground that the Independent Counsel so selected does not meet the requirements of "Independent Counsel" as defined in Section 2 of this Agreement, and the objection shall set forth with particularity the factual basis of such assertion. Absent a proper and timely objection, the person so selected shall act as Independent Counsel. If such written objection is so made and substantiated, the Independent Counsel so selected may not serve as Independent Counsel unless and until such objection is withdrawn or the Delaware Court has determined that such objection is without merit. If, within twenty (20) days after the later of (i) submission by Indemnitee of a written request for indemnification pursuant to Section 9(a), and (ii) the final disposition of the Proceeding, including any appeal therein, no Independent Counsel shall have been selected without objection, either Indemnitee or the Company may petition the Delaware Court for resolution of any objection which shall have been made by Indemnitee or the Company to the selection of Independent Counsel and/or for the appointment as Independent Counsel of a person selected by the court or by such other person as the court shall designate. The person with respect to whom all objections are so resolved or the person so appointed shall act as Independent Counsel under Section 10(a) hereof. Upon the due commencement of any judicial proceeding or arbitration pursuant to Section 12(a) of this Agreement, Independent Counsel shall be discharged and relieved of any further responsibility in such capacity (subject to the applicable standards of professional conduct then prevailing).

Section 11. Presumptions and Effect of Certain Proceedings.

(a) To the extent permitted by applicable law, in making a determination with respect to entitlement to indemnification hereunder, it shall be presumed that Indemnitee is entitled to indemnification under this Agreement if Indemnitee has submitted a request for indemnification in accordance with Section 9(a) of this Agreement, and the Company shall have the burden of proof to overcome that presumption in connection with the making of any determination contrary to that presumption. Neither (i) the failure of the Company or of Independent Counsel to have made a determination prior to the commencement of any action pursuant to this Agreement that indemnification is proper in the circumstances because Indemnitee has met the applicable standard of conduct, nor (ii) an actual determination by the Company or by Independent Counsel that Indemnitee has not met such applicable standard of conduct, shall be a defense to the action or create a presumption that Indemnitee has not met the applicable standard of conduct.

(b) The termination of any Proceeding or of any claim, issue or matter therein, by judgment, order, settlement or conviction, or upon a plea of guilty, nolo contendere or its equivalent, shall not (except as otherwise expressly provided in this Agreement) of itself adversely affect the right of Indemnitee to indemnification or create a presumption that

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Indemnitee did not act in good faith and in a manner which he or she reasonably believed to be in or not opposed to the best interests of the Company or, with respect to any criminal Proceeding, that Indemnitee had reasonable cause to believe that his or her conduct was unlawful.

(c) The knowledge and/or actions, or failure to act, of any director, manager, officer, employee, agent or trustee of the Company, any subsidiary of the Company, or any Enterprise shall not be imputed to Indemnitee for purposes of determining the right to indemnification under this Agreement.

Section 12. Remedies of Indemnitee.

(a) Subject to Section 12(f), in the event that (i) a determination is made pursuant to Section 10 of this Agreement that Indemnitee is not entitled to indemnification under this Agreement, (ii) advancement of Expenses is not timely made pursuant to Section 8 of this Agreement, (iii) no determination of entitlement to indemnification shall have been made pursuant to Section 10(a) of this Agreement within sixty (60) days after receipt by the Company of the request for indemnification for which a determination is to be made other than by Independent Counsel, (iv) payment of indemnification or reimbursement of expenses is not made pursuant to Section 5 or 6 or the last sentence of Section 10(a) of this Agreement within thirty (30) days after receipt by the Company of a written request therefor (which shall include any invoices received by Indemnitee but, in the case of invoices in connection with legal services, any references to legal work performed or to expenditures made that would cause Indemnitee to waive any privilege accorded by applicable law shall not be included with the invoice) or (v) payment of indemnification pursuant to Section 3 or 4 of this Agreement is not made within thirty (30) days after a determination has been made that Indemnitee is entitled to indemnification, Indemnitee shall be entitled to an adjudication by the Delaware Court of his or her entitlement to such indemnification or advancement. Alternatively, Indemnitee, at his or her option, may seek an award in arbitration to be conducted by a single arbitrator pursuant to the Commercial Arbitration Rules of the American Arbitration Association. Indemnitee shall commence such proceeding seeking an adjudication or an award in arbitration within 180 days following the date on which Indemnitee first has the right to commence such proceeding pursuant to this Section 12(a); provided, however, that the foregoing time limitation shall not apply in respect of a proceeding brought by Indemnitee to enforce his or her rights under Section 5 of this Agreement. The Company shall not oppose Indemnitee's right to seek any such adjudication or award in arbitration.

(b) In the event that a determination shall have been made pursuant to Section 10(a) of this Agreement that Indemnitee is not entitled to indemnification, any judicial proceeding or arbitration commenced pursuant to this Section 12 shall be conducted in all respects as a de novo trial, or arbitration, on the merits and Indemnitee shall not be prejudiced by reason of that adverse determination. In any judicial proceeding or arbitration commenced pursuant to this Section 12, the Company shall have the burden of proving Indemnitee is not entitled to indemnification or advancement, as the case may be.

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(c) If Indemnitee is entitled to indemnification pursuant to Section 10(a) of this Agreement, the Company shall be bound by such provision and/or determination in any judicial proceeding or arbitration commenced pursuant to this Section 12, absent (i) a misstatement by Indemnitee of a material fact, or an omission of a material fact necessary to make Indemnitee's statement not materially misleading, in connection with the request for indemnification, or (ii) a prohibition of such indemnification under applicable law.

(d) The Company shall be precluded from asserting in any judicial proceeding or arbitration commenced pursuant to this Section 12 that the procedures and presumptions of this Agreement are not valid, binding and enforceable and shall stipulate in any such court or before any such arbitrator that the Company is bound by all the provisions of this Agreement.

(e) The Company shall indemnify Indemnitee to the fullest extent permitted by law against any and all Enforcement Expenses and, if requested by Indemnitee, shall (within thirty (30) days after receipt by the Company of a written request therefor) advance, to the extent not prohibited by law, such Enforcement Expenses to Indemnitee, which are incurred by Indemnitee in connection with any action brought by Indemnitee for indemnification or advancement from the Company under this Agreement or under any directors' and officers' liability insurance policies maintained by the Company in the suit for which indemnification or advancement is being sought. Such written request for advancement shall include invoices received by Indemnitee in connection with such Enforcement Expenses but, in the case of invoices in connection with legal services, any references to legal work performed or to expenditures made that would cause Indemnitee to waive any privilege accorded by applicable law shall not be included with the invoice.

(f) Notwithstanding anything in this Agreement to the contrary, no determination as to entitlement to indemnification under this Agreement shall be required to be made prior to the final disposition of the Proceeding, including any appeal therein.



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Section 13. Non-exclusivity; Survival of Rights; Insurance; [Primacy of Indemnification;] Subrogation.

(a) The rights of indemnification and to receive advancement as provided by this Agreement shall not be deemed exclusive of any other rights to which Indemnitee may at any time be entitled under applicable law, the Charter, the Bylaws, any agreement, a vote of stockholders or a resolution of directors, or otherwise. No amendment, alteration or repeal of this Agreement or of any provision hereof shall limit or restrict any right of Indemnitee under this Agreement in respect of any action taken or omitted by such Indemnitee in his or her Corporate Status prior to such amendment, alteration or repeal. To the extent that a change in Delaware law, whether by statute or judicial decision, permits greater indemnification or advancement than would be afforded currently under the Charter, Bylaws and this Agreement, it is the intent of the parties hereto that Indemnitee shall enjoy by this Agreement the greater benefits so afforded by such change. No right or remedy herein conferred is intended to be exclusive of any other right or remedy, and every other right and remedy shall be cumulative and in addition to every other right and remedy given hereunder or now or hereafter existing at law or in equity or otherwise. The assertion or employment of any right or remedy hereunder, or otherwise, shall not prevent the concurrent assertion or employment of any other right or remedy.

(b) To the extent that the Company maintains an insurance policy or policies providing liability insurance for directors, managers, officers, employees, agents or trustees of the Company or of any other Enterprise, Indemnitee shall be covered by such policy or policies in accordance with its or their terms to the maximum extent of the coverage available for any such director, manager, officer, employee, agent or trustee under such policy or policies. If, at the time of the receipt of a notice of a claim pursuant to the terms hereof, the Company has director and officer liability insurance in effect, the Company shall give prompt notice of the commencement of such proceeding to the insurers in accordance with the procedures set forth in the respective policies. The Company shall thereafter take all necessary or desirable action to cause such insurers to pay, on behalf of Indemnitee, all amounts payable as a result of such proceeding in accordance with the terms of such policies.

(c) [The Company hereby acknowledges that Indemnitee has certain rights to indemnification, advancement of expenses and/or insurance provided by [Name of Fund/Sponsor] and certain of its affiliates (collectively, the “**Fund Indemnitors**”). The Company hereby agrees (i) that it is the indemnitor of first resort (*i.e.*, its obligations to Indemnitee are primary and any obligation of the Fund Indemnitors to advance expenses or to provide indemnification for the same expenses or liabilities incurred by Indemnitee are secondary), (ii) that it shall be required to advance the full amount of expenses incurred by Indemnitee and shall be liable for the full amount of all Expenses, judgments, penalties, fines and amounts paid in settlement to the extent legally permitted and as required by the terms of this Agreement and the Charter and/or Bylaws (or any other agreement between the Company and Indemnitee), without regard to any rights Indemnitee may have against the Fund Indemnitors, and (iii) that it irrevocably waives, relinquishes and releases the Fund Indemnitors from any and all claims against the Fund Indemnitors for contribution, subrogation or any other recovery of any kind in respect thereof. The Company further agrees that no advancement or payment by the Fund Indemnitors on behalf of Indemnitee with respect to any claim for which Indemnitee has sought indemnification from the Company shall affect the foregoing and the Fund Indemnitors

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shall have a right of contribution and/or be subrogated to the extent of such advancement or payment to all of the rights of recovery of Indemnitee against the Company. The Company and Indemnitee agree that the Fund Indemnitors are express third party beneficiaries of the terms of this Section 8(c).]

(d) [Except as provided in paragraph (c) above,] [I/i]n the event of any payment under this Agreement, the Company shall be subrogated to the extent of such payment to all of the rights of recovery of Indemnitee [(other than against the Fund Indemnitors)], who shall execute all papers required and take all action necessary to secure such rights, including execution of such documents as are necessary to enable the Company to bring suit to enforce such rights.

(e) [Except as provided in paragraph (c) above,] [T/t]he Company's obligation to provide indemnification or advancement hereunder to Indemnitee who is or was serving at the request of the Company as a director, manager, officer, employee, agent or trustee of any other Enterprise shall be reduced by any amount Indemnitee has actually received as indemnification or advancement from such other Enterprise.

Section 14. Duration of Agreement. This Agreement shall continue until and terminate upon the later of: (a) ten (10) years after the date that Indemnitee shall have ceased to serve as a director or officer of the Company or (b) one (1) year after the final termination of any Proceeding, including any appeal, then pending in respect of which Indemnitee is granted rights of indemnification or advancement hereunder and of any proceeding commenced by Indemnitee pursuant to Section 12 of this Agreement relating thereto. This Agreement shall be binding upon the Company and its successors and assigns and shall inure to the benefit of Indemnitee and his or her heirs, executors and administrators. The Company shall require and cause any successor (whether direct or indirect by purchase, merger, consolidation or otherwise) to all, substantially all or a substantial part, of the business and/or assets of the Company, by written agreement in form and substance satisfactory to Indemnitee, expressly to assume and agree to perform this Agreement in the same manner and to the same extent that the Company would be required to perform if no such succession had taken place.

Section 15. Severability. If any provision or provisions of this Agreement shall be held to be invalid, illegal or unenforceable for any reason whatsoever: (a) the validity, legality and enforceability of the remaining provisions of this Agreement (including, without limitation, each portion of any section of this Agreement containing any such provision held to be invalid, illegal or unenforceable, that is not itself invalid, illegal or unenforceable) shall not in any way be affected or impaired thereby and shall remain enforceable to the fullest extent permitted by law; (b) such provision or provisions shall be deemed reformed to the extent necessary to conform to applicable law and to give the maximum effect to the intent of the parties hereto; and (c) to the fullest extent possible, the provisions of this Agreement (including, without limitation, each portion of any section of this Agreement containing any such provision held to be invalid, illegal or unenforceable, that is not itself invalid, illegal or unenforceable) shall be construed so as to give effect to the intent manifested thereby.

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Section 16. Enforcement.

(a) The Company expressly confirms and agrees that it has entered into this Agreement and assumed the obligations imposed on it hereby in order to induce Indemnitee to serve or continue to serve as a director or officer of the Company, and the Company acknowledges that Indemnitee is relying upon this Agreement in serving as a director or officer of the Company.

(b) This Agreement constitutes the entire agreement between the parties hereto with respect to the subject matter hereof and supersedes all prior agreements and understandings, oral, written and implied, between the parties hereto with respect to the subject matter hereof; provided, however, that this Agreement is a supplement to and in furtherance of the Charter, the Bylaws and applicable law, and shall not be deemed a substitute therefor, nor to diminish or abrogate any rights of Indemnitee thereunder.

Section 17. Modification and Waiver. No supplement, modification or amendment, or waiver of any provision, of this Agreement shall be binding unless executed in writing by the parties thereto. No waiver of any of the provisions of this Agreement shall be deemed or shall constitute a waiver of any other provisions of this Agreement nor shall any waiver constitute a continuing waiver. No supplement, modification or amendment of this Agreement or of any provision hereof shall limit or restrict any right of Indemnitee under this Agreement in respect of any action taken or omitted by such Indemnitee prior to such supplement, modification or amendment.

Section 18. Notice by Indemnitee. Indemnitee agrees promptly to notify the Company in writing upon being served with any summons, citation, subpoena, complaint, indictment, information or other document relating to any Proceeding or matter which may be subject to indemnification, reimbursement or advancement as provided hereunder. The failure of Indemnitee to so notify the Company shall not relieve the Company of any obligation which it may have to Indemnitee under this Agreement or otherwise.

Section 19. Notices. All notices, requests, demands and other communications under this Agreement shall be in writing and shall be deemed to have been duly given if (a) delivered by hand and receipted for by the party to whom said notice or other communication shall have been directed, (b) mailed by certified or registered mail with postage prepaid, on the third business day after the date on which it is so mailed, (c) mailed by reputable overnight courier and receipted for by the party to whom said notice or other communication shall have been directed or (d) sent by facsimile transmission, with receipt of oral confirmation that such transmission has been received:

- (a) If to Indemnitee, at such address as Indemnitee shall provide to the Company.

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(b) If to the Company to:  
bluebird bio, Inc.  
840 Memorial Drive, 4<sup>th</sup> Floor  
Cambridge, MA 02139  
Attention: Counsel

or to any other address as may have been furnished to Indemnitee by the Company.

Section 20. Contribution. To the fullest extent permissible under applicable law, if the indemnification provided for in this Agreement is unavailable to Indemnitee for any reason whatsoever, the Company, in lieu of indemnifying Indemnitee, shall contribute to the amount incurred by Indemnitee, whether for judgments, fines, penalties, excise taxes, amounts paid or to be paid in settlement and/or for Expenses, in connection with any Proceeding in such proportion as is deemed fair and reasonable in light of all of the circumstances in order to reflect (i) the relative benefits received by the Company and Indemnitee in connection with the event(s) and/or transaction(s) giving rise to such Proceeding; and/or (ii) the relative fault of the Company (and its directors, officers, employees and agents) and Indemnitee in connection with such event(s) and/or transactions.

Section 21. Internal Revenue Code Section 409A. The Company intends for this Agreement to comply with the Indemnification exception under Section 1.409A-1(b)(10) of the regulations promulgated under the Internal Revenue Code of 1986, as amended (the “**Code**”), which provides that indemnification of, or the purchase of an insurance policy providing for payments of, all or part of the expenses incurred or damages paid or payable by the Indemnitee with respect to a bona fide claim against the Indemnitee or the Company do not provide for a deferral of compensation, subject to Section 409A of the Code, where such claim is based on actions or failures to act by the Indemnitee in his capacity as a service provider of the Company. The parties intend that this Agreement be interpreted and construed with such intent.

Section 22. Applicable Law and Consent to Jurisdiction. This Agreement and the legal relations among the parties shall be governed by, and construed and enforced in accordance with, the laws of the State of Delaware, without regard to its conflict of laws rules. Except with respect to any arbitration commenced by Indemnitee pursuant to Section 12(a) of this Agreement, the Company and Indemnitee hereby irrevocably and unconditionally (i) agree that any action or proceeding arising out of or in connection with this Agreement shall be brought only in the Delaware Court, and not in any other state or federal court in the United States of America or any court in any other country, (ii) consent to submit to the exclusive jurisdiction of the Delaware Court for purposes of any action or proceeding arising out of or in connection with this Agreement, (iii) consent to service of process at the address set forth in Section 19 of this Agreement with the same legal force and validity as if served upon such party personally within the State of Delaware, (iv) waive any objection to the laying of venue of any such action or proceeding in the Delaware Court, and (v) waive, and agree not to plead or to make, any claim that any such action or proceeding brought in the Delaware Court has been brought in an improper or inconvenient forum.

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Section 23. Headings. The headings of the paragraphs of this Agreement are inserted for convenience only and shall not be deemed to constitute part of this Agreement or to affect the construction thereof.

Section 24. Identical Counterparts. This Agreement may be executed in one or more counterparts, each of which shall for all purposes be deemed to be an original but all of which together shall constitute one and the same Agreement. Only one such counterpart signed by the party against whom enforceability is sought needs to be produced to evidence the existence of this Agreement.

[Remainder of Page Intentionally Left Blank]

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IN WITNESS WHEREOF, the parties have caused this Agreement to be signed as of the day and year first above written.

bluebird bio, Inc.

By: \_\_\_\_\_

Name:

Title:

\_\_\_\_\_  
[Name of Indemnatee]

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**Exhibit A**  
**Form of Undertaking**

[Date]

bluebird bio, Inc.  
840 Memorial Drive, 4<sup>th</sup> Floor  
Cambridge, MA 02139

**Re: Request for Advancement of Expenses**

Ladies and Gentlemen:

Reference is made to the Indemnification Agreement (the “**Agreement**”) by and between bluebird bio, Inc. (the “**Company**”) and the undersigned, \_\_\_\_\_ (“**Indemnitee**”). Capitalized terms not defined herein shall have those meanings as set forth in the Agreement. Pursuant to Section 8 of the Agreement, Indemnitee hereby requests advancement of Expenses incurred as a result of Indemnitee being, or being threatened to be made, a party in the following Proceeding(s): \_\_\_\_\_.

In accordance with Section 8 of the Agreement, Indemnitee undertakes to repay the advancement of Expenses if it shall ultimately be determined that Indemnitee is not entitled to be indemnified by the Company as authorized by Section 145 of the General Corporation Law of the State of Delaware.

Very truly yours,

\_\_\_\_\_, Indemnitee

**RIVERSIDE TECHNOLOGY CENTER  
AMENDED AND RESTATED LEASE AGREEMENT**

Agreement entered into this 18<sup>th</sup> day of May, 2007 by and between **Rivertech Associates II, LLC**, a Massachusetts limited liability company with a principal address at The Abbey Group, 575 Boylston Street Boston, Massachusetts (the "LESSOR"), and **Genetix Pharmaceuticals, Inc.** a corporation with a principal address at 840 Memorial Drive Cambridge, Massachusetts (the "LESSEE"); relative to certain space in the building owned by the LESSOR at 840 Memorial Drive Cambridge, Massachusetts (the "Building"), as follows:

**WHEREAS**, Rivertech Associates, LLC and Genetix Pharmaceuticals, Inc. entered into a certain lease agreement dated February 18, 2000 (the "Original Lease"); and,

**WHEREAS**, LESSOR and LESSEE each hereby represent to the other that as of the date hereof the foregoing Original Lease represents the full and complete agreement relative to LESSEE'S use and occupancy of certain space described thereunder, consisting (prior to this "Amended and Restated Lease Agreement") of approximately 10,593 rentable square feet of space on the fifth (5<sup>th</sup>) floor of the Building, which space is specifically referred to herein as the "Current Leased Premises"; and,

**WHEREAS**, certain space consisting of approximately 10,500 rentable square feet of space on the third (3<sup>rd</sup>) floor of the Building (the "Substituted Leased Premises"), is available as of June 1, 2007 and LESSOR has reached agreement with LESSEE for LESSEE to surrender the Current Leased Premises as of the date (the "Substitution Date") the Substituted Leased Premises are delivered to Tenant in accordance with Section 5 hereof (anticipated to be June 1, 2007), and to lease the Substituted Leased Premises as of the Substitution Date under the terms and conditions of the Lease as revised and amended by this Amended and Restated Lease Agreement; and,

**NOW THEREFORE**, for One (\$1.00) Dollar and other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, the parties hereto agree as follows:

**1. Original Term Expiration and Commencement of the Extended Lease Term**

The current Term under the Original Lease, prior to this Amended and Restated Lease Agreement, expires on June 30, 2007. Effective as of the Substitution Date, LESSEE shall vacate and surrender the Current Leased Premises to LESSOR and LESSOR shall accept such surrender from LESSEE and the Original Lease shall be deemed terminated provided that Subtenant shall have access to the Current Leased Premises for a period of up to thirty (30) days after the Substitution Date in order to satisfy the provisions of the Original Lease pertaining to the condition in which the Current Leased Premises are to be delivered to LESSOR (e.g. Lease Sections 26 and 28), and to effectuate the transfer of LESSEE'S equipment and furniture from the Current Leased Premises to the Substituted Leased Premises in a timely and orderly manner.



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As of the Substitution Date, the Term of the Original Lease (as amended by this Amended and Restated Lease Agreement) will be extended for a period beginning on the Substitution Date and expiring on May 31, 2010 (the "Extended Term"); and LESSEE shall lease the Substituted Leased Premises under the Rent, terms and conditions as set forth in this Amended and Restated Lease Agreement up to the expiration of said Extended Term.

To the extent LESSOR cannot deliver the Substituted Leased Premises to LESSEE on or prior to June 30, 2007, the term of the Original Lease shall be automatically extended through that date which subsequently becomes the Substitution Date, except that the Rent thereunder shall be amended to be equal to the Rent for the Substituted Leased Premises during the first Lease Year of the Extended Term.

The term "Lease Year" as used herein and for all purposes during the Extended Term shall mean the period beginning on the Substitution Date and ending on the day immediately prior to each anniversary of the Substitution Date, provided that the Third Lease Year shall end on May 31, 2010.

## **2. The Substituted Leased Premises**

LESSOR and LESSEE hereby agree that during the Extended Term, the Substituted Leased Premises, as defined herein and as depicted on Exhibit A attached hereto, shall be deemed to mean the "Leased Premises" as referred to in the Lease, unless the context specifically means the "Current Leased Premises."

The Substituted Leased Premises may be used by LESSEE for general office, research and laboratory uses only, as set forth in Section 6 of the Lease, throughout the Extended Term.

Provided the same does not materially interfere with LESSOR'S obligations under Section 5 hereof (in which case LESSEE'S rights under this paragraph shall be expressly subordinate in LESSOR'S sole discretion). LESSEE shall have reasonable supervised access to the Substituted Leased Premises commencing on the date of this Agreement to install trade fixtures and/or cabling and/or communications equipment, and to reconfigure and move laboratory and office equipment. Such early access by LESSEE shall be subject to such times and in such manner as LESSOR may reasonably determine to avoid material interference with LESSOR'S Work.

LESSEE shall not be charged any fees by LESSOR for use of the freight elevators of the building, supervision or otherwise in connection with LESSEE'S move from the Current Leased Premises to the Substituted Leased Premises.

LESSOR shall provide the services of LESSOR'S internal space planning and design staff, at LESSOR'S cost and expense, on a reasonable basis to assist LESSEE in its own space planning and design.

## **3. Annual Base Rent—Revised Annual Base Rent Schedule**

Commencing as of the beginning of the Extended Term, LESSEE'S monthly installments of Annual Base Rent for the Leased Premises shall be based on Annual Base Rent as set forth in the "Revised Annual Base Rent Schedule" appearing below.

**Revised Annual Base Rent Schedule**

First Lease Year	\$ 388,500.00 (\$ 32,375.00/mo.)
Second Lease Year	\$ 399,000.00 (\$ 33,250.00/mo.)
Third Lease Year	\$ 420,000.00 (\$ 35,000.00/mo.)

Annual Base Rent shall be payable by Lessee for each Lease Year of the Extended Term as set forth above in twelve installments during each Lease Year (reflecting the aforesaid Revised Annual Base Rent Schedule), in advance, on or before the first day of each calendar month.

Except as otherwise set forth in Section I on account of the Substitution Date occurring after June 30, 2007, LESSEE shall continue to pay Annual Base Rent under the Original Lease for the Current Leased Premises up to the Substitution Date.

**4. Additional Rent**

Notwithstanding the provisions of the Original Lease, this Amended and Restated Lease Agreement is structured as a “triple net” lease. Accordingly, the provisions for Additional Rent based on Operating Expenses and Taxes are rewritten as follows.

LESSEE shall also pay to LESSOR, as Additional Rent under the Lease, the following amounts based on LESSEE’S allocable percentage (which is 8.3441%, the “Allocable Percentage”):

A. Operating Expenses: LESSEE shall be responsible for payment of Additional Rent attributable to the Operating Expenses for the Building and site, based on LESSEE’S Allocable Percentage. Operating Expenses, as set forth in Exhibit B hereto, are the unaudited actuals for calendar year 2006 (and will be subject to change based on actual costs and expenses incurred for each of the categorized Exhibit B costs and expenses in 2007 and each subsequent year during the Extended Term). “Operating Expenses” shall not include the following: the costs of LESSEE’S improvements and services for which LESSEE or any tenant specifically and directly reimburses LESSOR, or pays third persons at LESSOR’S directions; income or franchise taxes of the LESSOR; the costs incurred in any rehabilitation, reconstruction or other work occasioned by any insured casualty (i.e. as to which LESSOR is required to carry insurance hereunder), or by the exercise of the right of eminent domain (except to the extent of any so-called “deductible” amount under policies of insurance or any costs actually incurred for which any insurance company does not reimburse or compensate LESSOR or Owner); depreciation of the Building; general corporate overhead of the LESSOR entity; legal expenses incurred in any direct dispute with any particular tenant (other than those incurred which are of benefit to or protect the rights of other tenants in the Building, generally); costs of renovations to other tenants’ spaces; costs of capital improvements; brokerage and advertising costs in seeking new tenants; and penalties incurred due to LESSOR’S willful violation of any direct violation of any government order.

B. Tax Expenses: LESSEE shall be responsible for payment of Additional Rent attributable to the municipal real estate taxes on the Building and land on which it is situated, based on LESSEE’S Allocable Percentage.

Additional Rent for Operating Expenses and Tax Expenses shall be payable as invoiced by LESSOR (accompanied by a copy of the applicable municipal tax bill), and failure to make any such payments within thirty (30) days of such invoice shall be a default under this Amended and Restated Lease Agreement.

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C. Utilities: Landlord shall provide and Tenant shall pay utilities attributable to the Substituted Leased Premises as set forth in Section 7 of the Original Lease.

**5. Condition of the Current Leased Premises on the Substitution Date; and Landlord's Work on the Substituted Leased Premises**

The Current Leased Premises shall be vacated and surrendered by the LESSEE in accordance with the provisions of Sections 26 and 28 of the Lease, upon delivery of the Substituted Leased Premises to the LESSEE by the LESSOR as contemplated herein.

LESSOR and LESSEE acknowledge that LESSOR shall deliver and LESSEE shall accept delivery of the Substituted Leased Premises as of the beginning of the Extended Term in an AS/IS condition in all respects, but for: (a) the specific work to be performed by LESSOR as set forth in Exhibit C hereto (the "LESSOR'S Work"); (b) in vacant and clean condition; (c) with the laboratory areas "decommissioned" (i.e. free of any environmental hazards or materials such that it is not in violation of applicable environmental laws consistent with the same standards set forth in Section 26 of the Lease) and sanitized. Landlord represents that the Substituted Leased Premises currently has the benefit of a certificate of occupancy from the City of Cambridge therefor which permits the use of the Substituted Leased Premises for the uses permitted under this Lease, and to the extent any additional building permit (or resulting certificate of occupancy is required on account of LESSOR'S Work), then LESSOR shall be required to procure and deliver the same to LESSEE. LESSOR'S Work shall be performed at LESSOR'S sole cost and expense, and shall be substantially completed as of the beginning of the Extended Term. LESSOR shall perform LESSOR'S Work in a good and workmanlike manner and in compliance with all applicable laws and governmental regulations. LESSEE shall inspect such work upon delivery by LESSOR, and hereby acknowledges there are no special installations or other requirements with respect thereto which do not appear on Exhibit C hereto. Notwithstanding LESSOR'S obligation to perform LESSOR'S Work, LESSOR shall not be liable for any damages resulting from LESSEE'S transfer of its operations and equipment from the Current Leased Premises to the Substituted Leased Premises or performance of any of its business functions (e.g. laboratory work) from the newly installed facilities in the Substituted Leased Premises; LESSEE assuming all such risk and waiving any and all claims against LESSOR with respect thereto. If LESSOR is unable to fulfill the conditions set forth above within one hundred twenty (120) days after June 15, 2007, then LESSEE, as its sole and exclusive remedy at law or in equity shall be entitled to terminate this Amended and Restated Lease Agreement by written notice to LESSOR delivered within ten (10) days of the expiration of said one hundred twenty (120) day period, and this Amended and Restated Lease Agreement shall be null and void and without recourse to either party, but LESSEE shall be entitled to remain in occupancy of the Current Leased Premises at the rent set forth in this Amended and Restated Lease Agreement for ninety (90) days after such termination.

LESSOR shall be solely responsible for any costs associated with the architectural and engineering work and permits required for LESSOR'S Work; and LESSOR shall provide such architectural and engineering and permitting services as part of LESSOR'S Work.

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**6. Security/Guaranty**

The LESSEE'S obligations to post and maintain a Security Deposit under Section 5 of the original Lease shall also be required during the Extended Term.

**7. Parking**

The provisions of Section 16 of the Lease shall continue to govern LESSEE'S parking rights and LESSEE shall be entitled to the same number of spaces set forth therein. LESSEE acknowledges that the current rate for such parking spaces is \$ 210.00 per space per month. However, notwithstanding the provisions of Section 16 of the Lease, LESSOR shall determine from time to time in its discretion the extent to which spaces are provided in the LESSOR'S Building or at 808 Memorial Drive (provided all the spaces contemplated in said Section 16 are provided from one location or the other, with a minimum of fifteen (15) to be provided at 840 Memorial Drive).

**8. Brokers Commissions/Indemnification**

LESSOR has retained Meredith & Grew, and LESSEE has had some dealings with said brokerage firm, relative to the Building. The LESSOR and LESSEE each represent to the other that they have not dealt, directly or indirectly, with any other broker, or other entity or individual entitled to any commission relative to the Substituted Leased Premises leased to LESSEE for the Extended Term hereunder. Each party agrees to indemnify and hold harmless the other from and against any claims for commission arising out the execution and delivery of this agreement and any renewals, extensions or expansions hereof other than the foregoing; LESSOR expressly agreeing that it shall be responsible for any claims made by Meredith & Grew relative to this transaction, based on separate agreement between LESSOR and Meredith & Grew.

**9. Assignment/Subletting**

The LESSEE shall have the right to assign the Lease and/or sublet the Leased Premises (in whole or in part) subject to all the terms and conditions of Section 12 of the Original Lease.

**10. Integration of Documents; Supremacy**

The parties hereto intend that this Amended and Restated Lease Agreement incorporate the provisions of the original Lease (to the extent not specifically superseded by the terms and conditions hereof) and that conjunctively these documents constitute the full and complete agreement as between the parties.

The following provisions of the Original Lease are not applicable to the Extended Term: Section 1 (Term); Section 2 (to the extent of the Schedule of Annual Base Rent); Section 3 (as to the first through fourth paragraphs, only); Section 4 (as to the first, and second, and fourth paragraphs, only); Section 22 (solely to the extent that the notice addresses appearing on the execution pages hereof supersede); Section 33 (which is wholly superseded by Section 5 hereof); and Exhibit B (which is wholly superseded by Exhibit B as it is attached hereto).

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As a condition to the effectiveness of this Agreement, LESSOR shall provide to LESSEE either (a) the written acknowledgement of the current mortgagee that the existing Subordination, Non-Disturbance and Attornment Agreement non-disturbance agreement remains in effect and applies to this Amended and Restated Lease Agreement or (b) a new Subordination, Non-Disturbance and Attornment Agreement in the form attached hereto in favor of LESSEE executed by the current mortgagee with respect to this Amended and Restated Lease Agreement.

This Agreement shall be governed by the laws of the Commonwealth of Massachusetts. Any provisions deemed unenforceable shall be severable, and the remainder of this Agreement shall be enforceable in accordance with its terms. This Agreement may only be modified in writing, signed by both parties. Unless otherwise provided herein, all capitalized terms used herein shall have the same meaning as set forth in the Original Lease.

Witness our hands and seals the first date above written.

**RIVERTECH ASSOCIATES II, LLC**

**By Rivertech Associates, Inc. its duly authorized Manager**

/s/ Robert Epstein

**Robert Epstein, President**

Notice Address: 575 Boylston Street 8<sup>th</sup> Floor  
Boston, Massachusetts 02116  
with a copy to: Christopher C. Tsouros, Esq.  
Posternak Blankstein & Lund LLP  
Prudential Tower  
800 Boylston Street Boston,  
Massachusetts 02199

**GENETIX PHARMACEUTICALS, INC.**

By: /s/ Alfred E. Slanetz

its duly authorized

(Attached Secretary/Clerk's Certificate As To Authority)

Notice Address: 800 Memorial Drive 3<sup>rd</sup> Floor  
Cambridge, Mass.  
with a copy to:

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**GENETIX PHARMACEUTICALS, INC.  
AMENDED AND RESTATED LEASE AGREEMENT**

**REVISED EXHIBIT B**

**Operating Expenses 2006**

840 Memorial Drive—Riverside Technology Center

<u>DESCRIPTION</u>	<u>PSF</u>
HEAT	\$ 0.54
BUILDING ELECTRIC	\$2.01
WATER & SEWER	\$0.21
ELEVATOR MAINTENANCE	\$0.15
PARKING/CAFE EXPENSE	\$0.26
RUBBISH REMOVAL	\$0.14
INSURANCE	\$0.36
GROUNDS CARE	\$0.27
LEGAL/ACCT/ADMIN	\$0.11
JANITORIAL SERVICES	\$0.50
GENERAL MAINTENANCE	\$1.38
HVAC MAINTENANCE	\$0.50
LIFE SAFETY SYSTEMS	\$0.11
MANAGEMENT	\$3.28
<b>Total Operating Expenses</b>	<b>\$9.82</b>
<b>Real Estate Taxes (FY 2007)</b>	<b>\$4.63</b>

Note: Actual numbers for 2007 and all subsequent years will vary based on actual costs and expenses incurred.

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**THE ABBEY GROUP**

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Genetix Pharmaceuticals

Relocation to Floor Three, 840 Memorial Drive Revised 5/1/2007

Scope of Work by Landlord

**Office Area**

- All walls to be repainted
- Existing lights to be relamped as required
- Space to be cleaned and prepared for occupancy
- Existing benchtop in support area to be relocated to 3 perimeter offices which presently do not have benchtops.

**Laboratory Area***Physical*

- Repaint all walls
- Vinyl tile floor in main labs and in all non-office areas where seamless tile does not exist.
- New seamless vinyl floor in BL-2A Lab, BL-2B Lab & autoclave/glasswashing room.
- New ceiling and lights throughout lab area.
- Provide existing laboratory cabinets with new bench tops as indicated in the "Floor Plan Exhibit".
- Provide 10 foot long single tier reagent shelving above three benchtops in "Biology Lab A"

*Mechanical*

- Assure proper operation of 10ton supplemental air conditioning supplying main Biology Labs A&B
- Assure proper operation of air conditioning units, humidification and dehumidification system in both BL-2 labs.
- Assure proper operation of cold room.
- Assure proper operation of supplemental air conditioning in equipment room.
- Provide adequate exhaust air for Tenant's (three) six foot hoods, BL2 exhaust hood, BL2 Lab sinks, chemical storage room and autoclave.

*Plumbing*

- Provide separate water supply with approved backflow prevention devices and a hot water tank in or near the location indicated in the "Floor Plan Exhibit".
- Provide drain connection from all sinks to a new acid neutralization system (location to be determined),
- Provide 7 sinks as indicated in plans with eyewash stations. Controls in each of the two BL-2 Labs to be infrared or foot pedal type.
- Provide emergency showers in each BL 2 lab.
- Provide connection of Tenant's glasswasher to cold water, drain -

- 
- Provide connection of Tenant's vacuum and air compressor to existing vacuum and air lines.
  - Provide connection of Tenant's "gas tanks" to Tenant's equipment in the two BL-2 labs.
  - Connect Tenant's ice machine to cold water and indirect drain.

*Electrical*

- Provide separate electric service to the premises including submetering of all equipment.
- Provide electric outlets on the three reagent shelving in Lab "A".
- Relocate, Provide power for and install the following Tenant's equipment:
  - Lab Services Area:*
    - glasswasher.
  - Biology Lab "A"*
    - 20 chest freezer, -20 Revco freezer, -20 Bench freezer, incubator 37°, Sorvall centrifuge, ultracentrifuge, shaker & ice machine.
  - BL-2A Lab equipment -*
    - (1) six foot hood, co2 Nuair Incubator, co2 Thermo incubator, refrigerator.
  - BL-2B Lab equipment -*
    - (2) six foot hoods, co2 stack incubator, GS-6K centrifuge, refrigerator.
- NOTE: Emergency power has, in the past, been taken from the building life safety system which is inconsistent with established building policy and must be remedied by the Tenant's installation of a separate emergency generator, most likely on the second floor roof proximate to both the leased premises and a natural gas supply. The Landlord shall assist the tenant by requesting the "building electrician" to provide Tenant a quote for the appropriate work.

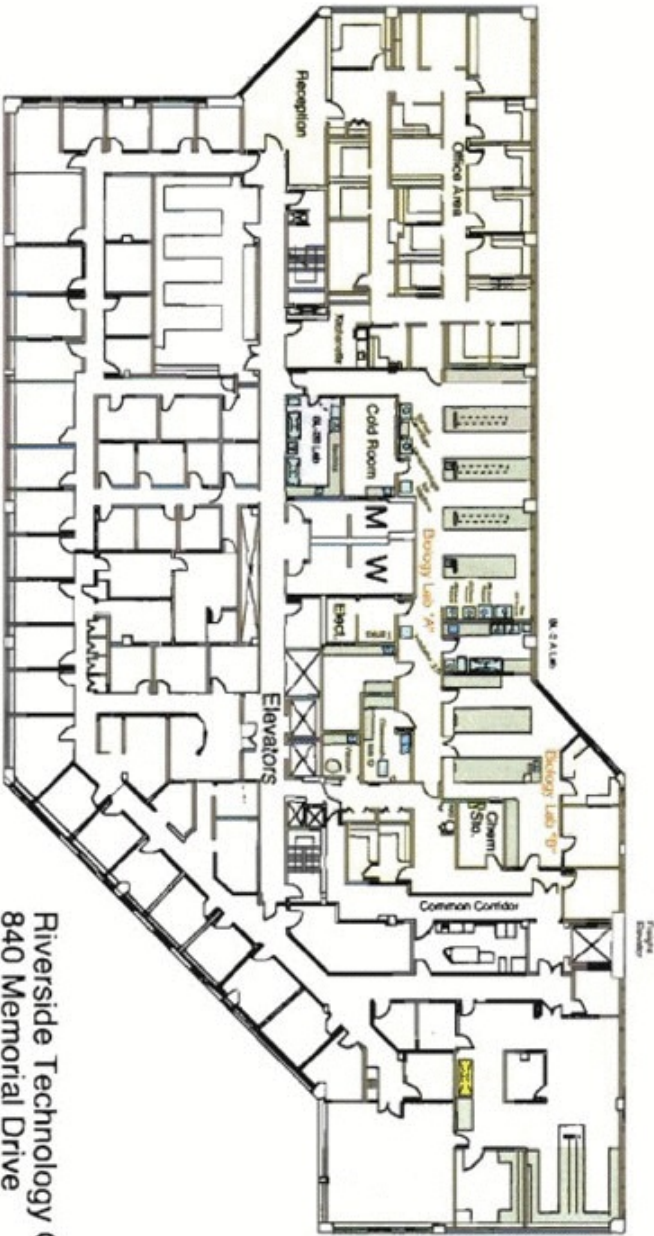
*Telecommunications*

- Landlord shall provide consultant to assist Tenant in the relocation of or purchase of new telecommunications system or but Landlord shall not be responsible for cost of either consultation or work associated with IT or telecommunications.

*Relocation of Equipment*

- The landlord shall arrange the move and reconnection of Tenant's large laboratory equipment as specified in equipment list above to the third floor leased premises.





Boston BioChem  
6,000RSF

Tenant's Equipment  
120V Outlet for Tenant's  
Emergency Generator

Riverside Technology Center  
840 Memorial Drive  
Cambridge, MA

Floor Three  
Genetix Pharmaceuticals  
10,500RSF

**Floor Plan Exhibit**

Revised May 1, 2007

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**RIVERSIDE TECHNOLOGY CENTER  
LEASE EXTENSION AND MODIFICATION AGREEMENT  
TO THE LEASE BETWEEN**

**RIVERTECH ASSOCIATES II LLC AND GENETIX PHARMACEUTICALS, INC.**

This Lease Extension and Modification Agreement entered into this 24th day of November, 2009 by and between **Rivertech Associates II LLC**, a Massachusetts limited liability company with a principal address c/o The Abbey Group, 575 Boylston Street Boston, Massachusetts 02116, (the "Lessor"); and **Genetix Pharmaceuticals, Inc.**, with a business address at 840 Memorial Drive Cambridge, Massachusetts (the "Lessee"); relative to a certain Lease between Lessor's predecessor (Rivertech Associates LLC) and Lessee dated February 18, 2000 referred to herein as the "Original Lease" as amended by a certain Amended and Restated Lease Agreement dated May 18, 2007 (the "Lease Amendment") for certain office and laboratory space in the building at 840 Memorial Drive Cambridge, Massachusetts currently consisting of 10,500 rentable square feet of space on the third (3<sup>rd</sup>) floor of the Building (the "Leased Premises"). The Original Lease, as amended by the Amended and Restated Lease Agreement, shall be referred to herein as the "Amended Lease".

**WHEREAS**, the Lessee desires to extend the Term of the Amended Lease, which is to expire on May 31, 2010, on terms and conditions agreeable to both Lessor and Lessee as a further modification to the Amended Lease, and Lessor assents to such extension of the Term by the Lessee on this basis;

**THEREFORE**, in consideration of One (\$1.00) Dollar and the other good and valuable consideration recited herein, effective and irrevocable as of the date hereof, the Lessor and Lessee hereby agree as follows:

**1. Modification to Original Lease/Extension of Term**

Lessee agrees to lease the Leased Premises commencing as of June 1, 2010 for an additional period of twelve (12) months, beginning June 1, 2010 (the "Extension Commencement Date") and ending on May 31, 2011 (the "Termination Date"); which additional period shall be referred to as the "Extended Term" or "Term".

Notwithstanding the commencement of the Extended Term on the Extension Commencement Date hereunder, this Lease Extension is to be considered a valid and binding obligation of the parties effective as of the date of execution, with the Amended Lease to continue to govern the Lessee's use and occupancy of the Leased Premises hereunder through the Term under the Amended Lease and up to the Extension Commencement Date hereunder. Thereafter, the Amended Lease (including amendment by this Lease Extension and Modification Agreement) shall conjunctively be and shall be referred to as the "Lease" as between the parties for the Extended Term.

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## **2. Terms And Conditions**

Lessee shall lease the Leased Premises commencing as of the Extension Commencement Date on the same terms and conditions of the Amended Lease, as modified by this Lease Extension and Modification Agreement, with exception only for those provisions as to which Lessor and Lessee have already performed their obligations as of the date hereof, (for example, Lessor has heretofore delivered the Leased Premises and Lessee has accepted the same).

## **3. Base Rent and Additional Rent**

Base Rent for each month of the Extended Term shall be Thirty Seven Thousand Six Hundred Twenty Five (\$ 37,625.00) Dollars per month, totaling Four Hundred Fifty One Thousand Five Hundred (\$ 451,500.00) Dollars for the twelve (12) month extension period.

In all instances Base Rent shall be payable in the corresponding monthly installments set forth above, due on the first of each month, in advance, and in all other respects shall be subject to the same provisions relating to Base Rent as set forth under the Original Lease.

In addition to Base Rent, Lessee shall continue to be responsible to pay all Additional Rent as set forth in Section 4 of the Amended Lease, consisting of Operating Expenses under Section 4A and Tax Expenses under Section 4B and Utilities under Section 4C of the Amended Lease; and all conditions thereunder are hereby restated and affirmed and shall govern the use and occupancy of the Leased Premises throughout the Extended Term.

## **4. Leased Premises in "AS/IS" Condition—No Defaults**

Lessee hereby acknowledges it is currently in possession of the Leased Premises and accordingly accepts the same for the Extended Term in its current "AS/IS" condition, without representation or warranty of any kind or nature arising from the extension of the Lease by Lessor and Lessee.

Lessor and Lessee each acknowledge that to the best of each of their respective knowledge, there are no material defaults by either presently existing under the Lease.

## **5. Brokers**

The parties hereby agree there are no brokerage or other third party fees or costs involved in this transaction and each agrees to indemnify, defend and hold harmless the other from and against any claims for brokerage fees, commissions or other such payments arising from this transaction.

## **6. Integration Of Documents: Supremacy**

The parties hereto intend that this Lease Extension and Modification Agreement operates to amend and modify the Amended Lease, and that those two documents shall be interpreted conjunctively; with any express conflict between the two to be resolved in favor of the stated terms of this Lease Extension and Modification Agreement. Except as modified hereby, all other terms and conditions of the Amended Lease shall remain unchanged and enforceable in a manner consistent with this Lease Extension And Modification Agreement.

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This Agreement shall be governed by the laws of the Commonwealth of Massachusetts. Any provisions deemed unenforceable shall be severable, and the remainder of this Lease Extension and Modification Agreement and the Original Lease shall be enforceable in accordance with their terms.

Witness our hands and seals as of the date first written above.

**LESSOR**

**RIVERTECH ASSOCIATES II, LLC**

By: /s/ Robert Epstein  
its duly authorized Manager

**LESSEE**

**GENETIX PHARMACEUTICALS, INC.**

By: /s/ Alfred E. Slanetz  
its duly authorized President/Vice President

By: Illegible Signature  
its duly authorized Treasurer/Ass't Treasurer

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**RIVERSIDE TECHNOLOGY CENTER**  
**SECOND LEASE EXTENSION AND MODIFICATION AGREEMENT**  
**TO THE LEASE BETWEEN**  
**RIVERTECH ASSOCIATES II LLC AND BLUEBIRD BIO, INC.**

This Second Lease Extension and Modification Agreement (the “Second Lease Extension Agreement”) entered into this 12<sup>th</sup> day of September, 2012 by and between **Rivertech Associates II LLC**, a Massachusetts limited liability company with a principal address c/o The Abbey Group, 575 Boylston Street Boston, Massachusetts 02116 (successor in interest as stated below, herein, the “Lessor”), and **Bluebird Bio, Inc.**, with a business address at 840 Memorial Drive Cambridge, Massachusetts (successor in interest as stated below, herein the “Lessee”), with respect to a certain Lease dated February 15, 2000 (as amended, as stated below) for certain office space in the building at 840 Memorial Drive Cambridge, Massachusetts.

WHEREAS, Rivertech Associates, LLC and Genetix Pharmaceuticals, Inc. entered into a certain lease agreement dated February 18, 2000 (the “Original Lease”); and,

WHEREAS, Rivertech Associates, LLC and Genetix Pharmaceuticals, Inc. entered into a certain Amended and Restated Lease Agreement dated May 18, 2007 (the “First Amended Lease Agreement”); and,

WHEREAS, Rivertech Associates II, LLC (as successor in interest to Rivertech Associates, LLC, the original signatory as Lessor under the Original Lease and First Amended Lease Agreement), and Genetix Pharmaceuticals, Inc., entered into a certain Lease Extension and Modification Agreement dated November 24, 2009 (the “First Lease Extension Agreement”); and,

WHEREAS, Rivertech Associates II, LLC and Bluebird Bio, Inc. (a Delaware corporation, formerly known as Genetix Pharmaceuticals, Inc.), entered into a certain Second Amended and Restated Lease Agreement dated October 19, 2010 (the “Second Amended Lease Agreement”); collectively, the Original Lease as amended and modified by the foregoing First Amended Lease Agreement, Lease Extension Agreement, and Second Amended Lease Agreement being referred to herein as the “Existing Lease”; and,

WHEREAS, the current Term under the Existing Lease expires on November 30, 2014 (the “Current Term”), and the Lessee seeks to extend the Current Term so as to expire on March 31, 2015, which date is referred to herein as the “Extended Term Termination Date”, and which entire term period as extended is referred to herein as the “Extended Term”; and,

WHEREAS, under the Existing Lease the Lessee leases and occupies approximately 9,488 rentable square feet of space located on the fourth (4<sup>th</sup>) floor of the Building, in addition to approximately fifty (50) rentable square feet of space on the third (3<sup>rd</sup>) floor of the Building, for a total of approximately 9,538 rentable square feet of space in the Building, collectively known under the Existing Lease as the New Leased Premises and referred to herein as the “Existing Premises”; and,

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WHEREAS, by this Second Lease Extension Agreement Lessee seeks to add approximately 8,060 rentable square feet of space located on the fourth (4<sup>th</sup>) floor of the Building, in addition to approximately fifty (50) rentable square feet of space on the third (3<sup>rd</sup>) floor of the Building, for a total of approximately 8,110 rentable square feet in the Building, as shown on Exhibit A hereto and collectively known as the “Expansion Space”; which, when added to the Existing Premises is collectively referred to herein as the “Total Leased Premises”; and,

WHEREAS, Rivertech Associates II, LLC and Bluebird Bio, Inc., seek by this current agreement to further amend and modify the Existing Lease to farther extend the Current Term of the Existing Lease, and to lease the Expansion Space, as set forth in detail below and under the terms and conditions of the Existing Lease as it is amended and modified hereby;

**THEREFORE**, in consideration of One (\$1.00) Dollar and the other good and valuable consideration recited herein, effective and irrevocable as of the date hereof, the Lessor and Lessee hereby agree as follows:

**1. Modification to Existing Lease / Extension of Current Term**

The Existing Lease expires on November 31, 2014 at the end of the Current Term. Lessee agrees to extend its tenancy as to the Existing Premises, commencing as of the end of the Current Term under the Existing Lease (i.e. from November 31, 2014), for an additional four (4) month period beginning on December 1, 2014 (the “Extension Commencement Date”) and ending on March 31, 2015 (the “Termination Date”); which additional period shall be referred to as the “Extended Term” or “Term”. Further, from the Expansion Space Delivery Dates (as defined herein) through the end of the Extended Term, Lessee also agrees to lease the Expansion Space (as defined herein) on the terms and conditions set forth herein.

Notwithstanding the commencement of the Extended Term on the Extension Commencement Date hereunder, this Lease Extension is to be considered a valid and binding obligation of the parties effective as of the date of execution of this Second Lease Extension Agreement by the parties and its approval by Lessor’s lender (which approval is an express condition to the extension and expansion contemplated herein and which Lessor shall seek immediately upon execution of this Agreement by both parties); with the Existing Lease to continue to govern the Lessee’s use and occupancy of the Existing Premises hereunder through the Term and up to the Extension Commencement Date hereunder, subject to the supplemental provisions hereof relating to the Expansion Space (defined herein). Lessee agrees to execute the Landlord’s lender’s standard Subordination Non-Disturbance and Attornment Agreement upon execution of this Second Lease Extension Agreement and Lessor agrees to promptly use commercially reasonable efforts to seek its lender’s execution of the same, but without any obligation to actually deliver the same to Lessee and without any material impact on this Second Lease Extension Agreement or the underlying Existing Lease upon any failure to do so.

**2. Terms and Conditions**

Lessee shall lease the Total Leased Premises (with the addition of the Expansion Space to be leased as of the Expansion Space Delivery Date) on the same terms and conditions of the Existing Lease, as modified by this Second Lease Extension Agreement, with exception only for

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those provisions as to which Lessor and Lessee have already performed their obligations as of the date hereof, (for example, Lessor has heretofore delivered the Existing Premises and Lessee has accepted the same). The Existing Premises is leased in the same "AS/IS" condition as it is as of the execution of this Second Lease Extension Agreement, and Lessee acknowledges Lessor is under no obligation to make any improvements or modifications thereto, in any manner.

### **3. Expansion Space Added to the Existing Premises**

Lessor shall deliver the Expansion Space to Lessee (to be added to and, in the aggregate, to constitute the Total Leased Premises). The Expansion Space shall be delivered to the Lessee upon Substantial Completion (as defined below) of Lessor's Work (as defined herein below) on the office portion of the Expansion Space (the "Office Expansion Space") and on the laboratory portion of the Expansion Space (the "Lab Expansion Space"), herein, the "Office Expansion Delivery Date" and the "Lab Expansion Delivery Date" respectively, and also referred to herein collectively as the "Expansion Delivery Dates". As of the respective Expansion Delivery Dates, the Office Expansion Space and the Lab Expansion Space, respectively, shall be Substantially Complete, vacated by any current tenants and occupants; with Lessor's Work having been performed in a good and workmanlike manner according to Lessor's Scope of Work; broom clean. All Lessee's Rent payments and other Lease obligations relating to the Expansion Space shall commence as of the Expansion Space Office Delivery Date and the Expansion Space Lab Delivery Date, respectively. All terms and conditions of the Lease shall govern the Lessee's use and occupancy of the Expansion Space as of the respective Expansion Space Delivery Dates.

Lessor's delivery of the Office Expansion Space and the Lab Expansion Space shall be evidenced in each instance by a written notice of delivery ("Lessor's Delivery Notice") given to Lessee on the actual date the respective portion of the Expansion Space is Substantially Complete and provided to Lessee for its occupancy. Lessee shall have five (5) business days to contest delivery if the Expansion Space is not Substantially Complete or it does not conform with the Lessor's Scope of Work and Lessor's Work by delivering its notice thereof in writing to Lessor; however, any listed items of a "punchlist" nature shall be agreed to by Lessor and Lessee and shall not be grounds to contest delivery, but nevertheless shall obligate Lessor to complete such punchlist items at the earliest practicable time under the circumstances.

The following conditions to the delivery of the Office Expansion Space and the Lab Expansion Space to the Lessee by the Lessor shall be met by the Lessor, at its sole cost and expense, prior to the respective Expansion Delivery Dates. The Lessor shall perform, at its sole cost and expense, such design and construction work as is necessary to deliver the respective portions of the Expansion Space to the Lessee in accord with the "Scope of Landlord's Work for Additional Premises Only" dated August 13, 2012 attached hereto as Exhibit A (the Exhibit referred to as the "Lessor's Scope of Work") and the design and construction obligations thereunder being referred to as the "Lessor's Work"). All components of Lessor's Work will be completed in accordance with all applicable laws, rules and regulations, including but not limited to the latest requirements of NFPA, ANSI Standards, ASHRAE Standards, National Electrical Code, Massachusetts State Building Code, and regulations of the City of Cambridge. Lessor shall deliver the Expansion Space with the base Building systems serving the same and with Lessee's specific mechanical, electrical and plumbing systems as required in Lessor's Scope of Work (i.e. Exhibit A hereto), in good operating condition and repair, and suitable for their intended uses.

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All utilities for the Expansion Space shall be in place and separately metered. The Building and the Expansion Space as delivered to the Lessee will be compliant with the Americans with Disabilities Act; NFPA compliant pursuant to the Massachusetts State Building Code; and with code compliant demising walls and common area corridors. Lessor shall provide Lessee with the environmental close-out report prepared by the former tenant for the Expansion Space, and said report shall not disclose any conditions as would materially impair Lessee's use of the Expansion Space. Subject to the foregoing, Lessor shall not be responsible for any other design or construction work with respect to either the Existing Premises under the Existing Lease, or the Expansion Space.

To the extent Lessee seeks to make any changes to the Lessor's Scope of Work as set forth on Exhibit A hereto: (i) such changes will be communicated in writing with sufficient specificity for Lessor to price the changes; (ii) Lessor will provide Lessee with pricing as to such changes and any estimated delays, if any, that may result for such changes; and (iii) in the event such changes affect the total cost of the Lessor's Scope of Work, Lessee shall either reimburse Lessor in advance for any increased costs and expenses, or Lessor shall credit Lessee for any savings against Lessee's next due payments of Rent hereunder. Upon receiving from Lessor the estimated cost and timing impacts of Lessee's proposed changes pursuant to subsection (ii) above, Lessee may choose to not move forward with such proposed changes provided it communicates its final intention to the Lessor in writing within ten (10) days of its receipt of Lessor's cost and timing impacts. Lessee shall be solely responsible for any delays in the completion of Lessor's Work arising from any such requested changes.

The Lessee shall be solely responsible, at its sole cost and expense, to perform such other specific design and construction work on the Expansion Space as it desires for its use and occupancy ("Lessee's Work"), upon completion of the Lessor's Work in the respective office and laboratory portions of the Expansion Space, and delivery of those respective portions the Expansion Space by the Lessor. Lessee shall be provided with access to the Expansion Space commencing upon execution of this Second Lease Extension Agreement, coordinated through the Lessor, for the purpose of performing preliminary work toward the installation of its equipment and wiring, provided such access and preliminary work does not materially interfere with Lessor's ability to perform and complete its Lessor's Work, which shall take precedence in all respects. Lessee's Work and all subsequent Lessee alterations to the Leased Premises that are performed by Lessee on or affecting the fire, life safety and/or sprinkler systems of the building shall be made in such a manner and under such conditions as to pose no adverse impact or interruption to such fire, life safety, and sprinkler systems, and so as not to delay, impair, or jeopardize the legal occupancy of other tenants in the Building as determined by Lessor and municipal fire and building inspection officials.

The Lessor's Work as to the Office Expansion Space and the Lab Expansion Space, separately, will be deemed "Substantially Complete" as to each: (a) when Lessor's Work is substantially complete in accordance with Lessor's Scope of Work as set forth in Exhibit A and the provisions of this Section 3, except for minor punch list items approved by Lessee that will not materially adversely affect Tenant's normal operations in said Expansion Space; Lessor's Work therein having been performed in a good and workmanlike manner with all necessary municipal approvals for occupancy.



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**4. Annual Base Rent and Additional Rent**

Annual Base Rent from the date of execution of this Second Lease Extension Agreement through the Extended Term, shall be as set forth below:

A. Annual Base Rent as applied to the Existing Premises  
(i.e. 9,538 rentable square feet of space)

- (i) Balance of the Current Term As set forth in the Existing Lease
- (ii) Extension Commencement Date through the Termination Date \$ 145,136.57 (\$ 36,284.14 / mo.)

B. Annual Base Rent as applied to the Expansion Space (i.e. 8,110 rentable square feet of space)

(i) Expansion Space Staggered Delivery Periods—Interim Rent/Per Diem

Lessor's estimated delivery of the Office Expansion Space is targeted to October 1, 2012. Lessor's estimated delivery of the Lab Expansion Space is targeted to December 1, 2012. The first day of the calendar month following the day on which the Office Expansion Delivery Date occurs is referred to herein as the "Expansion Rent Start Date". A per diem calculation is required to determine interim Rent obligations occurring upon delivery of the respective Office Expansion Space and Lab Expansion Space. Annual Base Rent for the total Expansion Space (office and laboratory) for the first full twelve month period following the Expansion Rent Start Date is \$395,362.50 (\$32,946.88 on a monthly basis). This computes to \$1,083.18 on a per diem basis. The per diem amount allocated to the Office Expansion Space is \$496.42, (i.e. 45.83% at 3,717 rentable square feet of a total 8,110 rentable square feet), the "Office Per Diem". The per diem amount allocated to the Lab Expansion Space is \$586.76 (i.e. 54.17% at 4,393 rentable square feet of a total 8,110 rentable square feet), the "Lab Per Diem".

(x) For the interim period between the Office Expansion Delivery Date and the Expansion Rent Start Date, the Annual Base Rent payment attributable to the Office Expansion Space shall be determined by taking the number of days from the Office Expansion Delivery Date through the Expansion Rent Start Date and *multiplying by* \$496.42. This amount shall be paid to Lessor.

(y) For the interim period between the Lab Expansion Delivery Date and the Expansion Rent Start Date (given the possibility the Expansion Rent Start Date may not have occurred due to delays in delivery of the Office Expansion Space), the Annual Base Rent payment attributable to the Lab Expansion Space shall be determined by taking the number of days from the Lab Expansion Delivery Date through the Expansion Rent Start Date (if applicable) and *multiplying by* \$586.76. This amount (if applicable) shall be paid to Lessor.

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(ii) Annual Base Rent due from the Expansion Rent Start Date through the next twelve (12) consecutive months is:

\$ 395,362.50 (\$ 32,946.88 /mo.)\*

\*[less the Lab Per Diem for each day in such period that the Lab Expansion Space is not delivered]

(iii) Annual Base Rent due through the next twelve (12) consecutive months (after (ii) above) is:

\$ 405,500.00 (\$ 33,791.60/mo.)

(iv) Annual Base Rent due through the next consecutive months (after (iii) above) to the end of the Extended Term is:

\$ *variable* (\$ 34,636.46 / mo.)

In all instances under A and B above, Annual Base Rent shall be payable in the corresponding monthly installments set forth above, due on the first of each month, in advance, and in all other respects shall be subject to the provisions relating to Annual Base Rent as set forth under the Existing Lease.

**C. Additional Rent.**

In addition to Annual Base Rent, Lessee shall continue to be responsible to pay all Additional Rent (Operating Expenses) under Section 3 of the Existing Lease, and all Additional Rent (Taxes) under Section 4 thereof, as applicable to both the Existing Premises and the Expansion Space, as invoiced by Lessor during the Extended Term. A current statement outlining the Operating Expenses incurred for the Building in 2011 is attached hereto as Exhibit B.

As the concept is used in the Lease to compute Additional Rent, Lessee's allocable pro rata share ("Allocable Percentage") shall be as follows:

(A) Allocable Percentage for the Existing Premises running through the end of the Term, shall be 7.4 %.

(B) Allocable Percentage for the Expansion Space, starting on the Expansion Space Delivery Dates and running through the end of the Term, shall be 6.29 %.

To the extent that the Expansion Space Commencement Dates do not fall on the first calendar day of a month, then the first month in which the Expansion Space Commencement Dates occur will have Additional Rent attributable to the Expansion Space prorated on a per diem basis for that month.

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**D. Rent and other Costs and Expenses.**

All Annual Base Rent, Additional Rent and other sums due as Rent shall be payable and in all other respects shall be governed during the remainder of the Current Term, and for the Extended Term, as contemplated under the Existing Lease, except to the extent modified and supplemented above. All other costs and expenses for utilities and services and attendant to operation of the Total Leased Premises (i.e. as applicable to both the Existing Premises, and to the Expansion Space as of the respective Expansion Space Delivery Dates), shall be borne by the respective parties as set forth in the Existing Lease.

**E. Security Deposit.**

The Security Deposit currently held by the Lessor shall continue to be held by Lessor during the Extended Term.

**5. Permitted Uses**

The Permitted Uses for the Total Leases Premises shall be office, R&D and laboratory use in conformity with all federal, state, municipal and local laws, codes, ordinances, rules and regulations of governmental authorities, committees, associations, or other regulatory committees, agencies or governing bodies having jurisdiction over the Total Leased Premises, and in conformity with the conditions and restrictions set forth in the Original Lease (which are hereby restated and affirmed by Lessee).

**6. Existing Premises in “AS/IS” Condition—No Defaults**

Lessee hereby acknowledges it is currently in possession of the Existing Premises and accordingly accepts the same from the date of this Second Lease Extension Agreement in its current “AS/IS” condition, without representation or warranty of any kind or nature arising from the extension of the Lease by Lessor and Lessee.

Lessor and Lessee each acknowledge that to the best of each of their respective knowledge, there are no material defaults by either presently existing under the Lease.

**7. Brokers**

The parties hereby agree there are no brokerage or other third party fees or costs involved in this transaction and each agrees to indemnify, defend and hold harmless the other from and against any claims for brokerage fees, commissions or other such payments arising from this transaction.

**8. Parking.**

Lessee shall be granted, at current rates (which may be increased from time to time to reflect market increases), the right (but not the obligation) to park up to twenty seven (27) cars in total in the Building’s on-site indoor parking lot or facility on an unassigned and unreserved basis, in single or tandem spaces or on a valet basis which Lessor in its sole discretion shall designate from time to time. The initial parking rate therefor shall be \$225 per month, per car, which monthly rate may be changed by Lessor in its discretion subject to and reflective of periodic

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market changes. Additionally, Lessee shall be entitled to rights to park additional cars in the Building garage (but subject to availability as determined by Lessor, and only on a valet basis, and then only to the extent Lessor is providing valet service to the Building garage which Lessor shall not be obligated to do), at then current rates as set by Lessor in its discretion. If Lessor cannot accommodate Lessee's needs for additional parking in the Building garage, then Lessor will assist Lessee in identifying alternative off-site parking, but Lessor shall bear no liability nor shall it be deemed any default under the Lease if such additional Building garage parking or off-site parking do not materialize. All payments for these parking rights shall be considered to be Additional Rent under this Lease. This provision supersedes any contrary provisions of the Existing Lease and the specific numeric rights set forth above supplant the numeric rights otherwise set forth in the Existing Lease.

#### **9. Lessee's Option to Extend**

Provided Lessee has not defaulted (after applicable notice, grace and cure periods, if any) under the Lease from the date hereof forward more than two (2) times, and further provided no uncured default on the part of Lessee is then existing, Lessee shall have the option to further extend the Term of this Lease as to the Total Leased Premises (i.e. inclusive of the Expansion Space) on the terms and conditions herein, for one additional period of thirty six (36) months (herein, the "Additional Term Extension Period") at the then current "Market Rent" (including annual escalations thereon for each year of the extended term based on increases in the Consumer Price Index or fixed increases, as the case may be, as determined by then prevailing market forces), but no less than an amount equal to the annualized Rent per rentable square foot of Total Leased Premises space as of the final full month of the last Lease Year hereunder (the "Extension Rent Floor"). Said Additional Term Extension Period shall commence, subject to proper exercise of Lessee's option hereunder, at the end of the Extended Term (i.e. at the end of March, 2015) and shall terminate on that date which is thirty six (36) consecutive months thereafter (i.e. March 31, 2018). Lessee shall exercise its option by delivering to Lessor its written notice not later than ten (10) full months (but not sooner than twelve (12) full months) prior to the end of the Extended Term. Once delivered, written notice to extend is irrevocable.

"Market Rent" as used herein, shall be that rent charged for comparable research laboratory and office space of similar age and condition in laboratory buildings the mid-Cambridge submarket as of the end of the Extended Term. If, after good faith attempts prior to the expiration of the original Term, the Lessor and Lessee cannot agree on a figure representing Market Rent, then either party, upon written notice to the other, may request appraisal and arbitration of the issue as provided in this section. Within fourteen (14) days of the request for arbitration, each party shall submit to the other the name of one unrelated individual or entity with proven expertise in the leasing of commercial real estate in greater Boston/Cambridge to serve as that party's appraiser. Each appraiser shall be paid by the party selecting him or it. The two appraisers shall each submit their final reports to the parties within thirty (30) days of their selection making their determination as to Market Rent (subject however, to the Extension Rent Floor). The two appraisers shall meet within the next fourteen (14) days to reconcile their reports and collaboratively determine the Market Rent. They shall each make their determination in writing (subject however, to the Extension Rent Floor), including a statement if such is the case, that they are at an impasse. Such a statement of impasse shall be submitted to the parties along with the Market Rent figure which each appraiser has selected and his reasons and substantiation

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therefor. The appraisers, in case of an impasse, shall also agree on one unrelated individual or entity with expertise in commercial real estate in greater Boston, who shall evaluate the reports of the two original appraisers and within fourteen (14) days of submission of the issue to him, make his own determination as to a figure representing Market Rent (subject however, to the Extension Rent Floor). The determination of this individual or entity (i.e. arbitrator) absent, fraud, bias or undue prejudice shall be binding upon the parties.

Annual Base Rent and Additional Rent during any Additional Term Extension Period shall be payable in advance, in equal monthly installments on the first day of each calendar month.

Lessee, in addition to the sums payable annually to Lessor as Annual Base Rent, shall pay to Lessor for each year of the Additional Term Extension Period, as Additional Rent, Lessee's Allocable Percentage (as determined by the approximate total rentable space leased) for Operating Expenses, Real Estate Taxes and utilities as contemplated in Section 4 hereof.

**10. Access; Elevators; Dumpsters; Signage**

Lessee shall have 24/7 access to the Total Leased Premises (with regard to the Expansion Space, such access shall be allowed following the applicable Expansion Delivery Dates), the common areas and elevators serving the Total Leases Premises, the freight elevators servicing the Building, the loading docks servicing the Building and the dumpster and/or compactor servicing the Building.

Lessor shall provide, at Lessor's expense, building standard signage in the Building's lobby, in any Building directory, and at the main entrance to the Total Leased Premises.

**11. Integration of Documents; Supremacy**

This Second Lease Extension Agreement contains the full understanding and agreement between the parties. The parties hereto intend that this Second Lease Extension Agreement operates to amend and modify the Existing Lease, and that those documents shall be interpreted conjunctively; with any express conflict between the two to be resolved in favor of the stated terms of this Second Lease Extension Agreement. Except as modified hereby, all other terms and conditions of the Existing Lease shall remain unchanged and enforceable in a manner consistent with this Second Lease Extension Agreement. Defined terms used in this Second Lease Extension Agreement that are not otherwise defined herein shall have the definitions ascribed to such terms in the Existing Lease.

This Agreement shall be governed by the laws of the Commonwealth of Massachusetts. Any provisions deemed unenforceable shall be severable, and the remainder of this Second Lease Extension Agreement and the Existing Lease shall be enforceable in accordance with their terms.

**[Signature Pages Follow]**

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Witness our hands and seals as of the date first written above.

**LESSOR**

**RIVERTECH ASSOCIATES II, LLC**

By: /s/ Robert Epstein  
its duly authorized Manager

**LESSEE**

**BLUEBIRD BIO, INC.**

By: /s/ Nick Leschly  
its duly authorized President/Vice President

By: /s/ Jeffrey T. Walsh  
its duly authorized Treasurer/Ass't Treasurer

**See Attached**

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**BLUEBIRD BIO**

Floor Four  
840 Memorial Drive  
Cambridge, MA

AUGUST 13, 2012

**SCOPE OF LANDLORDS WORK FOR ADDITIONAL PREMISES ONLY**

\*\*\* Space will be emptied of all furnishing prior to move-in date

**Partitions:** Partitions are to be removed and relocated as indicated on the accompanying plans. Existing and new walls are comprised of gwb over steel studs and extend from floor to the underside of the suspended ceiling. The walls demising the Administrative areas from R&D areas extend to the underside of the deck above. All walls will be finished with two coats water based paint. The entire office space will have 2 new coats of water based paint to match bluebird's existing space color scheme.

Existing "lavatory" room will have plumbing removed and remain as storage room. New storage room will have VCT removed and new matching carpeting.

**Glass Panels:** Where they exist, glass panels to remain. Glass panels from 4131 and 4130 will be removed to allow for the respective doors to be relocated. This is to allow for the modern-fold partition to be stored on the wall in between the doors when it is not separating 4131 and 4130.

**Entry:** Existing glass entry to remain.

**Doors:** Existing doors will be reused and relocated as necessary provided they have existing full lite glazing. All hardware are lever handles with brushed stainless finish. New office doors will be installed with full lite glazing where applicable. All office doors will have full lite glazing. Office and conference room door hardware will be comprised of passage function latchsets with brushed stainless finish. Storage room latchsets will be comprised of class room function latchsets with brushed stainless finish.

**Floors:** All carpeted areas in the administrative which are affected by construction will be re-carpeted with matching Shaw Contract nylon loop carpet from "Turn Key Collection. 4" vinyl cove base has been installed at intersection of walls and carpet/vct. Vinyl tile installed in the floor of the kitchenette to remain. VCT will be removed from the storage area and be replaced with matching carpeting, 4" vinyl cove base will be installed in the storage areas as well as areas affected by construction.

**Ceilings:** All existing ceiling tiles will remain or be replaced as necessary. Any damaged ceiling tiles will be replaced with matching ceiling tiles. Any damage to ceiling grid will be repaired.

**Lighting:** All existing fluorescent lights will remain and be relocated as necessary to accommodate the new configuration. All existing fluorescent lights will be inspected to insure proper functioning and be repaired or replaced as necessary.

BluebirdBio Final Scope of Work

8/13/2012



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HVAC: The base building HVAC distribution system will be inspected and adjusted as necessary to assure distribution, airflow, and proper operation of thermostats and variable air volume (VAV) boxes. There will be a redistribution of the system to accommodate 5 separate zones as indicated on the drawings. This work will be designed by landlord's consulting engineer. The office area will be provided with make-up air from either the 30 ton roof mounted MUA unit or house systems in order to provide fresh air to the office and maintain positive pressure with respect to R&D space. A pre-construction balancing/CFM reading will be done to show existing air balance in the space. The landlord's engineer will perform HVAC load calculations to take into account insulation, square footage, occupancy and equipment heat loss to determine heating and cooling loads. Expected equipment and occupant load shall be provided to the landlord's engineer for this purpose. The space will be balanced according to these calculations. All balancing will be conducted by a NEEB certified balancing technician. Pre-construction balancing, engineering calculations and post-construction "as built" documentation/balancing reports will be provided to the tenant or the tenant's designee. The new space will conform to ASHRAE Standard 55 "Thermal Environmental Conditions for Human Occupancy". All HVAC equipment shall be inspected by the landlord and replaced as necessary. The lessee accepts the existing HVAC system provided that it is in good working order, that it conforms to ASHRAE Standard 55, and that a reasonable temperature is maintained in the office areas. In the event during the lease term the HVAC system does not meet the aforementioned prerequisites the landlord will provide a new system which will be replaced at the Landlord's cost. The tenant will be responsible for maintaining and repairing all tenant specific supplemental mechanical equipment beyond the 6 month warranty period. Tenant specific mechanical equipment will be delivered in good operating condition.

Kitchenette: The kitchenette will remain undisturbed.

Electrical: Existing electrical outlets throughout the administration area and the office space in the R&D area in the form of existing and new 110v outlets to remain. All utilities servicing the tenant's premises and equipment are separately metered and will be read monthly by the landlord for reimbursement by the tenant. New outlets will be installed to accommodate the new layout. Each office will have a minimum of 3 120/20a 5-20R receptacles. All electrical circuits will be labeled via labels at the outlet, junction box, safety switch, or other corresponding electrical equipment and a corresponding label on the electrical panel indicating the appropriate circuit breaker.

Furnishings: No reception desk cubicles, work stations or furniture of any sort shall be provided by the landlord.

Specialties: A demountable partition by Modernfold or equal will be installed as indicated in the plans. This wall will have a "Dry Erase" surface from floor to ceiling. BluebirdBio or their designee will have access to the space during the renovation to inspect progress and that the work being done conforms to this scope.

Fire Protection: Fire protection will be added as needed and required by Massachusetts Building code, NFPA and local Fire Code.

Work included in this scope will comply with the Massachusetts Building Code and NFPA.

BluebirdBio Final Scope of Work

8/13/2012

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## **R&D AREA**

Demolition: Existing lab benches, plumbing, piping, vinyl tile, doors and walls required to be removed to accommodate new plan will be removed. The existing 6' hood shall be capped and removed from the premises. The existing wing wall adjacent to the hood shall be removed per attached drawings.

The entire R&D area will have 2 coats water based paint applied per Bluebird bio's existing paint scheme

New Partitions: New partitions enclosing new tissue culture suites shall consist of existing walls comprised of 5/8" gwb over 3 1/2" steel studs and extending from floor to 6" above the underside of the suspended ceiling. Walls and trim will be finished with two coats water based paint. Paint finish in tissue culture rooms to be semi gloss.

The tissue culture rooms will be separated from the main laboratory by a hallway as indicated in the drawing.

Existing glass panels in the exterior offices shall be removed.

The walls surrounding the "radio-isotope room" shall extend to the underside of the deck above and have an additional layer of 5/8" gwb applied to each accessible side of the wall. Walls will be constructed to the deck above with 2 layers of 5/8" GWB. Walls will be completely sealed with appropriate sealant in order to prevent any contamination of adjoining spaces. Work will be consistent with attached drawing and keynotes.

The existing exterior window in the office to become the darkroom shall be blacked out via either applied opaque vinyl or an opaque panel which will prevent any light from entering via the window.

Doors: Where indicated on the drawings, Horton Ultra-Clean Series Atmospheric 110 doors with push plate activators and safety scan sensors will be installed. Buttons to activate the doors will be installed.

A 36" diameter circular darkroom door shall be installed in the office to become the darkroom as indicated on the plans. The door shall fit within a 36" wall opening and have a "pop-out" feature with breakaway hardware for use in emergencies and shall conform to Massachusetts Building Code requirements.

Floors: All new tissue culture suite floors will be covered with seamless vinyl with integral vinyl base. Floors where noted in the drawing as indicated by keynotes, to include the radio-isotope room and hallway, will be covered with seamless vinyl with integral vinyl base. The damaged vinyl floor in the autoclave room will be repaired.

Ceilings: Except for the tissue culture suite, existing building standard ceiling tiles will remain in the existing grid. In the Tissue Culture Suites, and as indicated in attached drawing keynotes, to include the radio isotope room and hallway, new solid surface washable ceiling tiles will be installed in the new ceiling grid with vinyl gasketing. Any damaged ceiling tiles will be replaced. Any damage to ceiling grid will be repaired.

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Lighting: Except for the new Tissue areas, existing 2x4 and 2x2 fluorescent lights will remain. Solid surface lens type fluorescent lights will be used in the Tissue Culture, radio-isotope, and Glasswash/Autoclave rooms.

BENCHES: The three remaining lab benches with sink will remain. Oak benches with stainless steel sinks and acid resistant plastic laminate tops will be installed in the two new tissue culture suites while the existing bench and sink in the existing tissue culture suite shall be relocated as indicated on the plans. Oak benches/casework with a stainless steel sink and epoxy resin counter top will be installed in the radio-isotope room in the form of an L-shape as indicated in the attached drawing. The sink in the radio-isotope room will have a motion activated faucet. The casework and countertop in the radio-isotope room will be designed such that space for a 4' fume hood or BSC be provided for future installation. Each lab sink will have protected hot and cold water and deck mounted emergency eyewash stations.

HVAC: The base building HVAC distribution system will be inspected and adjusted as necessary to assure good operating condition and repair, distribution, airflow, and proper operation of thermostats and variable air volume (VAV) boxes. The existing supplemental air conditioning units will be inspected and put in good operating condition and repair. One of these supplementary systems will service the main laboratory (4114), the second will service the existing tissue culture suite 4118. The third existing unit will service the new tissue culture suite (4119) or suite's (4119 and 4120) depending on HVAC load requirements. If necessary a new unit with integral electric heat will serve the tissue culture suite (4120). The air conditioning units should correspond to what HVAC heating and cooling is required, as indicated by analysis conducted by the landlord's engineer. All tenant specific mechanical systems shall be warranted for proper operation for a period of six months provided tenant enters into an appropriate preventive maintenance agreement for this equipment as stated below. Tenant specific mechanical equipment will be delivered in good operating condition. A pre-construction balancing/cfm reading will be done to show existing air balance in the space. The landlord's engineer will perform load calculations to take into account insulation, square footage, occupancy and equipment heat loss to determine heating and cooling loads. Expected equipment and occupant load shall be provided to the landlord's engineer for analysis and design of the HVAC system. The space will be balanced according to these calculations. All balancing will be conducted by a NEED certified balancing technician. Pre-construction balancing, engineering calculations and post-construction "as built" documentation/balancing reports will be provided to the tenant or the tenant's designee. The new space will conform to ASHRAE 55 "Thermal Environmental Conditions for Human Occupancy" and ASHRAE Standard 62.1 2010 "Ventilation for Acceptable Indoor Air Quality". All HVAC equipment shall be inspected by the landlord and replaced as necessary to ensure good operating condition and repair. The lessee accepts the existing HVAC system provided that it is in good working order, that it conforms to ASHRAE Standard 55, and 62.1 2010, and that a reasonable temperature is maintained in the R&D areas. In the event during the lease term the HVAC system does not meet the aforementioned prerequisites the landlord will provide a new system which will be at the Landlord's cost. Tissue culture spaces will have 6 to 15 air changes per hour. Spaces will be pressurized according to the attached drawing.

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The fume hood which is to remain in the laboratory is connected to a roof mounted exhaust fan and shall be air balanced to assure a minimum of 100 FPM airflow across the sash at an 18" sash opening. Fume hood will be equipped with a flow monitoring device which will indicate high/low limits. The remaining approximately 800cfm of exhaust shall be connected to ceiling grilles in the main lab and shall exhaust the lab. If more CFM is required to exhaust the lab according to the attached pressurization drawing it will be provided at the Landlord's expense. Exhaust must create negative pressure in the hallway connecting the tissue culture suites as well as create a negative with regard the office and main lab. The landlord's engineer will make the necessary calculations and resulting balancing recommendations to ensure that the space is pressurized accordingly. Make up air shall be provided from a 30 ton roof mounted make-up air unit and shall be fed to each of the three tissue culture suites (4118, 4119, and 4120) and the "radio-isotope room" (4116). Make up air shall also be provided to the main lab space (4114). All air supplied to each of the tissue culture suites shall be via 95% HEPA filters with 30% pre-filters on each of the ac units, MUA units, feeding, the spaces. Each tissue culture suite shall have a pressure relief exhaust grill installed on a wall adjacent to the main lab. The tenant will be responsible for maintaining and repairing all tenant specific supplemental mechanical equipment beyond the 6 month warranty period.

The Lab/Office will be pressurized as indicated in the attached drawing. Room for a 4' wide fume hood or ducted BSC will be left in the Radio-Isotope Room. The cost of operating and maintaining both exhaust fan and make up air unit shall be a tenant expense. All Tenant-specific mechanical equipment shall be put on a preventive maintenance agreement by the tenant and at Tenant's expense for the duration of the Lease.

Plumbing and Waste: The main cold water supply to the lab is located in the "Lab support" room which also contains a hot water heater, a water check meter and a backflow prevention device. All lab waste is contained in polyethylene piping and leads to an acid waste system consisting of a tank with limestone chips located in an accessible location on the third floor directly below the premises. Lab waste and holding within the "isotope room" will be connected to the existing laboratory waste system. Before the room is used for radio-isotopes this drain will be disconnected and is subject to future engineering and installation by the tenant. Future modifications to this drain is not subject to this scope of work. The laboratory wastewater system will be maintained by the tenant who shall also be responsible for required municipal discharge permits, sampling and testing.

Safety Showers will be added in rooms 4116 4119 and 4120 and will meet or exceed ANSI standards. Requirements for safety showers and eyewash stations throughout the lab space will be accessed by the landlord and will also be made to comply with ANSI standards.

Autoclave Room: The landlord has provided a room in which an autoclave can be installed. Actual equipment will be provided by the tenant. The existing laboratory sink with protected hot and cold water and eyewash shall remain. The seamless vinyl floor with integral base shall be repaired as required. Electrical hot water, drain will be installed to accommodate the future installation of a dishwasher.

Electrical: Power to various locations within the laboratory exists in the form of existing 110v and 208v outlets. 12 additional 120v/20A circuits, 7 additional 220v/20A circuits shall be added along with a new electrical panel will be connected to the existing roof mounted back up generator. In addition, one existing outlet shall be placed on backup power as well as the 2hp

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motor driving the roof mounted exhaust fan. Location of these outlets are as indicated on the accompanying plans. Tenant shall share the cost of maintenance and repairs of the back up generator with any other tenants who are also connected to it. All electrical power, natural gas and water to the tenant's premises and equipment will be separately metered and read monthly by the landlord for reimbursement by the tenant. New standby power outlets will be added as indicated on attached drawing. New normal power outlets will be added as indicated on attached drawing. All electrical circuits will be labeled via labels at the outlet, junction box, safety switch, or other corresponding electrical equipment and a corresponding label on the electrical panel indicating the appropriate circuit breaker.

CO2 Piping: Piping for tenants CO2 shall be installed as indicated in the accompanying drawings. These shall consist of 1/2" copper piping from tenant's existing "tank farm", 20 CO2 drops to 1/2" ball valve to 1/4" hose barbs as indicated, 16 brass 1/2" ball valve shut-offs and 5 above ceiling capped 1/2" copper lines for future use. Gas regulators, if required, shall be provided by the tenant.

Vacuum lines will be installed as shown on the attached drawing. Vacuum drops will consist of 1/2" type L copper piping from the house vacuum system to drops as indicated on the attached drawing. Each drop will consist of 1/2" copper pipe to 1/2" ball valve to 1/4" hose barb.

BluebirdBio or their designee will have access to the space during renovation to inspect the progress and that the work being done conforms to this scope.

Fire Protection: Fire protection will be added as needed and required by Massachusetts Building Code, NFPA and local Fire Code.

Work included in this scope will comply with both the Massachusetts Building Code and NFPA.

Laboratory Safety Showers/Eyewash stations will be installed as required per ANSI standards.

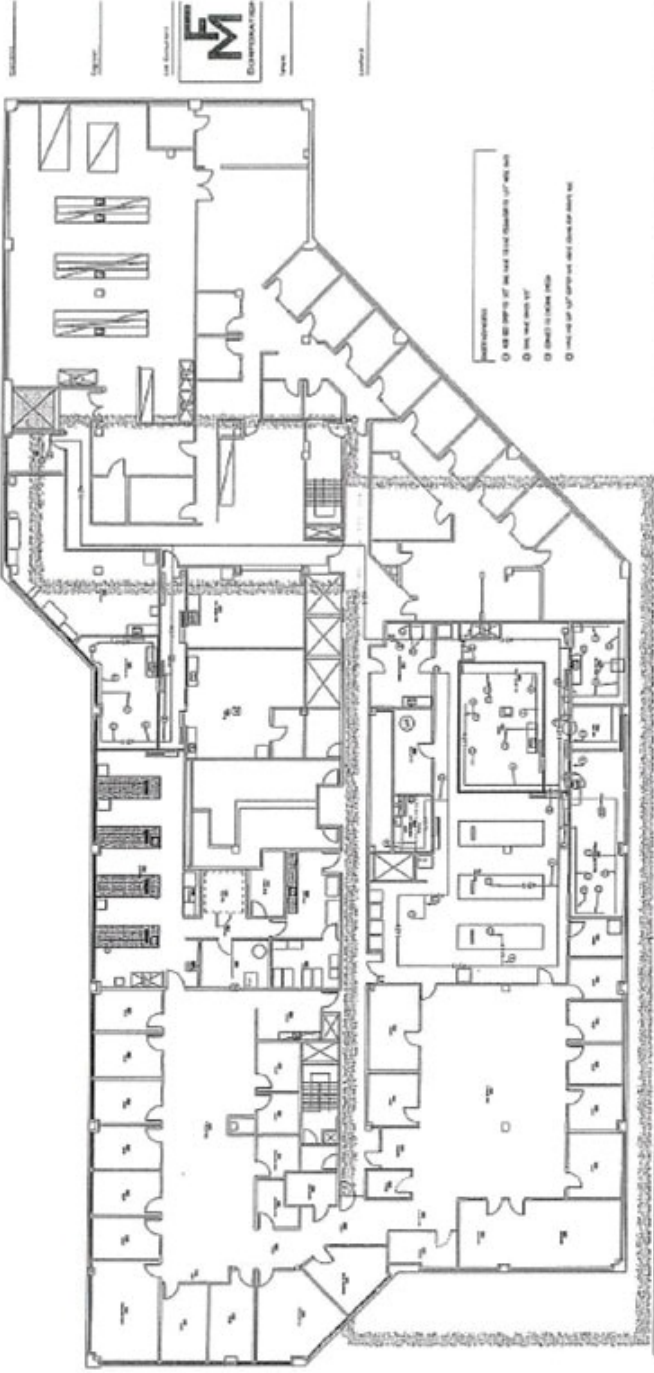
Work will be conducted as indicated by attached drawing keynotes.

Scope of Work to include all Keynotes in attached drawings with the exception of MEPs in the Radio Isotope Room.

Drawings included in this scope of work are diagrammatic in nature. All pre-existing construction and new construction will be reviewed by the landlord's architect and engineer to insure indicated changes are made in accordance with Massachusetts Building Code and the NFPA.

BluebirdBio Final Scope of Work

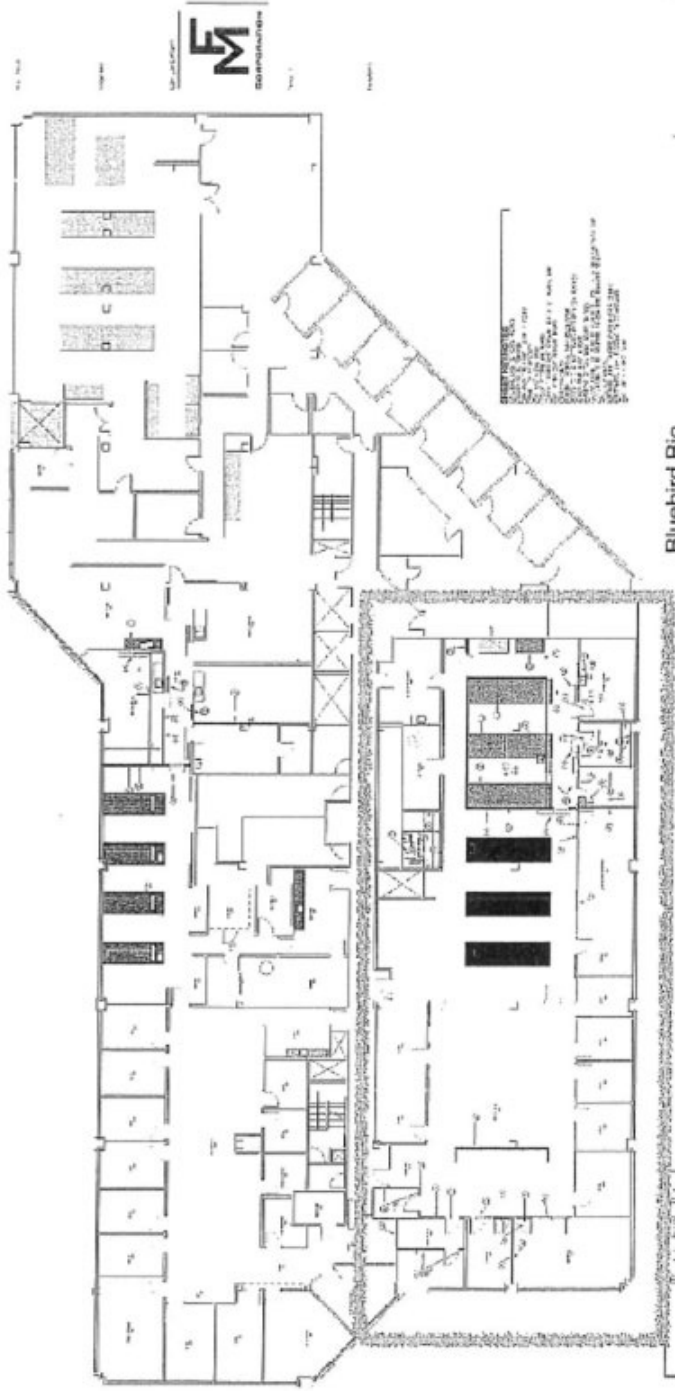
8/13/2012



P1.0

**Bluebird Bio  
840 Memorial Drive  
Floor 4  
Additional Premises CO2 Piping**

Project Name	Bluebird Bio
Project Address	840 Memorial Drive
Project City	San Francisco, CA
Project State	CA
Project Zip	94103

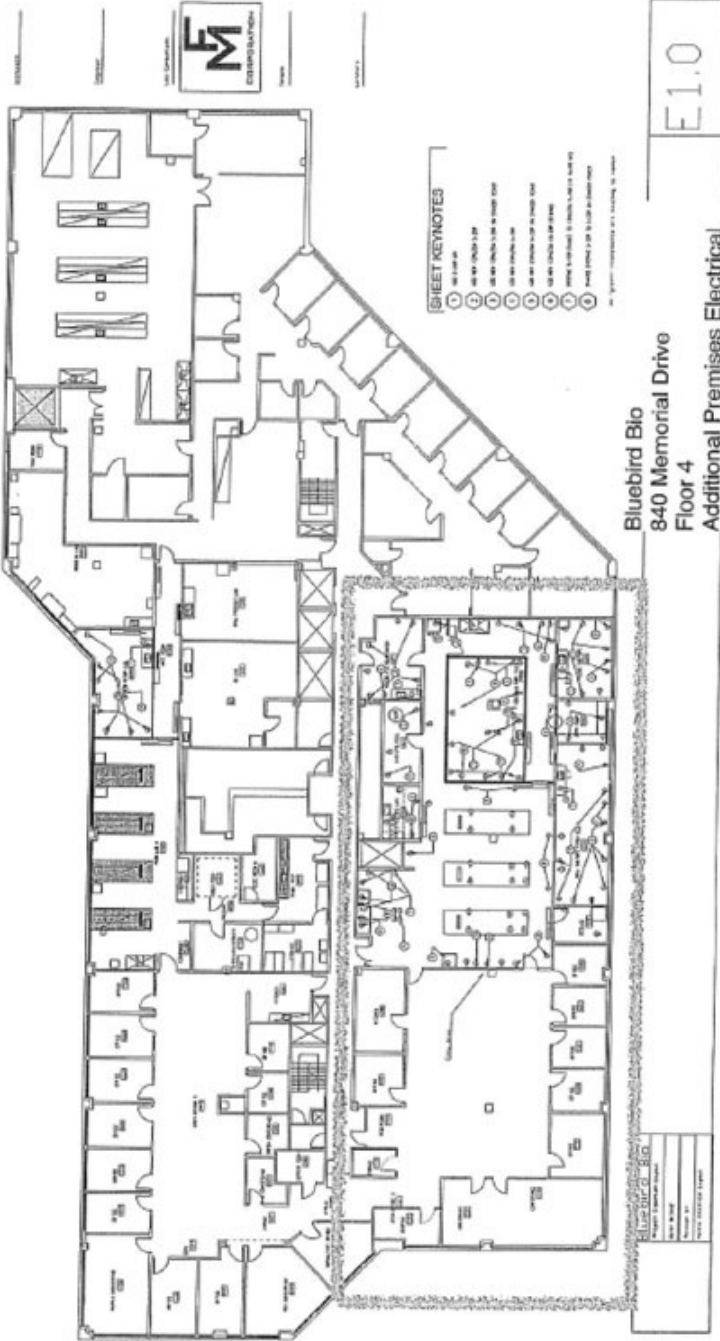


Bluebird Bio  
 840 Memorial Drive  
 Floor 4  
 Additional Premises Plan Revision

A 0

PROJECT NO. 15-0001  
 DATE 01/11/2016  
 DRAWN BY: [Name]  
 CHECKED BY: [Name]

NOT TO SCALE  
 SEE ARCHITECTURAL RECORDS FOR  
 ALL DIMENSIONS AND NOTES  
 ALL WORK SHALL BE IN ACCORDANCE  
 WITH THE LATEST EDITIONS OF THE  
 INTERNATIONAL BUILDING CODE (IBC)  
 AND ALL APPLICABLE LOCAL CODES  
 AND ORDINANCES.



- SHEET KEYNOTES**
- 1. SEE SHEET E1.1
  - 2. SEE SHEET E1.2
  - 3. SEE SHEET E1.3
  - 4. SEE SHEET E1.4
  - 5. SEE SHEET E1.5
  - 6. SEE SHEET E1.6
  - 7. SEE SHEET E1.7
  - 8. SEE SHEET E1.8
  - 9. SEE SHEET E1.9
  - 10. SEE SHEET E1.10

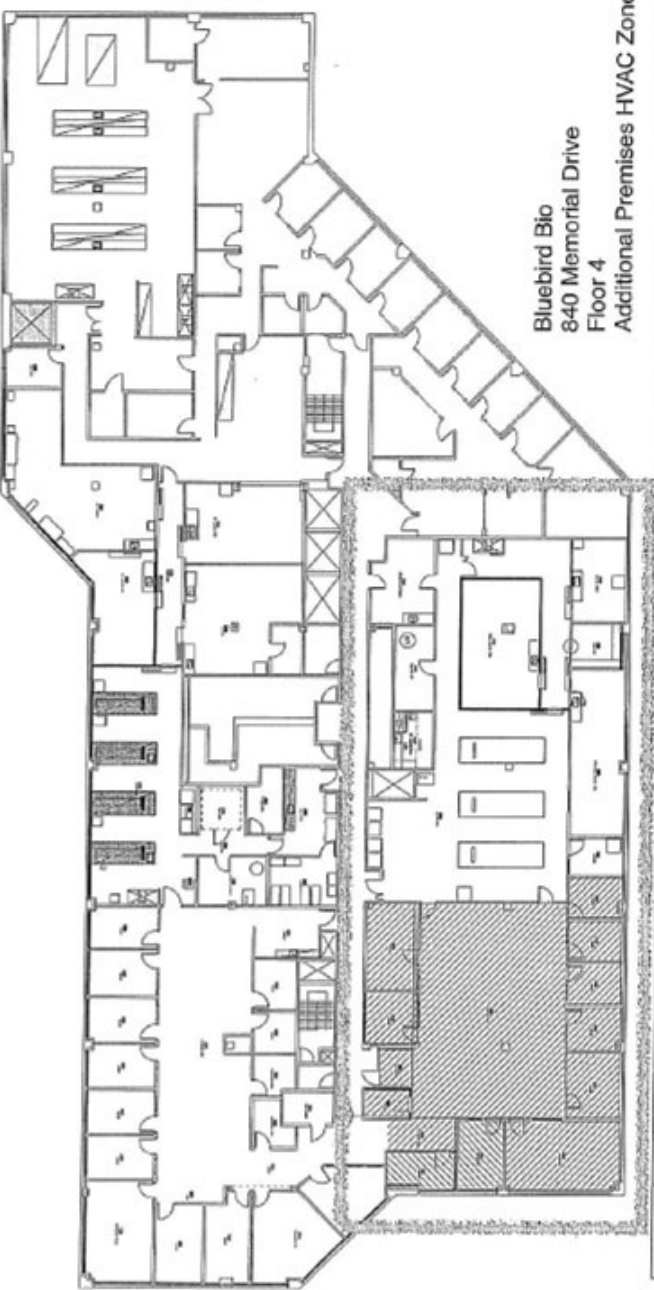
Bluebird Bio  
 840 Memorial Drive  
 Floor 4  
 Additional Premises Electrical

E1.0

Project Name	Bluebird Bio
Client	Bluebird Bio
Architect	Bluebird Bio
Electrical Engineer	Bluebird Bio
Date	10/10/2023
Sheet No.	E1.0
Scale	AS SHOWN



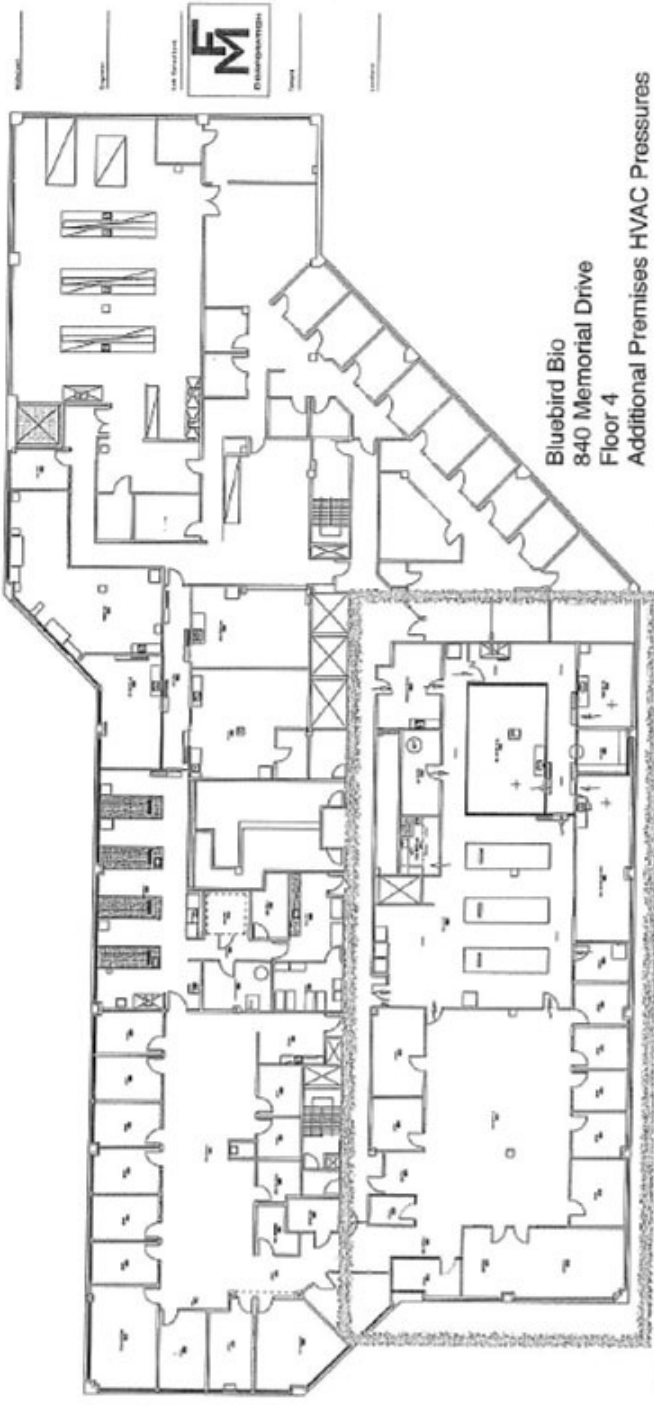




Bluebird Bio  
840 Memorial Drive  
Floor 4  
Additional Premises HVAC Zones

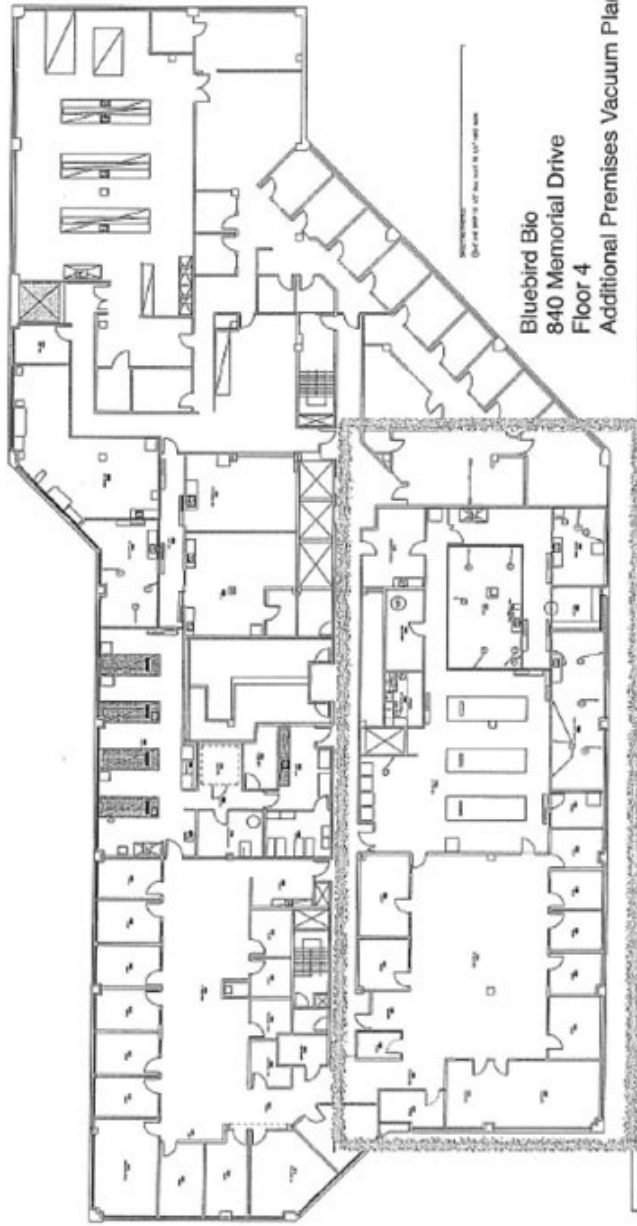
Bluebird Bio
840 Memorial Drive
Floor 4
Additional Premises HVAC Zones

H1.0



Bluebird Bio
Project Name
Date
Scale
Sheet No.

H1.1

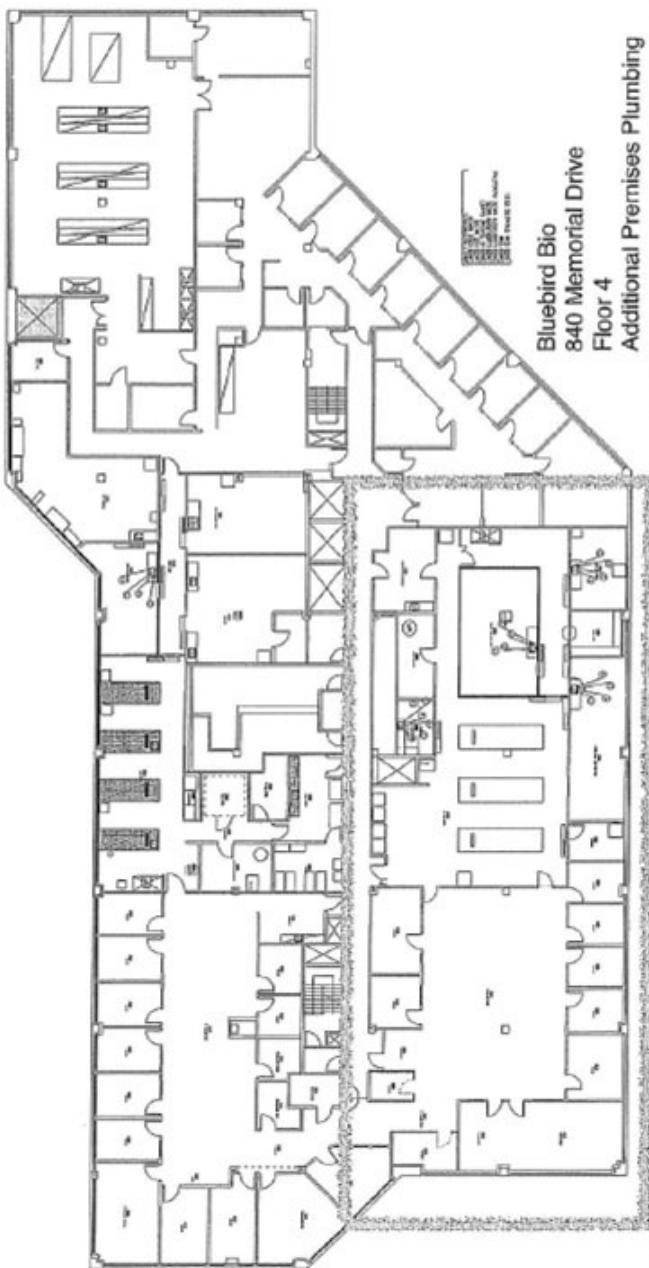


NOT TO SCALE  
DATE: 08/20/18

**Bluebird Bio  
840 Memorial Drive  
Floor 4  
Additional Premises Vacuum Plan**

Project Name	Bluebird Bio
Project No.	18-001
Revision No.	01
Revision Description	Final

P1.1

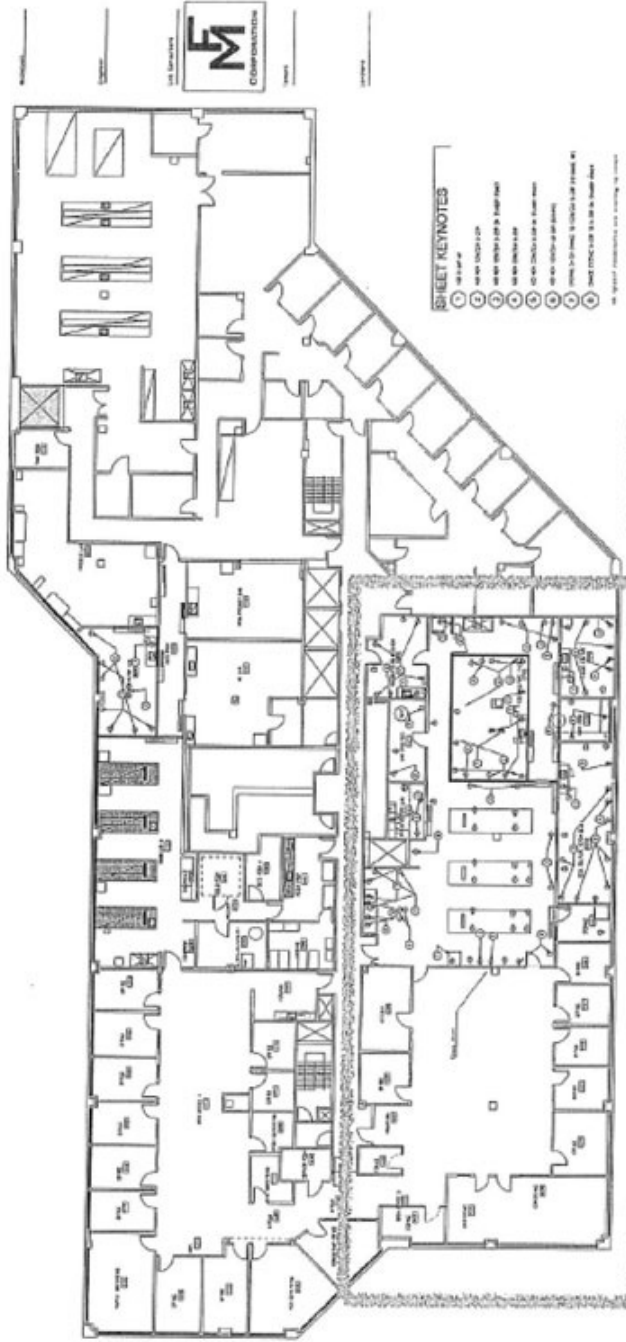


Bluebird Bio  
840 Memorial Drive  
Floor 4

**Bluebird Bio  
840 Memorial Drive  
Floor 4  
Additional Premises Plumbing**

Bluebird Bio  
Project Location  
Date: 03/20  
Revision:  
Sheet: P1.3

P1.3



- SHEET KEYNOTES**
- 1. SEE PLAN
  - 2. SEE SCHEDULE
  - 3. SEE SCHEDULE
  - 4. SEE SCHEDULE
  - 5. SEE SCHEDULE
  - 6. SEE SCHEDULE
  - 7. SEE SCHEDULE
  - 8. SEE SCHEDULE

Bluebird Bio  
 840 Memorial Drive  
 Floor 4  
 Additional Premises Electrical

Project Name:	Bluebird Bio
Sheet No.:	E1.0
Revision:	
Total Sheet Count:	

E1.0

**See Attached**

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**EXHIBIT B—Operating Expenses 2011**

840 Memorial Drive—Riverside Technology Center

<u>DESCRIPTION</u>	<u>Total</u>	<u>PSF</u>
HEAT	\$ 36,863	\$0.28
BUILDING ELECTRIC	\$152,969	\$1.18
WATER & SEWER	\$ 30,053	\$0.23
ELEVATOR MAINTENANCE	\$ 19,295	\$0.15
PARKING/CAFÉ EXPENSE	\$ 26,525	\$0.20
RUBBISH REMOVAL	\$ 17,104	\$0.13
INSURANCE	\$ 30,234	\$0.23
GROUNDS CARE	\$ 19,459	\$0.15
LEGAL/ACCT/ADMIN	\$ 18,675	\$0.14
JANITORIAL SERVICES	\$ 47,707	\$0.37
GENERAL MAINTENANCE	\$ 63,172	\$ 0.49
HVAC MAINTENANCE	\$ 47,827	\$0.37
LIFE SAFETY SYSTEMS	\$ 29,593	\$0.23
MANAGEMENT*	\$280,302	\$2.16
<b>Real Estate Taxes (FY 2011)</b>	<b>\$ 659,946</b>	<b>\$5.10</b>

\* Based upon 5% of income; but not less than this amount.

Tenant's Applicable Percentage is as follows:

As to the Leased Premises: 13.69%.

Existing Premises: 7.40%

Expansion Premises: 6.29%

CERTAIN CONFIDENTIAL PORTIONS OF THIS EXHIBIT WERE OMITTED AND REPLACED WITH “[\*\*\*]”. A COMPLETE VERSION OF THIS EXHIBIT HAS BEEN FILED SEPARATELY WITH THE SECRETARY OF THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO AN APPLICATION REQUESTING CONFIDENTIAL TREATMENT PURSUANT TO RULE 406 PROMULGATED UNDER THE SECURITIES ACT OF 1933, AS AMENDED.

MASSACHUSETTS INSTITUTE OF TECHNOLOGY

and

**INNOGENE PHARMACEUTICALS, INC.**

PATENT LICENSE AGREEMENT

(EXCLUSIVE)

-1-



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MASSACHUSETTS INSTITUTE OF TECHNOLOGY

and

INNOGENE PHARMACEUTICALS, INC.

PATENT LICENSE AGREEMENT

This Agreement is made and entered into this 11th day of December, 1996 (the "EFFECTIVE DATE") by and between the MASSACHUSETTS INSTITUTE OF TECHNOLOGY, a corporation duly organized and existing under the laws of the Commonwealth of Massachusetts and having its principal office at 77 Massachusetts Avenue, Cambridge, Massachusetts 02139, U.S.A. (hereinafter referred to as "M.I.T."), and Innogene Pharmaceuticals, Inc. a corporation duly organized under the laws of Delaware and having its principal office at 41 Fresh Pond Lane, Cambridge, MA 02138 (hereinafter referred to as "LICENSEE").

WITNESSETH

WHEREAS, M.I.T. is the owner of certain PATENT RIGHTS (as later defined herein) relating to [\*\*\*].

WHEREAS M.I.T. is the co-owner with Albert Einstein College of Medicine ("AECOM") of the PATENT RIGHTS of [\*\*\*];

WHEREAS, M.I.T. and AECOM desire to have the PATENT RIGHTS developed and commercialized to benefit the public and is willing to grant a license thereunder,

WHEREAS, LICENSEE has represented to M.I.T., to induce M.I.T. to enter into this Agreement, that LICENSEE is experienced in the development, production, manufacture, marketing and sale of products similar to the LICENSED PRODUCT(s) (as later defined herein) and/or the use of the LICENSED PROCESS(es) (as later defined herein) and that it shall commit itself to a thorough, vigorous and diligent program of exploiting the PATENT RIGHTS so that public utilization shall result therefrom; and

WHEREAS, LICENSEE desires to obtain a license under the PATENT RIGHTS upon the terms and conditions hereinafter set forth.

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NOW, THEREFORE, in consideration of the premises and the mutual covenants contained herein, the parties hereto agree as follows:

1 - DEFINITIONS

For the purposes of this Agreement, the following words and phrases shall have the following meanings:

1.1 “LICENSEE” shall include a related company of Innogene Pharmaceuticals, Inc. the voting stock of which is directly or indirectly at least Fifty Percent (50%) owned or controlled by Innogene Pharmaceuticals, Inc. an organization which directly or indirectly controls more than Fifty Percent (50%) of the voting stock of Innogene Pharmaceuticals, Inc. and an organization, the majority ownership of which is directly or indirectly common to the ownership of Innogene Pharmaceuticals, Inc.

1.2 “PATENT RIGHTS” shall mean all of the following M.I.T. intellectual property:

- a. the United States patents listed in Appendix A;
- b. the United States patent applications listed in Appendix A, and divisionals, continuations and claims of continuation-in-part applications which shall be directed to subject matter specifically described in such patent applications, and the resulting patents;
- c. any patents resulting from reissues or reexaminations of the United States patents described in a. and b. above;
- d. the Foreign patents listed in Appendix A;
- e. the Foreign patent applications listed in Appendix A, and divisionals, continuations and claims of continuation-in-part applications which shall be directed to subject matter specifically described in such Foreign patent applications, and the resulting patents;
- f. Foreign patent applications filed after the EFFECTIVE DATE and divisionals, continuations and claims of continuation-in-part applications which shall be directed to subject matter specifically described in such patent applications, and the resulting patents; and
- g. any Foreign patents, resulting from equivalent Foreign procedures to United States reissues and reexaminations, of the Foreign patents described in d., e. and f. above.
- h. any U.S. and foreign patent applications and the resulting patents and any reissues and reexaminations which may be filed on the technology of any of the M.I.T. Cases in Appendix A, as the technology existed on the Effective Date.

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1.3 A “LICENSED PRODUCT” shall mean any product or part thereof which:

- a. is covered in whole or in part by an issued, unexpired claim or a pending claim contained in the PATENT RIGHTS in the country in which any such product or part thereof is made, used or sold; or
- b. is manufactured by using a process or is employed to practice a process which is covered in whole or in part by an issued, unexpired claim or a pending claim contained in the PATENT RIGHTS in the country in which any LICENSED PROCESS is used or in which such product or part thereof is used or sold.

If a claim has been abandoned or judged invalid or unenforceable by a court of competent jurisdiction, or an administrative agency, from which no appeal can be or is taken in any country, then any product or process falling only under that claim shall not be considered to be a LICENSED PRODUCT or LICENSED PROCESS in that country for the purposes of this Agreement.

1.4 A “LICENSED PROCESS” shall mean any process which is covered in whole or in part by an issued, unexpired claim or a pending claim contained in the PATENT RIGHTS.

1.5 “NET SALES” shall mean LICENSEE’S billings for LICENSED PRODUCTS and LICENSED- PROCESSES less the sum of the following:

- a. discounts allowed in amounts customary in the trade for quantity purchases, cash payments, prompt payments, wholesalers and distributors;
- b. sales, tariff duties and/or use taxes directly imposed and with reference to particular sales;
- c. outbound transportation prepaid or allowed; and
- d. amounts allowed or credited on returns.

No deductions shall be made for commissions paid to individuals whether they be with independent sales agencies or regularly employed by LICENSEE and on its payroll, or for cost of collections. NET SALES shall occur when a LICENSED PRODUCT or LICENSED

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PROCESS shall be invoiced. If a LICENSED PRODUCT or LICENSED PROCESS shall be distributed or invoiced for a discounted price substantially lower than customary in the trade or distributed at no cost to affiliates or otherwise, NET SALES shall be based on the customary amount billed for such LICENSED PRODUCTS or LICENSED PROCESSES.

1.6 “TERRITORY” shall mean [\*\*\*].

1.7 “FIELD OF USE” shall mean all.

1.8 “TANGIBLE PROPERTY” shall mean the biological materials listed in Appendix B and any derivatives or progeny thereof.

## 2 - GRANT

2.1 M.I.T. hereby grants to LICENSEE the right and license in the TERRITORY for the FIELD OF USE to practice under the PATENT RIGHTS and, to the extent not prohibited by other patents, to make, have made, use, lease, sell and import LICENSED PRODUCTS and to practice the LICENSED PROCESSES, until the expiration of the last to expire of the PATENT RIGHTS, unless this Agreement shall be sooner terminated according to the terms hereof.

2.2 LICENSEE agrees that LICENSED PRODUCTS leased or sold in the United States shall be manufactured substantially in the United States.

2.3 In order to establish a period of exclusivity for LICENSEE, M.I.T. hereby agrees that it shall not grant any other license to make, have made, use, lease, sell and import LICENSED PRODUCTS or to utilize LICENSED PROCESSES subject to the royalty-free, nonexclusive license rights of the United States Government per FAR 52.227-11, in the TERRITORY for the FIELD OF USE.

2.4 M.I.T. reserves the right to practice under the PATENT RIGHTS for noncommercial research purposes. AECOM reserves the right to practice under the PATENT RIGHTS of M.I.T. Case No. 7410 for noncommercial research purposes.

2.5 M.I.T. hereby grants to LICENSEE, during the term of this Agreement, an exclusive, royalty-free commercial license to use, reproduce, modify, make derivatives of, and transfer the TANGIBLE PROPERTY in conjunction with the LICENSED PRODUCTS and LICENSED PROCESSES or as otherwise necessary or useful for the exercise of the PATENT RIGHTS licensed hereunder. LICENSEE shall have the right to sublicense the TANGIBLE

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PROPERTY within LICENSEE’S reasonable discretion in order to exercise the PATENT RIGHTS granted under this Agreement. M.I.T. reserves the right to use, reproduce, modify, and make derivatives of the TANGIBLE PROPERTY for non-commercial research purposes. M.I.T. shall make reasonable efforts to transfer to LICENSEE functional samples of the TANGIBLE PROPERTY in their original form as listed in Appendix B, but shall have no obligation to replace such samples, and makes no warranty of their fitness for use.

2.6 M.I.T. reserves the right to distribute the TANGIBLE PROPERTY for research purposes only, to third parties including commercial entities.

2.7 M.I.T. also grants to LICENSEE a nonexclusive, non-royalty-bearing license to non-tangible know-how associated with the technology of any of the Cases of Appendix A, and further agrees that this know-how may be transferred by LICENSEE to third parties.

2.8 LICENSEE shall have the right to enter into sublicensing agreements for the rights, privileges and licenses granted hereunder. Upon any termination of this Agreement, sublicensees’ rights shall be subject to Paragraph 13.6 hereof.

2.9 LICENSEE agrees to incorporate terms and conditions substantively similar to Articles 2, 5.1, 7.1, 7.2, 7.3, 7.5, 7.6, 8, 9, 10, 12 and 15 of this Agreement into its sublicense agreements, that are sufficient to enable LICENSEE to comply with this Agreement.

2.10 LICENSEE agrees to forward to M.I.T. a copy of any and all sublicense agreements promptly upon execution by the parties.

2.11 LICENSEE shall not receive from sublicensees anything of value in lieu of cash payments in consideration for any sublicense under this Agreement, without the express prior written permission of M.I.T.

2.12 Nothing in this Agreement shall be construed to confer any rights upon LICENSEE by implication, estoppel or otherwise as to any technology or PATENT RIGHTS of M.I.T., or any other entity other than the PATENT RIGHTS and TANGIBLE PROPERTY.

### 3 - DILIGENCE

3.1 LICENSEE shall use diligent efforts to bring LICENSED PRODUCTS to market through a thorough, vigorous program for exploitation of the PATENT RIGHTS and to continue active, diligent development and marketing efforts for LICENSED PRODUCTS throughout the life of this Agreement.

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3.2 LICENSEE shall raise a cumulative total of investment capital of at least:

- a. [\*\*\*]; and
- b. [\*\*\*]; and
- c. [\*\*\*].

3.3 [\*\*\*]

- a. [\*\*\*];
- b. [\*\*\*];
- c. [\*\*\*]; and
- d. [\*\*\*].

3.4 LICENSEE'S failure to perform in accordance with either Paragraph 3.1 or 3.2 above shall be grounds for M.I.T. to terminate this Agreement pursuant to Paragraph 13.3 hereof, provided, however, if LICENSEE has expended at least [\*\*\*], and is otherwise in compliance with the terms and conditions of this Agreement, the license under this Agreement will become nonexclusive, without the right to sublicense, except by approval of M.I.T., such approval not to be unreasonably withheld.

#### 4 - ROYALTIES

4.1 For the rights, privileges and license granted hereunder, LICENSEE shall pay royalties to M.I.T. in the manner hereinafter provided to the end of the term of the PATENT RIGHTS or until this Agreement shall be terminated:

- a. License Issue Fee of [\*\*\*], which said License Issue Fee shall be deemed earned and due in three parts:
  - (i) [\*\*\*] due upon the signing of the Agreement; and
  - (ii) [\*\*\*] due upon the raising of Two Million Dollars (\$2,000,000) in investment capital by LICENSEE.
  - (iii) [\*\*\*] upon the filing by LICENSEE of a New Drug Application for the first LICENSED PRODUCT.

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- b. License Maintenance Fees of [\*\*\*] per year payable on January 1, 1999 and on January 1 of each year thereafter, provided, however, License Maintenance Fees may be credited to Running Royalties subsequently due on NET SALES for each said year, if any. License Maintenance Fees paid in excess of Running Royalties shall not be creditable to Running Royalties for future years.
- c. Running Royalties in an amount equal to [\*\*\*] of NET SALES of the LICENSED PRODUCTS and LICENSED PROCESSES used, leased or sold by and/or for LICENSEE.
- d. The following proportion of payments, including, but not limited to, sublicense issue fees and royalties, received from sublicensees in consideration for the LICENSED PRODUCTS and LICENSED PROCESSES, but excluding payments made for research funding:
  - (i) [\*\*\*]; or
  - (ii) [\*\*\*].

Provided, however, that in no case shall the payments for each sublicense in any given year be less than [\*\*\*] of the net sales of the sublicensee in that year, determined on the same basis on which such net sales are reported to LICENSEE for purposes of determining royalties payable to LICENSEE under its sublicense.

Net sales shall be determined on substantially the same terms as “NET SALES” are defined herein, with only such changes as M.I.T. may approve, which approval shall not be unreasonably withheld.

4.2 If LICENSEE must pay royalties to a third party for patents necessary to the reduction or sale LICENSED PRODUCTS or LICENSED PROCESSES, LICENSEE may credit [\*\*\*] of the royalties paid to the third party against the Running Royalties otherwise due under P. 4.1 (c) above, provided that in no event shall the amount paid to M.I.T. for that LICENSED PRODUCT or LICENSED PROCESS be less than [\*\*\*] of the NET SALES of that LICENSED PRODUCT or LICENSED PROCESS.

4.3 No royalties shall be due on any LICENSED PRODUCT or LICENSED PROCESS which falls only under a pending patent which has not issued five years following its



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priority date. If a patent subsequently issues, royalties on the LICENSED PRODUCT or LICENSED PROCESS shall resume from the issue date of such patent. If a patent subsequently issues, royalties on the LICENSED PRODUCT or LICENSED PROCESS shall resume from the issue date of such patent.

4.4 If LICENSEE sells a LICENSED PRODUCT or LICENSED PROCESS which delivers several active genes (totaling “B”), the delivery of only “A” of which fall under the PATENT RIGHTS, then the NET SALES for the purposes of the Running Royalties of P.4.1(c) and 4.1(d) above shall be [\*\*\*].

4.5 All payments due hereunder shall be paid in full, without deduction of taxes or other fees which may be imposed by any government, except as otherwise provided in Paragraph 1.5(b).

4.6 If LICENSEE is prevented from [\*\*\*], then LICENSEE shall not be required to pay to M.I.T. [\*\*\*].

4.7 No multiple royalties shall be payable because any LICENSED PRODUCT, its manufacture, use, lease or sale are or shall be covered by more than one PATENT RIGHTS patent application or PATENT RIGHTS patent licensed under this Agreement.

4.8 Royalty payments shall be paid in United States dollars in Cambridge, Massachusetts, or at such other place as M.I.T. may reasonably designate consistent with the laws and regulations controlling in any foreign country. If any currency conversion shall be required in connection with the payment of royalties hereunder, such conversion shall be made by using the exchange rate prevailing at the Chase Manhattan Bank (N.A.) on the last business day of the calendar quarterly reporting period to which such royalty payments relate.

#### 5 - REPORTS AND RECORDS

5.1 LICENSEE shall keep full, true and accurate books of account containing all particulars that may be necessary for the purpose of showing the amounts payable to M.I.T. hereunder. Said books of account shall be kept at LICENSEE’S principal place of business or the principal place of business of the appropriate division of LICENSEE to which this Agreement relates. Said books and the supporting data shall be open at all reasonable times[\*\*\*] for five (5) years following the end of the calendar year to which they pertain, to the inspection

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of a M.I.T. agent, who shall be an accounting firm of national standing, for the purpose of verifying LICENSEE’S royalty statement or compliance in other respects with this Agreement. Should such inspection lead to the discovery of a greater than [\*\*\*] discrepancy in reporting to M.I.T.’s detriment, LICENSEE agrees to pay the full cost of such inspection.

5.2 LICENSEE shall deliver to M.I.T. true and accurate reports, giving such particulars of the business conducted by LICENSEE and its sublicensees under this Agreement as shall be pertinent to diligence under Article 3 and royalty accounting hereunder:

- a. before the first commercial sale of a LICENSED PRODUCT or LICENSED PROCESS, annually, on January 31 of each year; and
- b. after the first commercial sale of a LICENSED PRODUCT or LICENSED PROCESS, quarterly, within sixty (60) days after March 31, June 30, September 30 and December 31, of each year.

These reports shall include at least the following:

- a. [\*\*\*];
- b. [\*\*\*];
- c. [\*\*\*];
- d. [\*\*\*];
- e. [\*\*\*];
- f. [\*\*\*]; and
- g. [\*\*\*].

5.3 With each such report submitted, LICENSEE shall pay to M.I.T. the royalties due and payable under this Agreement. If no royalties shall be due, LICENSEE shall so report.

5.4 On or before the ninetieth (90th) day following the close of LICENSEE’S fiscal year, LICENSEE shall provide M.I.T. with LICENSEE’S certified financial statements for the preceding fiscal year including, at a minimum, a balance sheet and an income statement.

5.5 The amounts due under Articles 4 and 6 shall, if overdue, bear interest until payment at a per annum rate [\*\*\*]. The payment of such interest shall not foreclose M.I.T. from exercising any other rights it may have as a consequence of the lateness of any payment.

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#### 6 - PATENT PROSECUTION

6.1 Upon the Effective Date, LICENSEE shall assume responsibility for the filing, prosecution and maintenance of the PATENT RIGHTS in the U.S. and in foreign countries elected by LICENSEE, using a patent attorney of LICENSEE'S choice. Such prosecution shall be in M.I.T.'s name. LICENSEE shall not abandon any substantive claim or fail to make a payment with respect to any of the PATENT RIGHTS filed by M.I.T. prior to the Effective Date in the U.S., Canada, the countries of the European Patent Office, or Japan unless LICENSEE has notified M.I.T. in sufficient time for M.I.T. to assume such prosecution or make payment at its own expense. M.I.T. shall be copied on all correspondence with government patent offices relating to prosecution and maintenance of the PATENT RIGHTS, M.I.T. shall have reasonable opportunities to advise LICENSEE and shall cooperate with LICENSEE in such filing, prosecution and maintenance.

6.2 Payment of all fees and costs relating to the filing, prosecution and maintenance of the PATENT RIGHTS shall be the responsibility of [\*\*\*].

#### 7 - INFRINGEMENT

7.1 LICENSEE and M.I.T. shall each inform the other promptly in writing of any alleged infringement of the PATENT RIGHTS by any third party that comes to the notifying party's attention and of any available evidence thereof of which the notifying party is aware.

7.2 [\*\*\*]

7.3 [\*\*\*]

7.4 [\*\*\*]

7.5 [\*\*\*]

7.6 [\*\*\*]

7.7 [\*\*\*]

#### 8 - PRODUCT LIABILITY

8.1 LICENSEE shall at all times during the term of this Agreement and thereafter, indemnify, defend and hold M.I.T, AECOM, their trustees, directors, officers, employees and affiliates, harmless against all claims, proceedings, demands and liabilities of any kind

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whatsoever, including legal expenses and reasonable attorneys’ fees, arising out of the death of or injury to any person or persons or out of any damage to property, resulting from the production, manufacture, sale, use, lease, consumption or advertisement of the LICENSED PRODUCT(s) and/or LICENSED PROCESS(es) and/or TANGIBLE PROPERTY or arising from any obligation of LICENSEE hereunder.

8.2 LICENSEE shall obtain and carry in full force and effect commercial, general liability insurance, including product liability and errors and omissions insurance, which shall protect LICENSEE, M.I.T. and AECOM with respect to events covered by Paragraph 8.1 above. Such insurance shall be written by a reputable insurance company authorized to do business in the Commonwealth of Massachusetts, shall list M.I.T. as an additional named insured thereunder, shall be endorsed to include product liability coverage and shall require thirty (30) days written notice to be given to M.I.T. prior to any cancellation or material change thereof. The limits of such insurance shall not be less than [\*\*\*]. LICENSEE shall provide M.I.T. with Certificates of Insurance evidencing the same.

8.3 EXCEPT AS OTHERWISE EXPRESSLY SET FORTH IN THIS AGREEMENT, M.I.T., ALBERT EINSTEIN COLLEGE OF MEDICINE (AECOM), THEIR TRUSTEES, DIRECTORS, OFFICERS, EMPLOYEES, AND AFFILIATES MAKE NO REPRESENTATIONS AND EXTEND NO WARRANTIES OF ANY KIND, EITHER EXPRESS OR IMPLIED, INCLUDING BUT NOT LIMITED TO WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, VALIDITY OF PATENT RIGHTS CLAIMS, ISSUED OR PENDING, AND THE ABSENCE OF LATENT OR OTHER DEFECTS, WHETHER OR NOT DISCOVERABLE. NOTHING IN THIS AGREEMENT SHALL BE CONSTRUED AS A REPRESENTATION MADE OR WARRANTY GIVEN BY M.I.T. OR AECOM THAT THE PRACTICE BY LICENSEE OF THE LICENSE GRANTED HEREUNDER SHALL NOT INFRINGE THE PATENT RIGHTS OF ANY THIRD PARTY. IN NO EVENT SHALL M.I.T. OR AECOM, THEIR TRUSTEES, DIRECTORS, OFFICERS, EMPLOYEES AND AFFILIATES BE LIABLE FOR INCIDENTAL OR CONSEQUENTIAL DAMAGES OF ANY KIND, INCLUDING ECONOMIC DAMAGE OR INJURY TO PROPERTY AND LOST PROFITS, REGARDLESS OF WHETHER M.I.T. OR AECOM SHALL BE ADVISED, SHALL HAVE OTHER REASON TO KNOW, OR IN FACT SHALL KNOW OF THE POSSIBILITY OF THE FOREGOING.

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#### 9 - EXPORT CONTROLS

LICENSEE acknowledges that it is subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes and other commodities (including the Arms Export Control Act, as amended and the United States Department of Commerce Export Administration Regulations). The transfer of such items may require a license from the cognizant agency of the United States Government and/or written assurances by LICENSEE that LICENSEE shall not export data or commodities to certain foreign countries without prior approval of such agency. M.I.T. neither represents that a license shall not be required nor that, if required, it shall be issued.

#### 10 - NON-USE OF NAMES

LICENSEE shall not use the names or trademarks of the Massachusetts Institute of Technology or Lincoln Laboratory, nor Albert Einstein College of Medicine (AECOM), nor any adaptation thereof, nor the names of any of their employees, in any advertising, promotional or sales literature without prior written consent obtained from M.I.T., AECOM, or said employee, in each case, except that LICENSEE may state that it is licensed by M.I.T. under one or more of the patents and/or applications comprising the PATENT RIGHTS.

#### 11 - ASSIGNMENT

This Agreement is not assignable except to successors of substantially all of LICENSEE’S business related to the subject matter of this Agreement, by merger or other operation of law, and in the case a merger in which the stockholders of LICENSEE will own less than 50% of the outstanding voting power of the resulting corporation, with the consent of M.I.T., such consent not to be unreasonably withheld.

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## 12 - DISPUTE RESOLUTION

12.1 Except for the right of either party to apply to a court of competent jurisdiction for a temporary restraining order, a preliminary injunction, or other equitable relief to preserve the status quo or prevent irreparable harm, any and all claims, disputes or controversies arising under, out of, or in connection with the Agreement, including any dispute relating to patent validity or infringement, which the parties shall be unable to resolve within [\*\*\*] shall be mediated in good faith. The party raising such dispute shall promptly advise the other party of such claim, dispute or controversy in a writing which describes in reasonable detail the nature of such dispute. By not later than [\*\*\*] after the recipient has received such notice of dispute, each party shall have selected for itself a representative who shall have the authority to bind such party, and shall additionally have advised the other party in writing of the name and title of such representative. By not later than [\*\*\*] after the date of such notice of dispute, the party against whom the dispute shall be raised shall select a mediation firm in the Boston area and such representatives shall schedule a date with such firm for a mediation hearing. The parties shall enter into good faith mediation and shall share the costs equally. If the representatives of the parties have not been able to resolve the dispute within [\*\*\*] after such mediation hearing, then any and all claims, disputes or controversies arising under, out of, or in connection with this Agreement, including any dispute relating to patent validity or infringement, shall be resolved by final and binding arbitration in Boston, Massachusetts under the rules of the American Arbitration Association, or the Patent Arbitration Rules if applicable, then obtaining. The arbitrators shall have no power to add to, subtract from or modify any of the terms or conditions of this Agreement, nor to award punitive damages. Any award rendered in such arbitration may be enforced by either party in either the courts of the Commonwealth of Massachusetts or in the United States District Court for the District of Massachusetts, to whose jurisdiction for such purposes M.I.T. and LICENSEE each hereby irrevocably consents and submits.

12.2 Notwithstanding the foregoing, nothing in this Article shall be construed to waive any rights or timely performance of any obligations existing under this Agreement.

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### 13 - TERMINATION

13.1 If LICENSEE shall cease to carry on its business, this Agreement shall terminate upon notice by M.I.T.

13.2 Should LICENSEE fail to make any payment whatsoever due and payable to M.I.T. hereunder, M.I.T. shall have the right to terminate this Agreement effective on thirty (30) days' notice, unless LICENSEE shall make all such payments to M.I.T. within said thirty (30) day period. Upon the expiration of the thirty (30) day period, if LICENSEE shall not have made all such payments to M.I.T., the rights, privileges and license granted hereunder shall automatically terminate.

13.3 Upon any material breach or default of this Agreement by LICENSEE (including, but not limited to, breach or default under Paragraph 3.3), other than those occurrences set out in Paragraphs 13.1 and 13.2 hereinabove, which shall always take precedence in that order over any material breach or default referred to in this Paragraph 13.3, M.I.T. shall have the right to terminate this Agreement and the rights, privileges and license granted hereunder effective on ninety (90) days' notice to LICENSEE. Such termination shall become automatically effective unless LICENSEE shall have cured any such material breach or default prior to the expiration of the ninety (90) day period.

13.4 LICENSEE shall have the right to terminate this Agreement at any time on six (6) months' notice to M.I.T., and upon payment of all amounts due M.I.T. through the effective date of the termination.

13.5 Upon termination of this Agreement for any reason, nothing herein shall be construed to release either party from any obligation that matured prior to the effective date of such termination; and Articles 1, 8, 9, 10, 12, 13.5, 13.6, and 15 shall survive any such termination. LICENSEE and any sublicensee thereof may, however, after the effective date of such termination sell all LICENSED PRODUCTS, and complete LICENSED PRODUCTS in the process of manufacture at the time of such termination and sell the same, provided that LICENSEE shall make the payments to M.I.T. as required by Article 4 of this Agreement and shall submit the reports required by Article 5 hereof.

13.6 Upon termination of this Agreement for any reason, any sublicensee not then in default shall remain in force and effect in accordance with its terms, with M.I.T. taking the place of LICENSEE, but not subject to any performance obligations of LICENSEE.

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14 - PAYMENTS, NOTICES AND OTHER COMMUNICATIONS

Any payments, notice or other communication pursuant to this Agreement shall be sufficiently made or given on the date of mailing if sent to such party by certified first class mail, return receipt requested, postage prepaid, addressed to it at its address below or as it shall designate by written notice given to the other party:

In the case of M.I.T.:

Director  
Technology Licensing Office  
Massachusetts Institute of Technology  
77 Massachusetts Avenue, NE25-230  
Cambridge, Massachusetts 02139

In the case of LICENSEE:

Innogene Pharmaceuticals, Inc.  
41 Fresh Pond Lane  
Cambridge, MA 02138

15 - MISCELLANEOUS PROVISIONS

15.1 All disputes arising out of or related to this Agreement, or the performance, enforcement, breach or termination hereof, and any remedies relating thereto, shall be construed, governed, interpreted and applied in accordance with the laws of the Commonwealth of Massachusetts, U.S.A., except that questions affecting the construction and effect of any patent shall be determined by the law of the country in which the patent shall have been granted.

15.2 The parties hereto acknowledge that this Agreement sets forth the entire Agreement and understanding of the parties hereto as to the subject matter hereof, and shall not be subject to any change or modification except by the execution of a written instrument signed by the parties.



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15.3 The provisions of this Agreement are severable, and in the event that any provisions of this Agreement shall be determined to be invalid or unenforceable under any controlling body of the law, such invalidity or unenforceability shall not in any way affect the validity or enforceability of the remaining provisions hereof.

15.4 LICENSEE agrees to mark the LICENSED PRODUCTS sold in the United States with all applicable United States patent numbers. All LICENSED PRODUCTS shipped to or sold in other countries shall be marked in such a manner as to conform with the patent laws and practice of the country of manufacture or sale.

15.5 The failure of either party to assert a right hereunder or to insist upon compliance with any term or condition of this Agreement shall not constitute a waiver of that right or excuse a similar subsequent failure to perform any such term or condition by the other party.

IN WITNESS WHEREOF, the parties have duly executed this Agreement the day and year set forth below.

MASSACHUSETTS INSTITUTE OF TECHNOLOGY

INNOGENE PHARMACEUTICALS, INC.

By /s/ Lita Nelsen

By /s/ Irving M. London

Name Lita L. Nelsen, Director

Name Irving M. London

Title Technology Licensing Office

Title President

Date Dec 18, 1996

Date Dec 18, 1996

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APPENDIX A

PATENT RIGHTS on the EFFECTIVE DATE

UNITED STATES PATENT RIGHTS

[\*\*\*]

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APPENDIX B

DESIGNATED FOREIGN COUNTRIES

Foreign countries in which PATENT RIGHTS shall be filed, prosecuted and maintained accordance with Article 6:

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#### **FIRST AMENDMENT**

This First Amendment, effective as of the date set forth above the signatures of the parties below, is between the Massachusetts Institute of Technology (“M.I.T.”), a Massachusetts corporation having its principal office at 77 Massachusetts Avenue, Cambridge, Massachusetts, 02139 and Genetix Pharmaceuticals, Inc. (“COMPANY”), a Delaware corporation, with a principal place of business at 840 Memorial Drive, Cambridge, Massachusetts 02139.

WHEREAS, COMPANY and M.I.T. wish to modify the provisions of the Exclusive Patent License Agreement dated December 18, 1996 as amended, (“LICENSE AGREEMENT”).

NOW, THEREFORE, in consideration of the promises and mutual covenants contained herein, the parties hereby agree to modify the LICENSE AGREEMENT as follows:

1. The following text shall be added to the end of Appendix A:  
[\*\*\*]
2. Section 4.1.b shall be changed such that [\*\*\*] shall be deleted and replaced with [\*\*\*].
3. A Case Addition Fee for MIT Case 10104 [\*\*\*] shall be due February 1, 2004.

The remaining terms and conditions of the LICENSE AGREEMENT remain intact.

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IN WITNESS WHEREOF, the parties have caused this Agreement to be executed under seal by their duly authorized representatives.

**The Effective Date of this First Amendment is December 12, 2003.**

MASSACHUSETTS INSTITUTE OF TECHNOLOGY

GENETIX PHARMACEUTICALS, INC.

By: /s/ Lita Nelsen

By: /s/ Ronald Dorazio MD

Name: Lita L. Nelsen, Director

Name: Ronald Dorazio, MD

Title: Technology Licensing Office

Title: Vice President

Date: 12/22/03

Date: Dec. 17, 2003

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## SECOND AMENDMENT

WHEREAS the Massachusetts Institute of Technology (“MIT”) and Genetix Pharmaceuticals Inc. of Cambridge, Massachusetts (“Genetix” or “Company,” formerly Innogene Pharmaceuticals, Inc.) are parties to the Patent License Agreement (“License Agreement”) entered into on December 18, 1996; and

WHEREAS Genetix currently lacks sufficient cash funds to substantially advance the development of its lead product candidate LentiGlobin™ which incorporates technology subject to the License Agreement or to make further payments under the License Agreement which could become due; and

WHEREAS Genetix has entered into a subsequent letter agreement (“Letter Agreement”) with MIT dated April 9, 2004 and providing for conditional retraction of MIT’s earlier Letter of Termination upon a financing of Genetix and certain payments by Genetix to MIT, as well as a further agreement (“First Amendment”) dated December 12, 2003 amending the License Agreement to incorporate certain new technology and payments by Genetix; and

WHEREAS Genetix’ lead product in development, LentiGlobin™, incorporates multiple technologies requiring licenses from multiple parties each bearing royalty, license fee, and sublicensing terms which are substantial and in the case of sublicensing terms are prohibitive in and of themselves, and Genetix now anticipates requirement for an additional license from at least one further party; and

WHEREAS [\*\*\*]; and

WHEREAS Genetix has not entered into any sublicense under the License Agreement nor has it initiated negotiation of such a sublicense; and

WHEREAS Genetix desires to realize the equity investment so that it may continue operations and advance LentiGlobin™ into human clinical trials, and Genetix has therefore proposed to MIT to amend Section 4.1.d of the License Agreement to eliminate the requirement for certain payments to made pursuant to future sublicenses, for the satisfaction of new investors; and

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WHEREAS MIT stands to benefit financially from receipt by Genetix of the investment, through the intended further development and potential commercialization of LentiGlobin™ under the License Agreement as amended below;

Now, therefore, MIT and Genetix agree as follows (the “Second Amendment”):

1. The first sentence of Section 4.1.d of the License Agreement shall be amended and replaced with the following:

“The following proportion of payments received from sublicensees in consideration for the LICENSED PRODUCTS and LICENSED PROCESSES:

- i. [\*\*\*]; or
- ii. [\*\*\*].”

The remainder of the same Section, beginning “Provided, however...” shall remain as previously agreed and written.

2. The License Agreement, the Letter Agreement, the First Amendment, and this Second Amendment reflect the entire Agreement between MIT and Genetix. This Agreement may only be modified in writing signed by a duly authorized representative of MIT and a duly authorized representative of Genetix.

MASSACHUSETTS INSTITUTE OF TECHNOLOGY

GENETIX PHARMACEUTICALS, INC.

/s/ Lita Nelsen

/s/ Walter C. Ogier

Lita Nelsen, Director, Technology Licensing Office

Walter C. Ogier, President and CEO

Dated: May 6, 2004

Dated: May 6, 2004

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THIRD AMENDMENT

This Amendment is to the license agreement dated December 11, 1996 between Massachusetts Institute of Technology and Bluebird Bio Inc. (formerly Innogene Pharmaceuticals, Inc., formerly Genetix Pharmaceuticals Inc.), hereinafter referred to as "LICENSEE", as subsequently amended by the First Amendment dated December 12, 2003 and the Second Amendment dated May 6, 2004.

The parties hereby further agree as follows:

1. The annual License Maintenance Fee payments of Paragraph 4.1(b) shall be changed to [\*\*\*] per year.
2. Paragraph 4.1(c) shall be replaced with the following:  
**Running Royalties in an amount equal to [\*\*\*] of NET SALES of LICENSED PRODUCTS and LICENSED PROCESSES used, leased and/or sold by LICENSEE or its SUBLICENSEES:**
3. Paragraph 4.1 (d), as amended in the Second Amendment, shall be replaced with the following:  
**(i) If only the PATENT RIGHTS are sublicensed: [\*\*\*]; excluding however:**
  - ((a)) [\*\*\*]; and**
  - ((b)) [\*\*\*]; and**
  - ((c)) [\*\*\*].****(ii) If the sublicense revenue is paid for a package including the PATENT RIGHTS and products developed by LICENSEE and/or substantial technology and/or intellectual property developed by LICENSEE: [\*\*\*]; excluding, however:**
  - ((a)) [\*\*\*]; and**
  - ((b)) [\*\*\*]; and**
  - ((c)) [\*\*\*]; and**
  - ((d)) [\*\*\*].**
4. Section 4.2 shall be deleted and replaced in its entirety by:  
**4.2 If LICENSEE (or its SUBLICENSEE) must pay royalties to a third party for patents necessary to the production or sale of LICENSED PRODUCTS or**



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**LICENSED PROCESSES, LICENSEE may credit [\*\*\*] of the royalties paid to the third party against the Running Royalties otherwise due under P.4.1 (c) above, provided that in no event shall the amount paid to M.I.T. for that LICENSED PRODUCT or LICENSED PROCESS be less than [\*\*\*] of NET SALES.**

This Amendment with the effective date of June 1, 2011 is hereby agreed to by:

MASSACHUSETTS INSTITUTE OF TECHNOLOGY

BLUEBIRD BIO, INC.

By: /s/ Lita Nelsen

By: /s/ Nick Leschly

Name: Lita L. Nelsen, Director

Name: Nick Leschly

Title: Technology Licensing Office

Title: CEO

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**PATENT AND KNOW-HOW LICENSE AGREEMENT**  
N° 07554F30

**BY AND BETWEEN**

**INSERM-TRANSFERT**, a limited company organized under the laws of France, whose registered headquarters are located 7 rue Watt, 75013 PARIS, France, N° SIRET 434 033 619 00025, code APE 731Z, RCS Paris B 434 033 619, represented by its Chairman of the Board of Management, Mrs. Cecile Tharaud, acting as delegatee of Institut National de la Santé Et de la Recherche Médicale (hereinafter referred to as “**INSERM**”), French National Institute of Health and Medical Research, a public scientific and technological establishment having its principal offices at 101 rue de Tolbiac, 75654 Paris Cedex 13, France,

Hereinafter referred to as “**INSERM-TRANSFERT**”,

Acting as representative of INSERM Unit U745 “*Genetic and Biotherapy of Degenerative and Proliferative Diseases of the Nervous System*”, directed by Pr Aubourg (hereinafter referred to as “**Laboratory**”) and located at [\*\*\*]

On the one hand

**AND**

**GENETIX, Inc.** having its principal place of business at 840 Memorial Drive, Cambridge, MA 02139 USA,

Hereinafter referred to as “**LICENSEE**”,

On the other hand

Hereinafter individually or collectively designated by “**Party**” or “**Parties**”

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#### **BACKGROUND**

- A. LICENSEE is a biotechnology company which is doing business in the field of gene therapy. It has offices in the US and in France.
- B. INSERM-TRANSFERT is INSERM’s wholly owned technology transfer subsidiary, created by a French decree in June 6, 2000. Effective January 1<sup>st</sup> 2006, INSERM delegated to INSERM-TRANSFERT the management of its technology transfer activities. Accordingly INSERM-TRANSFERT is empowered to negotiate, sign and manage license agreements including the present agreement.
- C. LICENSEE wishes to obtain a license from INSERM-TRANSFERT to the Patents and the related Know How (as these terms are defined hereunder), and INSERM-TRANSFERT is willing to grant LICENSEE such a license, all on the terms and conditions set forth below. The Parties entered into a Term Sheet N° 07554F10 for a license agreement on April 18, 2008, which detailed part of the conditions of the present agreement.
- D. In parallel to such discussions, the Parties and INSERM have also been discussing the execution of a collaborative research agreement (N° 07554F20). Considering the Parties’ common interests, they decided to enter into this Patent and Know-How License Agreement concurrently with said “collaborative research agreement N° 07554F20”.

The Parties wish to state in this preamble that the financing undertaking by LICENSEE was one of causes which led INSERM-TRANSFERT to sign both of these agreements (this Patent and Know-How Licence Agreement and the collaborative research agreement N° 07554F20); this is the reason why, should LICENSEE fail to perform its financing undertaking, INSERM-TRANSFERT will be provided with certain rights and obligations on the research programs and results therefrom in the conditions more fully detailed herein in order to seek a new partner to finalize the works and the files so as to obtain the marketing authorizations for the medicines in connection with the LentiD for Adrenoleukodystrophy therapies.

**NOW, THEREFORE, in consideration of the mutual covenants, conditions and undertakings herein contained, the Parties hereto agree as follows:**

#### **Preliminary ARTICLE - DEFINITIONS**

As used in this agreement (hereinafter the “**Agreement**”), the following terms shall have the meanings indicated:

“**Affiliate**” shall mean an entity that directly, or indirectly through one or more intermediaries, controls, or is controlled by, or is under common control with

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LICENSEE. For this purpose, the term « control » shall mean the ownership of more than 50% of the voting shares of such corporation or 50% of the ownership interests in such other business entity.

“**Field**” shall mean [\*\*\*].

“**Know-How**”: shall mean all technical information, know-how, process, biological material, data or other subject matter developed by [\*\*\*] of INSERM Unit [\*\*\*], owned or controlled by INSERM that exists as of the effective date of this Agreement, which is reasonably necessary or useful for the practice of the Patent, and know how / data (published / unpublished) which has lead to French Afssaps clinical study authorization, development of the study to be carrying out between Unit [\*\*\*] and LICENSEE and the future results of this study. This includes all data generated before the collaboration between INSERM and [\*\*\*].

“**Net Sales**” shall mean the amount of sales, excluding taxes, of the Products (in all its forms) invoiced to third parties, including distributors, by LICENSEE or its Affiliates, less any customary COGS, SG&A marketing, sales distribution costs, normal trade discounts and credit notes issued in respect of returned Products in each country of the Territory. It is understood that the deductions shall not exceed [\*\*\*]. In case LICENSEE could prove that the costs incurred by LICENSEE to sell Products are much higher than the deductions allowed in this Agreement, the Parties shall discuss to re-evaluate the percentage of deductions allowed.

Net Sales shall not include intermediate sales between LICENSEE and its Affiliate or its Affiliates between them. Net Sales shall only include the sales between the Affiliates (or LICENSEE) and third parties (and not the sales between LICENSEE and its Affiliates or its Affiliates between them).

“**Patents**”: shall mean patent N° [\*\*\*] filed on [\*\*\*] (US) by INSERM and issued under N° [\*\*\*], related to X-Linked adrenoleucodystrophy gene and corresponding protein Patent, and any foreign patent application corresponding thereto, and any divisional, continuation, or re-examination application, and each patent that issues or reissues from any of these patent applications. The following divisional application have been issued from the patent N° [\*\*\*]: N° [\*\*\*] and N° [\*\*\*].

“**Products**” shall mean any therapeutic product, gene, composition or process the manufacture, use or sale of which would constitute, but for the license granted herein, an infringement of the Patent and/or Know-How.

“**Sublicensee**” shall mean any non-Affiliate third party to whom LICENSEE has granted the right to manufacture and sell Products, with respect to Products made and sold by such third party.

“**Territory**” shall mean [\*\*\*].

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#### **ARTICLE 1 - OBJECT AND SCOPE OF THE AGREEMENT**

1.1 Subject to the limitations set forth in this Agreement, INSERM-TRANSFERT hereby grants to LICENSEE an exclusive, royalty-bearing license, with the right to grant sublicenses, in the Territory under the Patents and Know-How to develop, make, have made, use, and sell or otherwise distribute Products within the Field.

For the avoidance of doubt, this license authorizes LICENSEE to use the Patents and Know-How in the context of conducting research and development of [\*\*\*], with INSERM and INSERM-TRANSFERT as outlined and defined in the “collaborative research agreement N° 07554F20” concluded separately by LICENSEE, INSERM and INSERM-TRANSFERT. In case the Laboratory fails in carrying out its part of the research program of the “collaborative research agreement N° 07554F20”, this license authorizes LICENSEE to use the Patents and Know-How to conduct research alone or with any third party for the purpose of developing, make, have made, use and sell or otherwise distribute Products within the Field as described herein.

In the event that LICENSEE shall develop a Product and/or in the event that a result of any nature, whether patentable or not, is discovered and/or developed by LICENSEE, independently from its collaboration with INSERM and/or INSERM TRANSFERT within the “collaborative research agreement N° 07554F20”, the Parties agree that such Product and/or result shall belong exclusively to LICENSEE and that any new patent in relation thereto shall be filed by LICENSEE.

1.2 INSERM reserves the right to use the Patent and the related Know-How for educational, clinical and preclinical studies purposes.

#### **ARTICLE 2 - DURATION**

The Agreement is effective as of its last date of signature and shall last until the expiration on a country by country basis of the last to expire of any patent encompassed within the scope of the Patent or ten (10) years from the date of the first commercial sale of a Product whichever is later.

#### **ARTICLE 3 - SUBLICENSE**

Prior to the execution of any sublicense, LICENSEE shall provide INSERM-TRANSFERT written notification of the identity and address of the potential Sublicensee for approval, which approval shall not be unreasonably withheld. Should not INSERM-TRANSFERT withhold the Sublicensee within thirty (30) days from LICENSEE notification, then the Sublicensee shall be deemed approved by INSERM-TRANSFERT.

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Subject to any confidentiality obligation of LICENSEE, LICENSEE shall also notify INSERM-TRANSFERT with the terms of the sublicense for minimum information necessary for INSERM-TRANSFERT for internal reports, prior the signature of any sublicense.

Promptly following the execution of any sublicense, LICENSEE will communicate INSERM-TRANSFERT a signed copy of the agreement (possibly with some blank on information having to stay confidential between LICENSEE and Sublicensee).

For the avoidance of doubt, INSERM-TRANSFERT acknowledges that the terms of the sublicense and the sublicense agreement itself may be covered by a confidentiality obligation and that, as a consequence, the notification and communication obligations of LICENSEE described above may be subject to obtaining prior written consent of Sublicensee. In this respect, failure from Sublicensee to agree to such notification and communication of the sublicense terms and agreement to INSERM-TRANSFERT shall not give rise to any claim from INSERM-TRANSFERT and/or liability of LICENSEE.

#### **ARTICLE 4 - DUE DILIGENCE**

- 4.1 LICENSEE agrees to undertake all commercially reasonable efforts to develop Product as soon as practical, consistent with its reasonable business practices and judgment in compliance with the steps of the development plan attached in Exhibit 1 of the “collaborative research agreement N° 07554F20” as may be amended from time to time by the Parties. Should a significant variance from the development plan occurs and/or the LICENSEE fail to reasonably comply with the steps of the said development plan, the Parties shall meet within one (1) month following the notification made by INSERM-TRANSFERT to LICENSEE in order to engage in good faith discussions aiming at amending the development plan and/or finding a remedy to such failure from LICENSEE. Should the Parties fail to reach an agreement in this respect within three (3) months from the start of their discussions, INSERM-TRANSFERT may terminate the present Agreement in accordance with the termination procedure set out in Articles 9.4 and 9.5 below. Notwithstanding the above, as long as reasonable efforts are being pursued to maintain the development of a Product, the Agreement shall remain valid in force and INSERM-TRANSFERT shall not be able to terminate the Agreement as set out in Articles 9.4 and 9.5 below.
- 4.2 LICENSEE undertakes to use all commercially reasonable efforts to introduce Product into the commercial market as soon as practical, consistent with its reasonable business practices and judgment and necessary approvals by the regulatory authorities in the Territory.
- 4.3 LICENSEE shall comply with all applicable laws and regulations in connection with its activities pursuant to the Agreement.
- 4.4 LICENSEE shall provide to INSERM-TRANSFERT, within sixty (60) days from December 31 of each calendar year, a written annual progress report on the progress of its

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Product development or efforts to commercialize under the development plan. Such progress reports shall include, among others, the following topics: summary of work completed, summary of work in progress, current schedule of anticipated regulatory approvals, manufacturing, sublicensing efforts and market plans for introduction of Product.

4.5 INSERM-TRANSFERT shall inform its third party licensees of LICENSEE’S interests in the development and the commercialization strategies of any diagnosis product in the field of [\*\*\*].

**ARTICLE 5 - FINANCIAL TERMS**

5.1 Milestone Payments.

In partial consideration of the rights and license granted by INSERM-TRANSFERT to LICENSEE under this Agreement, LICENSEE agrees to make the following payments to INSERM-TRANSFERT upon the completion by LICENSEE or its Affiliates of each milestone specified below:

- Issue fee: [\*\*\*] excluding taxes, upon the execution of the Agreement.
- Milestone fees shall be paid to INSERM-TRANSFERT according to the following schedule for maintenance of the license:

<u>Milestone</u>	<u>Fee in euros, excluding taxes</u>
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]

Above payments shall be payable by LICENSEE for each Product and shall be non-refundable and non-creditable against royalty payments hereunder. For the avoidance of doubt, payments shall not be payable in relation to different versions of the Product containing the [\*\*\*] gene.

5.2 Royalty.

5.2.1 LICENSEE agrees to pay to INSERM-TRANSFERT a running royalty equal to [\*\*\*] of Net Sales by LICENSEE or its Affiliates, in the Territory. Should third party intellectual property rights be required for commercialization with total royalties due above [\*\*\*], INSERM-TRANSFERT shall have a reduction of its royalty equal to [\*\*\*] of the incremental royalty. None-the-less, the INSERM-TRANSFERT royalty shall not be reduced below [\*\*\*] of Net Sales by LICENSEE or its Affiliates, in the Territory.

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5.2.2 In the event of a Sublicense, LICENSEE shall also pay to INSERM-TRANSFERT the following royalties under any royalty and non-royalty sublicense income (except in the case of equity investment) received by LICENSEE or its Affiliates from any Sublicensee if sublicense is granted before:

- [\*\*\*]
- [\*\*\*]
- [\*\*\*]

For the avoidance of doubt, non-royalty sublicense income shall include any upfront license fee and milestone payments.

In case of equity investment by LICENSEE in counterpart of a sublicense of Patents and Know-How, LICENSEE shall pay sublicense royalties set forth above, and INSERM-TRANSFERT and LICENSEE shall confer together to define an appropriate and fair compensation for INSERM-TRANSFERT when LICENSEE shall sell said equities as part of the aforementioned sublicense.

5.3 Royalty Reports and Payments.

After the first commercial sale by LICENSEE, its Affiliate or Sublicensees of a Product for which royalties are payable under this Article 5, LICENSEE shall make annual written reports to INSERM-TRANSFERT within ninety (90) days after the end of each calendar year, stating in each such report [\*\*\*]. Simultaneously with the delivery of each such report, LICENSEE shall pay to INSERM-TRANSFERT the total royalties and non sublicense income, if any, due to INSERM-TRANSFERT for the period of such report. If no payments are due, LICENSEE shall so report. The aforesaid reports shall be certified as true and accurate by a duly authorized officer of LICENSEE.

5.4 Payment Method.

The sums due by LICENSEE shall be paid within thirty (30) days following receipt of the invoice from INSERM-TRANSFERT which shall state the amount of the sums due in application of the present Agreement and shall be paid by bank transfer to [\*\*\*].

All payments hereunder shall be made in Euros.

Any payments or portions thereof due under this Article 5 which are not paid on the date such payments are due under this Agreement shall bear interest equal to [\*\*\*] on the date such payment is due.



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5.5 Records; Inspection.

LICENSEE shall keep complete, true and accurate books of account and records for the purpose of determining the royalty amounts payable under this Article 5. Such books and records shall be kept reasonably accessible for at least three (3) years following the end of the calendar quarter to which they pertain. Such records will be open [\*\*\*] during such three (3) year period by an independent auditor for the purpose of verifying the royalty statements. INSERM-TRANSFERT shall bear the costs and expenses of inspections conducted under this Article 5.5, unless a variation or error producing an underpayment in royalties payable exceeding [\*\*\*] of the amount paid for the period covered by the inspection is established in the course of any such inspection, whereupon all costs relating to the inspection and any unpaid amounts that are discovered will be paid by LICENSEE, together with interest on such unpaid amounts at the rate specified in Article 5.4 above. In any case, all underpayments claimed by INSERM-TRANSFERT shall be dully documented by the auditor and LICENSEE shall have the right to contest the results of any inspection conducted under this Article 5.5.

5.6 Withholding tax:

LICENSEE shall assist INSERM-TRANSFERT in taking steps to avoid any double taxation and shall provide INSERM-TRANSFERT at its request with any document necessary to that end. LICENSEE shall use its best efforts to enable INSERM-TRANSFERT to have any withholding tax taken into account under the respective applicable Double Taxation Treaty.

**ARTICLE 6 - INTELLECTUAL PROPERTY**

6.1 Starting three (3) months after the execution of this Agreement, LICENSEE shall contribute to the payments of the Patent costs on the Territory, including costs imparted as from the execution of this Agreement.

INSERM-TRANSFERT shall control the prosecution, defence and maintenance of the Patent in the Territory; provided however that INSERM-TRANSFERT shall keep LICENSEE reasonably informed and consult with LICENSEE with respect to (i) the scope and content of all patent applications within the Patent; and (ii) content or proposed responses to official actions of national patent offices regarding the prosecution of the Patent. For purposes of this provision, “prosecution, defence and maintenance” of patents and patent applications shall be deemed to include, without limitation, the conduct of interferences or oppositions, invalidity suit and/or requests for re-examinations, reissues or extensions of patent terms.

LICENSEE shall pay [\*\*\*] of all expenses pre-approved in writing by LICENSEE and incurred in accordance with the present Article 6.1.

Such patent expenses are non-refundable and non-creditable against any royalty payments and milestones payments due hereunder.

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6.2 Infringement by third party

6.2.1 If it is believed in good faith that Patents are infringed by a third party, the Party first having knowledge of such infringement shall promptly notify the other in writing, which notice shall set forth the facts of such infringement in reasonable detail.

[\*\*\*]

[\*\*\*]

6.2.2 [\*\*\*]

6.3 [\*\*\*]

**ARTICLE 7 - CONFIDENTIALITY**

The Parties agree to respect and keep strictly confidential all scientific and technical information belonging to the other Party about which they may have knowledge due to the negotiation and execution of the Agreement.

In particular, LICENSEE agrees to keep and maintain strictly confidential all information that it may receive during the transfer of the Know-How.

The Parties agree to insure that their personnel and any other persons in their relationship in any respect whatsoever, respect and accept the obligations of confidentiality described in the Agreement.

The bilateral confidentiality obligations between the Parties pursuant to the present Article shall not include the use or disclosure of confidential information which the receiving Party can prove:

- a) was disclosed by the mutual agreement of both Parties, or was disclosed by the owning Party,
- b) was in the public domain at the moment of disclosure or entered the public domain through no act or fault of the receiving Party,
- c) was made available as a matter of lawful right by a third party,
- d) was in the possession of the receiving Party at the time of disclosure by the owning Party or was developed independently by its agents or employees who did not have access to confidential information,
- e) was disclosed by lawful right, to remain in compliance with existing regulations, an arbitration settlement or a final legal decision.

The obligations of confidentiality set out herein shall remain in effect during the term of the Agreement [\*\*\*].

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CERTAIN CONFIDENTIAL PORTIONS OF THIS EXHIBIT WERE OMITTED AND REPLACED WITH “[\*\*\*]”. A COMPLETE VERSION OF THIS EXHIBIT HAS BEEN FILED SEPARATELY WITH THE SECRETARY OF THE SECURITIES AND EXCHANGE COMMISSION PURSUANT TO AN APPLICATION REQUESTING CONFIDENTIAL TREATMENT PURSUANT TO RULE 406 PROMULGATED UNDER THE SECURITIES ACT OF 1933, AS AMENDED.

#### ARTICLE 8 - REPRESENTATIONS AND WARRANTIES

8.1 INSERM-TRANSFERT represents and warrants that the entering into the Agreement with LICENSEE, the grant of rights to LICENSEE under such Agreement and the exploitation of such rights by LICENSEE does not and will not constitute a breach of any preexisting agreements with any third party, including [\*\*\*], nor has or will INSERM-TRANSFERT share any third party's, including [\*\*\*]'s, confidential and/or proprietary information with LICENSEE.

The rights granted herein shall not include any right under [\*\*\*]'s confidential and/or proprietary information.

8.2 LICENSEE hereby represents and warrants to INSERM-TRANSFERT that it had access to all patent files and information necessary to fully appreciate the scope of the Patents and Know-How granted hereunder.

LICENSEE accepts the license to the Patents and Know-How “as is”. Neither INSERM-TRANSFERT nor INSERM and the inventors offer any guarantee as to the validity or scope of the Patents and Know-How under this Agreement. No warranties, express or implied, are offered under this Agreement as to the merchantability or the fitness for a particular purpose of the Patents and Know-How under this Agreement or that the use of the Patents and Know-How by LICENSEE, its Affiliates or its Sublicensees will not infringe any other patents or any other intellectual property rights of third parties.

8.3 LICENSEE shall hold harmless each of INSERM-TRANSFERT, INSERM and their directors, trustees, officers, employees, agents and the successors and assigns of any of the foregoing (collectively, the “**Indemnitees**”) against any and all claims brought by third parties alleging personal injury or property damage in conjunction with, or arising out of (1) practice by LICENSEE, its Affiliate and Sublicensees, their directors, trustees, officers, employees, contractors, subcontractors and agents, of the Patents or (2) the design, manufacture, distribution or use of Products by or under the authority of LICENSEE; provided that any Indemnitee seeking indemnification hereunder shall (i) promptly notify LICENSEE of such claim (ii) gives LICENSEE sole control of the defense or settlement of such claim, and (iii) provides LICENSEE, at LICENSEE's expenses, with reasonable assistance and full information with respect to such claim. Such indemnity shall include all costs and expenses, including reasonable attorneys' fees and any costs of settlement.

8.4 INSERM-TRANSFERT shall hold harmless LICENSEE and their directors, trustees, officers, employees, agents and the successors and assigns of any of the foregoing (collectively, the “**Licensee Indemnitees**”) against any and all claims brought by third parties alleging any loss, damage or prejudice in conjunction with, or arising out of any use or practice of the Patents before the signature date of this Agreement by or under the authority of INSERM-TRANSFERT, INSERM, their Affiliates, their directors, trustees, officers, employees, contractors, subcontractors and agents; provided that any Licensee

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Indemnitee seeking indemnification hereunder shall (i) promptly notify INSERM-TRANSFERT of such claim (ii) gives INSERM-TRANSFERT sole control of the defense or settlement of such claim, and (iii) provides INSERM-TRANSFERT, at INSERM-TRANSFERT’s expenses, with reasonable assistance and full information with respect to such claim. Such indemnity shall include all costs and expenses, including reasonable attorneys’ fees and any costs of settlement.

#### **ARTICLE 9 - TERMINATION**

- 9.1 INSERM-TRANSFERT grants to LICENSEE a right to unilaterally terminate the Agreement three (3) months after execution of the Agreement; in such case, the issue fee mentioned hereabove shall not be refundable to LICENSEE. No other monies will be due to INSERM-TRANSFERT.
- 9.2 Either LICENSEE or INSERM-TRANSFERT may terminate the Agreement, in the event the other shall have materially breached or defaulted in the performance of any of its material obligations hereunder and such breach shall have continued for sixty (60) days after written notice is given by the non breaching Party to the breaching Party specifying the breach.
- 9.3 In the event LICENSEE becomes the subject of a voluntary or involuntary petition in bankruptcy, LICENSEE shall immediately notify INSERM-TRANSFERT in writing. If such petition is not dismissed with prejudice within one hundred and twenty (120) days after filing, INSERM-TRANSFERT shall have the right to terminate this Agreement by giving LICENSEE written notice. Termination of this Agreement pursuant to this provision shall be effective upon LICENSEE’s receipt of such written notice.
- 9.4 INSERM-TRANSFERT may terminate this Agreement in case LICENSEE can not prove, within sixty (60) days from written notice by INSERM-TRANSFERT to do so, that it has been diligent or it has made its commercially reasonable efforts as described in Article 4 above.
- 9.5 Specific provision

In consideration of:

- (i) the financing undertaking set out in article 4.1 of the “collaborative research agreement N° 07554F20”,
- (ii) the financial undertaking set out in Article 5 of this Agreement,
- (iii) the undertakings relating to intellectual property matters defined in article 8 of the “collaborative research agreement N° 07554F20” and in Article 6 of this Agreement as well as the forecast budget to finance in accordance with the step plan set out in Exhibit 1 of the “collaborative research agreement N° 07554F20” (“development plan”).

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LICENSEE accepts the principle that should INSERM-TRANSFERT exercise its termination right for breach stated in the last paragraph of the preamble by application of Articles 4.1, 9.4 and/or 9.5 herein, the status of all the works and research programs performed in connection with the “collaborative research agreement N° 07554F20” signed among the same Parties concurrently with this Agreement as well as the results, whether protected or not, which will derive therefrom will be deemed to follow the principles of article 10.3 of the “collaborative research agreement N° 07554F20”.

All the files carried out and the results obtained, which will constitute the state of the works in progress, under any form whatsoever, at the date on which INSERM-TRANSFERT exercises its termination right in compliance with the terms stated in the last paragraph of the preamble by application of Articles 4.1 will be dealt with and transferred to the INSERM and LICENSEE in consideration of the status of the works concerned based on the above mentioned principles.

Each Party undertakes to carry out all the procedures and sign all the documents that the other Party may request for the proper performance of such transfer in favor of one or the other Party, based on the above mentioned principles.

Should the “collaborative research agreement N° 07554F20” be terminated in accordance with articles 10.3 of said agreement, this Agreement shall be automatically terminated.

- 9.6 Articles 7 and 8 shall survive the expiration and any termination of this Agreement. Except as otherwise provided in this Article 9, all rights and obligations of the Parties under this Agreement shall terminate upon the expiration or termination of this Agreement.

## **ARTICLE 10 - GENERAL**

### 10.1 Independent Contractors.

The relationship of LICENSEE and INSERM-TRANSFERT established by this Agreement is that of independent contractors. Nothing in this Agreement shall be construed to create any other relationship between LICENSEE and INSERM-TRANSFERT. No Party shall have any right, power or authority to contract or incur any expense, liability or obligation, express or implied, on behalf of the other Party.

### 10.2 Use of name.

Except as required by law, neither LICENSEE nor INSERM-TRANSFERT shall use the name of LICENSEE, INSERM-TRANSFERT or INSERM in issuing any press release or other public statements in connection with this Agreement intended for use in the public media without the approval of such Party, which approval shall not be unreasonably withheld.

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10.3 Assignment.

This Agreement may not be assigned by any Party without the prior written consent of the other Party hereto.

10.4 Change of control.

Should a change in control occur of LICENSEE, the Agreement shall be transferable to the acquiring third party with notice to INSERM-TRANSFERT. Such third party will be bound by the terms of this Agreement. An amendment shall be sign between INSERM-TRANSFERT and said acquiring third party. Said acquiring third party shall provide a new development plan to INSERM-TRANSFERT stating its development strategy to exploit Patents and Know-How.

10.5 Force Majeure.

In the event any Party hereto is prevented from or delayed in the performance of any of its obligations hereunder by reason of acts of God, war, strikes, riots, storms, fires, or any other cause whatsoever beyond the reasonable control of the Party, the Party so prevented or delayed shall be excused from the performance of any such obligation to the extent and during the period of such prevention or delay.

10.6 Notices.

Any notice or other communication required by this Agreement shall be made in writing and given by prepaid, first class, certified mail, return receipt requested, and shall be deemed to have been served on the date received by the addressee at the following address or such other address as may from time to time be designated to the other Party in writing:

*If to INSERM-TRANSFERT:*

Inserm Transfer SA  
7 rue Watt  
75013 Paris  
France

*If to LICENSEE:*

Genetix Pharmaceuticals, Inc.  
840 Memorial Drive  
Cambridge, MA 02139 USA  
Attn: CEO

10.7 Governing Law - Dispute.

The Agreement shall be construed in accordance with the laws of FRANCE.

Any dispute or controversy arising out of this Agreement which could not have been resolved amicably shall be settled by French Courts.

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10.8 Headings.

Headings included herein are for convenience only, do not form a part of this Agreement and shall not be used in any way to construe or interpret this Agreement.

10.9 Severability.

If any provision of this Agreement shall be found by a court to be void, invalid or unenforceable, the same shall be reformed to comply with applicable law or stricken if not so conformable, so as not to affect the validity or enforceability of the remainder of this Agreement.

10.10 Entire Agreement.

This Agreement and its Exhibits constitute the entire understanding and agreement between the Parties with respect to the subject matter hereof and may not be modified, amended or discharged except as expressly stated herein or by a written agreement duly executed by both Parties.

10.11 Counterparts.

This Agreement may be executed in counterparts, each of which shall be deemed an original, and taken together shall constitute one and the same agreement.

10.12 Government approval or registration.

If this Agreement or any associated transaction (in particular registration at the Registre National des Brevets and National Patent Office, and any fiscal registration) is required by the law of any nation to be either approved or registered with any governmental agency, LICENSEE shall assume all legal obligations to do so.

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IN WITNESS WHEREOF, the Parties hereto have caused their duly authorized representatives to execute this Agreement.

Done in two (2) original copies, on May 14, 2009

**For Inserm-Transfert**

/s/ Cecile Tharaud

Mrs. Cecile Tharaud

Chairman of the Board of Management

**For Genetix**

/s/ Alfred Slanetz

Mr. Alfred Slanetz

CEO



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**PATENT AND KNOW-HOW LICENSE AGREEMENT No 07554F30  
AMENDMENT No 1**

**BY AND BETWEEN**

**INSERM-TRANSFERT**, a limited company (*société anonyme à directoire et conseil de surveillance*) organized under the laws of France, whose registered headquarters are located 7 rue Watt, 75013 **PARIS**, France, N° SIRET 434 033 619 00025, code APE 731 Z, RCS Paris B 434 033 619, represented by its Chairman of the Board of Management, Mrs. Cecile Tharaud, acting as delegate of Institute National de la Santé Et de la Recherche Médicale (hereinafter referred to as “**INSERM**”), French National Institute of Health and Medical Research, a public scientific and technological establishment having its principal offices at 101 rue de Tolbiac, 75654 Paris Cedex, 13, France,

Hereafter referred to as “**INSERM-TRANSFERT**”,

Acting as representative of INSERM Unit U986 GENOMIQUE, FACTEURS ENVIRONNEMENTAUX ET BIOTHERAPIE DES MALADIES ENDOCRINIENNES ET NEUROLOGIQUES directed by Professor Pierre Bougnères (hereinafter referred to as “**Laboratory**”) and located at [\*\*\*].

On the one hand

**AND**

**bluebird bio, Inc.** (formerly Genetix, Inc.) having its principal place of business at 840 Memorial Drive, Cambridge, MA 02139,

Hereinafter referred to as “**LICENSEE**”,

On the other hand

Hereinafter individually or collectively designated as “**Party**” or “**Parties**”

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## BACKGROUND

A. INSERM-TRANSFERT and LICENSEE are parties to that certain Patent and Know-How License Agreement, N° 07554F30, dated as of May 14, 2009 (the “**License Agreement**”), pursuant to which, among other things, INSERM-TRANSFERT granted a license to LICENSEE under the Patents and related Know-How on terms and conditions set forth in the License Agreement.

B. Following execution of the License Agreement, LICENSEE will start clinical trials and has used Know-How in connection with its regulatory filings relating thereto.

C. INSERM-TRANSFERT and LICENSEE wish to enter into this Amendment No 1 to the License Agreement ( “**Amendment No 1**”) to amend the financial terms of the License Agreement so as to accurately reflect the value of the license granted.

D. Capitalized terms used but not otherwise defined herein shall have the meanings ascribed to such terms in the License Agreement.

NOW, THEREFORE, in consideration of the mutual covenants, conditions and undertakings herein contained, the Parties hereto agree as follows:

1. Amendment Fee. In consideration of the execution of this Amendment No 1, LICENSEE agrees to pay to INSERM-TRANSFERT a one-time payment of [\*\*\*] within thirty (30) business days of the full execution of this Amendment No 1. This payment is irrevocable, non refundable and non creditable against any past or future payments under the License Agreement.

2. Additional Payments. The Parties agree to negotiate in good faith towards the entry of a new research and collaboration agreement involving research between LICENSEE and Prs. Patrick Aubourg and [\*\*\*], which agreement will include funding from LICENSEE to INSERM in support of such research in an amount not to exceed [\*\*\*].

3. Definitions.

The definition of Net Sales in the License Agreement is hereby amended and restated as follows:

“**Net Sales**” shall mean the amount of sales, excluding taxes, of the Products (in all its forms) invoiced to third parties, including distributors, by LICENSEE or its Affiliates or its Sublicensees, less any customary COGS, SG&A marketing, sales distribution costs, normal trade discounts and credit notes issued in respect of returned Products in each country of the Territory. It is understood that the deductions shall not exceed [\*\*\*]. In case LICENSEE could prove that costs incurred by LICENSEE to sell Products are much higher than the deductions allowed in this Agreement, the Parties shall discuss to re-evaluate the percentage of deductions allowed. Net Sales shall not include intermediate sales between LICENSEE and its Affiliates or between LICENSEE or its Affiliates and any Sublicensee.

Further, the Parties agree that the definition of Know-How will be expanded to include clinical data [\*\*\*], as well as other clinical data that relates to follow up on the subjects participating in the clinical study. Such expanded definition will be included in a second amendment to the License Agreement that the Parties have agreed to execute by February 1, 2013.

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4. Section 1.1. Section 1.1 is amended and restated in its entirety as follows:

“1.1. Subject to the terms of this Agreement, INSERM-TRANSFERT hereby grants to LICENSEE an exclusive, royalty-bearing license, with the right to grant sublicenses (“Sublicense(s)”), in the Territory under the Patents and Know-How to develop, make, have made, use and sell or otherwise distribute Products within the Field.

For the avoidance of doubt, this license authorizes LICENSEE to use the Patents and Know-How, alone or with any third party, in the context of conducting research and development of [\*\*\*], including, without limitation, making submissions to regulatory authorities in the Territory, to develop, make, have made, use and sell or otherwise distribute Products within the Field.

5. Section 5.1. Section 5.1 of the Original Agreement is hereby amended and restated as follows:

“5.1 Milestone Payments.

In partial consideration of the rights and license granted by INSERM-TRANSFERT to LICENSEE under this Agreement, LICENSEE agrees to make the following payments to INSERM-TRANSFERT upon the completion by LICENSEE or its Affiliates or Sublicensees of each milestone specified below:

Milestone fees shall be paid to INSERM-TRANSFERT according to the following schedule for maintenance of the license:

<u>Milestone</u>	<u>excluding taxes</u>
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]

Each milestone is payable one time only no matter how many times any of the milestone events are achieved (i.e., if one Product is replaced by another Product, then the milestone shall be paid one time only, on the first Product to achieve that milestone). The above payments shall be non-refundable and non-creditable against other payments hereunder.”

For the avoidance of doubt, the above milestones payments are due for the whole duration of the License Agreement, even if the last to expire of any patent encompassed within the scope of the Patents has expired.

6. Section 5.2.1. Section 5.2.1 of the License Agreement is hereby amended and restated as follows:

“5.2.1 LICENSEE agrees to pay to INSERM-TRANSFERT a running royalty equal to [\*\*\*] of Net Sales by LICENSEE or its Affiliates or Sublicensees in the Territory.”

For the avoidance of doubt, royalty payments at the rate of [\*\*\*] of Net Sales are due for the whole duration of the License Agreement, even if the last to expire of any patent encompassed within the scope of the Patents has expired.

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7. Section 5.2.2. Section 5.2.2 of the License Agreement is hereby amended and restated as follows:

“5.2.2 In the event LICENSEE or its Affiliates executes a Sublicense for Product commercialization rights, LICENSEE shall pay to INSERM-TRANSFERT a sublicensing fee equal to [\*\*\*] upon receipt of the first Sublicense income (whether for a royalty or non royalty income) from such Sublicense. For further clarity, no Sublicense fee would be owed for LICENSEE’s or its Affiliates’ grant to a contract manufacturing organization of Product commercial manufacturing rights. For the avoidance of doubt, the foregoing sublicensing fee shall be paid by LICENSEE only one time for each of LICENSEE’s commercial partners regardless of the number of Sublicenses necessary for LICENSEE’s relationship with such commercial partner (e.g., LICENSEE shall only pay one sublicensing fee in connection with granting a Sublicense to a commercial partner and a Sublicense to a contract manufacturing organization to manufacture Products for such commercial partner).”

8. Section 9.5. Section 9.5 of the License Agreement is hereby deleted and shall be of no further force and effect.

9. Miscellaneous. Except as specifically amended hereby, the License Agreement shall remain in full force and effect in accordance with its terms.

10. Counterparts. This Amendment No 1 may be executed in counterparts, each of which shall be deemed an original, and taken together shall constitute one and the same instrument.

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IN WITNESS WHEREOF, the Parties hereto have caused their duly authorized representatives to execute this Amendment.

Done in two (2) original copies, on December 21, 2012.

FOR INSERM-TRANSFERT:

Alexandra CARREL  
General Counsel  
Inserm Transfert

/s/ Cecile Tharaud

P/O Name: Cecile Tharaud  
Title: CEO

FOR LICENSEE:

bluebird bio, Inc.

/s/ Jeffrey T. Walsh

Name: Jeffrey T. Walsh  
Title: COO

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**PATENT AND KNOW-HOW LICENSE AGREEMENT No 07554F32  
AMENDMENT No 2**

**BETWEEN**

**INSERM-TRANSFERT SA**, “*Société Anonyme à Directoire et Conseil de Surveillance*”, a limited company organized under the laws of France, with share capital of €9,573,470, whose registered headquarters are located at 7 rue Watt, 75013 Paris, France, SIRET No. 434 033 619 00025 business (APE) code 7219Z, Paris Trade and Companies Registry No. B 434 033 619, represented by its Chairman of the Management Board, Mrs. Cécile Tharaud,

Acting as delegatee of the French National Institute of Health and Medical Research ( *Institut National de la Santé et de la Recherche Médicale* - hereinafter “**INSERM**”), a public scientific and technological institute, having its registered headquarters at 101 rue de Tolbiac, 75013 Paris, France.

OF THE FIRST PART

**AND**

**bluebird bio, Inc.** (formerly known as Genetix, Inc.) a corporation organized under the laws of the state of Delaware, U.S.A., having its principal place of business at 840 Memorial Drive, Cambridge, Massachusetts 02139 U.S.A.

Hereinafter referred to as “**LICENSEE**”

OF THE SECOND PART,

Referred to individually as a “**Party**” and collectively as the “**Parties**”

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#### **BACKGROUND**

- A. INSERM delegated to INSERM-TRANSFERT, its wholly-owned private law technology transfer subsidiary, the management of its technology transfer activities including the negotiation and signature of research, collaborative, license, service and confidentiality and non disclosure agreements relating to the research conducted solely or jointly by INSERM’s research units.
- Pursuant to this agreement, INSERM-TRANSFERT is authorized to use and call on the material and human resources of the INSERM laboratories for the proper performance of the agreements entered into by INSERM-TRANSFERT, it being specified that INSERM-TRANSFERT is in charge of the negotiation, signature and follow-up of these agreements, and INSERM is in charge of the implementation and performance of the works that are the subject of these agreements as well as the financial management thereof.
- It is however specified that this delegation does not entail the transfer to INSERM-TRANSFERT of the property rights held by or jointly held with INSERM.
- For the performance of this License Agreement (as defined below), INSERM is not considered as a third party.
- B. INSERM is the sponsor of the INSERM Trial (as defined below).
- C. INSERM-TRANSFERT, acting on behalf of INSERM, and LICENSEE entered into a Patent and Know-How License Agreement on 14 May 2009 (“**Original License Agreement**”), and amended on 21 December, 2012 (“**Amendment No 1**”) (Original License Agreement and Amendment No 1 collectively, “**License Agreement**”). Pursuant to the License Agreement, INSERM-TRANSFERT granted to LICENSEE an exclusive, royalty bearing license, with the right to grant sublicenses, in the Territory under the Patents and Know-How to further develop a treatment for adrenoleukodystrophy.
- D. The purpose of Amendment No 1 was to amend the financial terms of the License Agreement so as to accurately reflect the value of the license granted.
- E. INSERM has collected and will continue to collect certain clinical data from the INSERM Trial. In addition to the rights granted under the License Agreement, INSERM-TRANSFERT agrees to provide LICENSEE with access to Clinical Data (as defined below) and LICENSEE, in connection with such access, is willing to pay a lump sum payment, as further described in this Amendment No 2 to the License Agreement (“**Amendment No 2**”).
- F. The purpose of this Amendment No 2 is to expand the definition of Know-How to include Clinical Data and to provide for the conditions of (i) the transfer of Clinical Data from INSERM to LICENSEE, (ii) access by LICENSEE to Clinical Data, and (iii) additional funding from LICENSEE to INSERM-TRANSFERT in connection with such transfer and access.
- G. Terms used in this Amendment No 2 and not defined herein will have the meaning ascribed to them in the License Agreement.

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NOW, THEREFORE, in consideration of the mutual covenants, conditions and undertakings herein contained, the Parties hereto agree to amend the License Agreement as follows:

1. The following definitions are added to the Preliminary Article - DEFINITIONS of the License Agreement:

“**Applicable Law**” means all European and national laws, regulations, rules and guidances applicable to the conduct of the INSERM Trial, including, without limitation, the EU Clinical Trial Directive, the EU Data Protection Directive and publications of the International Conference on Harmonization of Technical Requirements for Registration of Pharmaceuticals for Human Use.

“**Clinical Data**” means the Trial Subject (as defined below) de-identified data (i.e. not containing any direct identifiers about human subjects) in the CRFs (as defined below) and/or medical records collected and to be collected by the Laboratory (as defined below) pursuant to the Clinical Protocol (as defined below) and which INSERM and Investigator agreed to transfer to LICENSEE as reflected in the description of the content of the Database to be attached as Exhibit A.

“**Clinical Data Term**” means (a) for Clinical Data that is not Published Clinical Data (as defined below), ten (10) years from the Clinical Data Transfer Effective Date (as defined below), it being specified that INSERM-TRANSFERT agrees to discuss in good faith a no-fee extension of this term should the LICENSEE Purpose not be met at the end of the initial ten (10) year period, and (b) for Published Clinical Data that is generally available to the public, for such period of time following the Clinical Data Transfer Effective Date as LICENSEE deems necessary to meet the Licensee Purpose.

“**Clinical Data Transfer Effective Date**” means the date specified under Article 11 below.

“**Clinical Protocol**” means protocol no. [\*\*\*], as well as amendments that relate to the Gap Period and the Follow Up Period (each as defined below), together with all other amendments thereto, approved by the relevant French regulatory authorities.

“**CRFs**” means the paper (or, as the case may be, electronic) case report forms prepared and owned by INSERM containing, among other information, the Clinical Data generated during the Original Period and the Follow-Up Period (all as defined below).

“**Database**” means the secured electronic data entry system built by or on behalf of LICENSEE, with content pursuant to Exhibit A, for use by INSERM and Investigator (as defined below) as a repository for Clinical Data.

“**Follow Up Period**” means the portion of the INSERM Trial that extends from the Gap Period to fifteen (15) years post transplant for each Trial Subject.

“**Gap Period**” means the portion of a Trial Subject’s participation in the INSERM Trial that begins on completion of the year 2 visit and ends on the date the first Clinical Protocol amendment is approved by the relevant French regulatory authorities, at which time, the Follow Up Period will begin.

“**Laboratory**” means INSERM Unit [\*\*\*].

“**INSERM Trial**” means the clinical trial sponsored by INSERM pursuant to the Clinical Protocol and conducted during the Original Period, the Gap Period and the Follow Up Period.

“**LICENSEE Purpose**” means the use by LICENSEE (a) of Clinical Data that is not Published Clinical Data (i) to support regulatory filings to put a Product into registration phase, (ii) in publications and presentations subject to the terms of Section 11.7(b) and provided the Trial Subject is not directly or indirectly identifiable by name (of Trial Subject or his/her relatives), initials, contact information, date of birth or country of origin, and (iii) as may be required to comply with legal and regulatory obligations, and (b) of Published Clinical Data, for any and all purposes.



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“**Original Period**” means the first two (2) years of a Trial Subject’s participation in the INSERM Trial.

“**Published Clinical Data**” means the Clinical Data published in the following article:

- [\*\*\*],

and any other Clinical Data published in a scientific publication or presented in scientific congresses. Any Clinical Data which is not Published Clinical Data as specified in this definition shall be deemed confidential and subject to all terms applicable to the use of Clinical Data under this Agreement.

“**Project Plan**” means the set of activities, timeframes and requirements set forth in Exhibit B agreed to by INSERM and LICENSEE in connection with INSERM’s provision of Clinical Data to LICENSEE pursuant to this Agreement, as may be amended from time by mutual written agreement of INSERM, INSERM-TRANSFERT, and LICENSEE.

“**Summary Clinical Data**” means a summarized version of the Clinical Data that has been subjected to processing or manipulation by LICENSEE and which does not contain the name (of Trial Subject or his/her relatives), initials, contact information, date of birth or country of origin of the Trial Subject. An exhaustive list of what shall constitute Summary Clinical Data is attached as Exhibit C to this Amendment No 2.

“**Trial Subjects**” means all patients enrolled in the INSERM Trial.

2. The definition of Know-How contained to the Preliminary Article - DEFINITIONS of the License Agreement is hereby amended and restated as follows:

“**Know-How**”: shall mean (a) all technical information, know-how, process, biological material, data or other subject matter developed by [\*\*\*] of the Laboratory, owned or controlled by INSERM that exists as of the effective date of this Amendment No 2, which is reasonably necessary or useful for the practice of the Patent, (b) all know how/data (published/unpublished) which has led to French regulatory agency authorization of the INSERM Trial, development of the INSERM Trial and the future results of the INSERM Trial, and (c) Clinical Data. For further clarity, Know-How includes all data generated before the collaboration between INSERM [\*\*\*].

3. Section 1.1 to ARTICLE 1 - OBJECT AND SCOPE OF THE AGREEMENT of the License Agreement is amended and restated as follows:

“1.1. Subject to the terms of this Agreement (including its amendments), INSERM-TRANSFERT hereby grants to LICENSEE an exclusive, royalty-bearing license, with the right to grant Sublicenses, in the Territory under the Patents and Know-How to develop, make, have made, use and sell or otherwise distribute Products within the Field.

(a) For the avoidance of doubt and without prejudice to the terms of use of the Clinical Data by LICENSEE, this license authorizes LICENSEE to use the Patents and Know-How, alone or with any third party, in the context of conducting research and development of [\*\*\*], including, without limitation, making submissions to regulatory authorities, to develop, make, have made, use and sell or otherwise distribute Products within the Field and in the Territory.

(b) For the avoidance of doubt, this license authorizes LICENSEE to release Summary Clinical Data to support LICENSEE’s publications and presentations (subject to INSERM and Investigator’s approval under Section 11.7) and for business purposes, including raising capital, as LICENSEE deems necessary in connection with the due diligence requirements under the License Agreement, it being specified that Section 11.7(a) (ii) shall not apply to such release but will be subject to any other terms of use as provided in this License Agreement (including its amendments).”

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4. The following Subsection 1.3 is added to ARTICLE 1 - OBJECT AND SCOPE OF THE AGREEMENT of the License Agreement.

“1.3 More specifically, as owner of the Clinical Data, INSERM may use Clinical Data for any non-profit research (including clinical research) and educational purposes, to the exclusion of any use by an industrial partner in the Field or transmission of the Clinical Data to an industrial partner for any use inside the Field. For the avoidance of doubt, it is specified that Clinical Data may be freely used for any purpose by INSERM outside the Field, including any transmission and use by an industrial partner .”

5. ARTICLE 2 - DURATION of the License Agreement is hereby amended and restated as follows:

“2.1 The Agreement is effective as of its last date of signature and shall last until the last to occur of the following: (a) the expiration on a country by country basis of the last to expire of any patent encompassed within the scope of the Patents, including any supplementary protection certificate (SPC) (or other extension of protection) and any commercial exclusivity for orphan and/or paediatric drugs, if applicable, or (b) the end of a ten (10) year period after the date of the first commercial sale of a Product.”

2.2

- (a) In the event of an early termination of the License Agreement by LICENSEE as provided under Section 9.1, or by INSERM-TRANSFERT as provided under Sections 9.2, 9.3 and/or 9.4, LICENSEE shall return to INSERM all Clinical Data that is not Published Clinical Data and shall not make any further use of such Clinical Data and more generally of the Patents and Know-How; provided however, that any such early termination shall not affect any use of Clinical Data, Patents or Know-How made by LICENSEE prior to the effective date of such early termination. By way of example, LICENSEE shall not be required to remove any Clinical Data incorporated by LICENSEE into regulatory submissions made prior to the effective date of termination.
- (b) In the event of a material breach or default by INSERM or INSERM TRANSFERT in the performance of any of its material obligations hereunder and such breach shall have continued for sixty (60) days after written notice given by LICENSEE specifying the breach, LICENSEE may either:
  - (i) terminate the License Agreement (as amended under this Amendment No 2) as a whole pursuant to Section 9.2, in which case, LICENSEE shall return to INSERM all Clinical Data that is not Published Clinical Data and shall not make any further use of such Clinical Data and more generally of the Patents and Know-How; provided however, that any such early termination shall not affect any use of Clinical Data, Patents or Know-How made by LICENSEE prior to the effective date of such early termination. By way of example, LICENSEE shall not be required to remove any Clinical Data incorporated by LICENSEE into regulatory submissions made prior to the effective date of termination; or
  - (ii) partially terminate the License Agreement (as amended under this Amendment No 2) (“**Partial Termination**”). “Partial Termination” means (1) all rights and obligations of the Parties under the License Agreement shall terminate for the future except for the Parties rights and obligations under Articles 7, 8, and 10.7 which shall survive such termination, and (2) the rights of LICENSEE to (a) use Clinical Data transferred to LICENSEE prior to the effective date of such Partial Termination in accordance with the LICENSEE Purpose and Summary Clinical Data in accordance with Section 1.1(b) and to use Patents and Know-How transferred to LICENSEE prior to the effective date of such Partial Termination, in each case, in accordance with the terms of the License Agreement (as amended by this Amendment No 2) and (b) conduct source document verification [\*\*\*], shall survive such Partial Termination. In any case, the License Agreement (as amended by this Amendment No 2) and notably the financial terms, shall remain in force regarding the rights and obligations of the Parties concerning Clinical Data, the Patents and Know-How transferred to LICENSEE prior to the effective date of such Partial Termination.

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6. The following Subsection 5.1.2 is added to ARTICLE 5 - FINANCIAL TERMS of the License Agreement:

“5.1.2 Clinical Data Access Fee: In consideration for the transfer of Clinical Data from four (4) Trial Subjects from INSERM to LICENSEE and the access to Clinical Data granted to LICENSEE pursuant to the License Agreement and this Amendment No. 2, LICENSEE shall pay to INSERM-TRANSFERT the total sum of [\*\*\*]. Invoices are to be submitted to LICENSEE by email to: [\*\*\*]. Installments will be paid within thirty (30) days of receipt of an invoice as follows:

- (i) [\*\*\*];
- (ii) [\*\*\*];
- (iii) [\*\*\*], and
- (iv) [\*\*\*].”

7. A new paragraph is added at the end of ARTICLE 7 - CONFIDENTIALITY of the License Agreement:

“Notwithstanding the above paragraph and subject to the rights to Clinical Data granted to LICENSEE under Article 11 which will prevail in the event of a conflict with the provisions of this Article 7, it is specified that the Clinical Data must be maintained confidential by LICENSEE without any time limit, unless it can prove that any of the exceptions listed under a) to e) above applies. For the avoidance of doubt, confidentiality obligations do not apply to Published Clinical Data”

8. Sections 8.3 and 8.4 of ARTICLE 8 - REPRESENTATIONS AND WARRANTIES of the License Agreement are replaced in their entirety with the following new Sections 8.3 and 8.4:

“8.3 LICENSEE shall hold harmless each of INSERM-TRANSFERT and INSERM and their respective directors, trustees, officers, employees, and agents, and the successors and assigns of any of the foregoing (collectively, the “**INSERM Indemnitees**”) against any and all claims brought by third parties alleging any loss, damage or prejudice in conjunction with, or arising out of (i) practice by any LICENSEE Indemnatee (as defined below) of the Patents, or (ii) the design, manufacture, distribution or use of Products by or under the authority of LICENSEE, or (iii) use by a LICENSEE Indemnatee of the Know-How, except, in each case, to the extent resulting from (a) an INSERM Indemnatee’s gross negligence or willful misconduct, or (b) INSERM-TRANSFERT’s breach of the terms of the License Agreement or this Amendment No 2; provided that any INSERM Indemnatee seeking indemnification

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hereunder shall (1) promptly notify LICENSEE of such claim, (2) give LICENSEE sole control of the defense or settlement of such claim, and (3) provide LICENSEE, at LICENSEE’s expenses, with reasonable assistance and full information with respect to such claim. Such indemnity shall include all costs and expenses, including reasonable attorneys’ fees and any costs of settlement.”

“8.4 INSERM-TRANSFERT and INSERM shall hold harmless LICENSEE, its Affiliates and Sublicensees, and their respective directors, trustees, officers, employees, contractors, subcontractors and agents, and the successors and assigns of any of the foregoing (collectively, the “**LICENSEE Indemnitees**”) against any and all claims brought by third parties alleging any loss, damage or prejudice in conjunction with, or arising out of (i) any use or practice of the Patents before the signature date of the Original License Agreement by or under the authority of an INSERM Indemnitee, or (ii) use of the Know-How by an INSERM Indemnitee or (iii) the conduct of the INSERM Trial, except, in each case, to the extent resulting from (a) a LICENSEE Indemnitee’s gross negligence or willful misconduct, or (b) LICENSEE’s breach of the terms of the License Agreement or this Amendment No 2; provided that any LICENSEE Indemnitee seeking indemnification hereunder shall (1) promptly notify INSERM-TRANSFERT of such claim, (2) give INSERM-TRANSFERT sole control of the defense or settlement of such claim, and (3) provide INSERM-TRANSFERT, at INSERM-TRANSFERT’s expenses, with reasonable assistance and full information with respect to such claim. Such indemnity shall include all costs and expenses, including reasonable attorneys’ fees and any costs of settlement.”

9. Sections 9.1 and 9.6 of ARTICLE 9 - TERMINATION of the License Agreement are replaced in their entirety with the following new Sections 9.1 and 9.6:

- 9.1 INSERM-TRANSFERT grants to LICENSEE a right to unilaterally terminate the Agreement (as amended) three (3) months after execution of the Agreement; in such case, the issue fee, including the Amendment Fee provided for in Amendment No 1 and the Clinical Data access fee mentioned hereabove shall not be refundable to LICENSEE. No other monies will be due to INSERM-TRANSFERT.
- 9.6 Articles 7, 8, and 10.7 shall survive the expiration and any termination of this License Agreement (as amended). Further, in the event of a Partial Termination by LICENSEE pursuant to Section 2.2 (b) (ii), the rights of LICENSEE to specified in such Section 2.2(b) (ii) shall survive such termination. Except as otherwise provided in this Article 9, all rights and obligations of the Parties under this License Agreement (as amended) shall terminate upon the expiration or termination of this License Agreement (as amended).

10. The following new Article, ARTICLE 11 - CLINICAL DATA ACCESS, is added to the License Agreement:

**“ARTICLE 11 - CLINICAL DATA ACCESS**

11.1 Scope of granted access: INSERM, through the Laboratory, shall provide LICENSEE with the Clinical Data for use for the LICENSEE Purpose only, during the Clinical Data Term and in accordance with the terms of the exclusive license to Patents and Know-How granted to LICENSEE under the License Agreement to the extent they do not conflict with the specific provisions contained in this Amendment No 2, in which case, the latter shall prevail. INSERM retains full ownership of the Clinical Data, no ownership right or any other license being transferred to LICENSEE other than as specifically contemplated in the License Agreement and this Amendment No 2. INSERM-TRANSFERT and/or INSERM and/or Investigator (as defined below) shall be free to publish the Clinical Data. All Trial Subject medical records and other source documents of INSERM will remain the property of INSERM.

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11.2 Investigator/Compliance: (i) Dr. Patrick Aubourg is experienced in the conduct of clinical research studies in humans and will personally conduct and supervise the INSERM Trial (the “**Investigator**”). INSERM represents and warrants that INSERM and the Investigator will conduct the INSERM Trial in accordance with (a) Applicable Law, (b) the Clinical Protocol, and (c) the terms of this Amendment No 2. INSERM is the regulatory sponsor of the INSERM Trial and will fulfill, or cause the fulfillment of, all responsibilities of a regulatory sponsor.

(ii) Should Dr. Patrick Aubourg no longer be able to perform his duties as Investigator of the INSERM Trial, INSERM-TRANSFERT shall inform LICENSEE within ten (10) business days and the Parties shall together seek in good faith a solution of replacement. Should no mutually acceptable replacement be found within three (3) months of INSERM-TRANSFERT’s information notice, INSERM will freely appoint a replacement for Dr. Patrick Aubourg who will be responsible for the remaining of the INSERM Trial .

11.3 Approvals: INSERM represents and warrants that INSERM has obtained or will obtain, and maintain, written approval from ANSM, the Comité de Protection des Personnes (CPP), the appropriate ethics committee (the “**EC**”) and any other relevant regulatory authorities (a) for the conduct of the INSERM Trial and (b) the relevant informed consent form (which form includes an express authorizing for the transfer of Clinical Data to countries and parties outside the European Union) to be signed by all Trial Subjects (or their legal representatives) (together with any amendments thereto, the “**Informed Consent Form**”),.

11.4 Database: LICENSEE will be responsible for building and maintaining, at its own cost, the Database. LICENSEE will own the Database, without prejudice to INSERM’s ownership of the Clinical Data, and provide the Database to INSERM for use in the INSERM Trial.

[\*\*\*]

11.5 Transfer Authorizations: INSERM-TRANSFERT and INSERM agree that, subject to approval of the Informed Consent Form by the relevant regulatory authorities, they will include express language in such Informed Consent Form specifying that the Trial Subject authorizes transfer and use of his/her personal data contained in the Clinical Data to third parties located in countries outside the European Union, in particular the United States.

Provided that INSERM, INSERM TRANSFERT and Investigator have used diligent efforts and acted in good faith to obtain signed Informed Consent Forms from Trial Subjects and regulatory approvals for the transfer to and use of Clinical Data by LICENSEE as contemplated herein, INSERM, INSERM TRANSFERT and/or Investigator cannot be held liable (i) in case the Trial Subjects (or their legal representatives) do not execute the Informed Consent Form and/or (ii) in case the prior regulatory authorization is not obtained for the transfer and use of the Clinical Data and/or in case, as a consequence of (i) or (ii), INSERM is not in the capacity to provide LICENSEE with Clinical Data. In such a case, this Amendment 2 shall be immediately terminated, without INSERM and/or INSERM TRANSFERT and/or Investigator being liable for any damages or other kind of compensation. For clarity, in such event, no Clinical Data Access Fees shall be due to INSERM and/or INSERM TRANSFERT from LICENSEE under Section 5.1.2.

11.6 [\*\*\*]

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11.7 Project Plan and Use of Clinical Data:

(a) INSERM and Investigator will collect the specified data as defined in the Clinical Protocol onto a source document and will perform entry of Clinical Data into the Database as set forth in the Project Plan.

LICENSEE agrees that the Clinical Data that is not Published Clinical Data:

(i) is to be used by LICENSEE or by approved subcontractors solely in accordance with the terms of the License Agreement and this Amendment No 2; and the International Data Transfer Agreement (as defined below).

(ii) will not be released by LICENSEE to any third parties or entities except as permitted under the License Agreement and this Amendment No 2 and provided that LICENSEE guarantees that said third parties and entities (including Affiliates) to which Clinical Data that is not Published Clinical Data is released are subject to obligations regarding the transfer and confidentiality of personal data that are at least as stringent as those imposed on LICENSEE under the License Agreement, this Amendment No 2 and the International Data Transfer Agreement and remains liable towards INSERM and/or INSERM TRANSFERT and/or Investigator for compliance by said third parties and entities of such obligations; and

(iii) is to be used by LICENSEE in compliance with applicable laws and regulations.

If INSERM, INSERM-TRANSFERT and/or Investigator receive a request from a Trial Subject and/or the French competent authorities to obtain a list of all third parties to which Clinical Data have been released by LICENSEE in accordance with the terms of the License Agreement and this Amendment No 2, LICENSEE undertakes to provide such list within seven (7) business days of receiving a written request for such a list from INSERM, INSERM-TRANSFERT and/or Investigator.

LICENSEE warrants that, without prejudice to Section 11.6 it shall take no action to identify any Trial Subject.

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(b) The contribution of INSERM and the Laboratory will be reflected expressly in all written or oral public disclosures within the framework of the LICENSEE Purpose. LICENSEE is not authorized to publish or present (i) the Clinical Data that is not Published Clinical Data or (ii) the Summary Clinical Data (other than as authorized under this Agreement), without prior written authorization by INSERM and the Investigator. Absent any answer or objection of INSERM and/or Investigator within sixty (60) days of submission of an intended publication or presentation by LICENSEE to INSERM and Investigator, INSERM and Investigator shall be deemed to have agreed to such publication or presentation.

**ARTICLE 11 - 11.8.** The following general terms will apply to this Amendment No 2:

Subject to the following paragraph, this Amendment No 2 shall be deemed executed and enter into effect on the latest date of signature by either of the Parties (the “**Signature Date**”).

Notwithstanding the above, the transfer to LICENSEE of the Clinical Data and use by LICENSEE of the Clinical Data shall only intervene and Article 11.2(i) is only applicable after the date the latest of the following conditions precedent has been completed (the “**Clinical Data Transfer Effective Date**”):

- (a) Agreement between INSERM and LICENSEE on the terms of an international data transfer agreement (the “**International Data Transfer Agreement**”), which shall contain terms similar to that of the Standard contractual clauses for the transfer of personal data from the Community to third parties as defined in the Commission decision 2004/915/EC,
- (b) The prior authorization of the relevant regulatory authorities for the transfer of the Clinical Data to LICENSEE and use for the LICENSEE Purpose on the basis of the draft of International Data Transfer Agreement which INSERM and LICENSEE shall have agreed upon beforehand. The Parties agree to modify the negotiated draft of the International Data Transfer Agreement and, if applicable, the provisions of this Amendment No 2 which were incorporated in the International Data Transfer Agreement, as reasonably appropriate, so as to comply with the decision and potential requirements for amendments of the relevant regulatory authorities, and
- (c) The execution of the International Data Transfer Agreement and, if applicable of an amendment to the present Amendment No 2 as provided under paragraph (b) above, and
- (d) INSERM obtaining the approval of the Informed Consent Forms containing the transfer authorization language described in paragraphs 11.3 and 11.5 above by the competent regulatory authority and EC, and execution of at least one Informed Consent Form by a Trial Subject (or his/her legal representative), and
- (e) INSERM obtaining the approval of the competent regulatory authorities, especially on the Clinical Protocol.

Should one or several of the above conditions precedent fail to be completed within twelve (12) months from the Signature Date at the latest, then this Amendment No 2 shall be immediately terminated, without INSERM and/or INSERM TRANSFERT and/or Investigator being liable for any damages or other kind of compensation. For clarity, in such event, no Clinical Data access fees shall be due to INSERM and/or INSERM TRANSFERT from LICENSEE under Section 5.1.2.

It is specified that in the case not all four (4) Trial Subjects (or their legal representatives), but at least one Trial Subject (or his/her legal representative), execute the Informed Consent Form, the Clinical Data access fees provided under Section 5.1.2 shall be prorated based on the number of Trial Subjects having executed the Informed Consent Form.

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This Amendment No 2 shall be effective for the term of the License Agreement, it being however specified that, other than as expressly stated below, neither INSERM nor INSERM-TRANSFERT nor Investigator shall be liable in case of (i) completion, abandonment or termination of the INSERM Trial, whether decided by INSERM or resulting from the fact that INSERM is no more authorised by the relevant French authority to perform the INSERM Trial (and notably to perform the Gap Period and/or the Follow-Up Period) or if the performance of the INSERM Trial is suspended by the said authority or (ii) in case one or several Trial Subjects later withdraw(s) its (their) consent(s).

In the case of the foregoing (i) or (ii), the terms of Amendment No 2 governing the transfer and use of the Clinical Data shall only apply to Clinical Data collected until the INSERM Trial is terminated or the consent(s) is withdrawn and (a) any future payments due from LICENSEE pursuant to subsection 5.1.2 of Article 5 for access to Clinical Data shall be prorated based on the amount of Clinical Data received by LICENSEE, and (b) the rights and obligations of LICENSEE under this Amendment No 2 to use Clinical Data already received shall continue, unless applicable law or relevant regulatory authorities forbid any use of already collected Clinical Data. Notwithstanding the foregoing, to the extent Clinical Data from a Trial Subject has already been transferred to LICENSEE and used for the LICENSEE Purpose, LICENSEE shall not have to remove Clinical Data from any regulatory filings.

For clarity, to the extent this Agreement imposes restrictions on use and disclosure of Clinical Data, such restrictions shall not apply to Clinical Data that is Published Clinical Data.

The use and access by LICENSEE to the Clinical Data that is not Published Clinical Data shall automatically terminate at the earliest of the following two dates:

- At the expiry of the Clinical Data Term; or
- in case of early termination as provided under Article 2.2.

Except as specifically set forth in this Amendment No 2, all provisions of the License Agreement shall remain in full force and effect.

In witness whereof, the Parties have executed this Amendment No 2 in English by their respective duly authorized representatives on the date indicated below in two (2) copies, one (1) for each Party.

**INSERM-TRANSFERT**

Signature /s/ Cécile Tharaud

Name: Cécile Tharaud  
Title : CEO  
Date : 3/15/13

**bluebird bio, Inc.**

Signature /s/ Nick Leschly  
*(Authorized signatory of the company)*

Name: Nick Leschly  
Title: CEO  
Date : 3/11/13



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**EXHIBIT A**  
**DESCRIPTION OF DATABASE CONTENT (eg categories of data collected)**

[\*\*\*]

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**EXHIBIT B**  
**PROJECT PLAN**

[\*\*\*]

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**EXHIBIT C**  
**LIST OF WHAT SUMMARY CLINICAL DATA SHALL CONTAIN**

[\*\*\*]

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### LICENSE AGREEMENT

Between

**INSTITUT PASTEUR**, a non profit private foundation organized under the laws of France with offices at 25-28 rue du Docteur Roux, 75 724 Paris Cedex 15, France, VAT FR 65 775 684 897, represented by M. Christophe Mauriet, Senior Executive Vice-President for Administration, and M. Jean Derégnacourt, Executive Vice President Business Development

Hereinafter referred to as “Institut Pasteur”

On one hand,

And

**BLUEBIRDBIO INC.**, a company incorporated under the laws of Massachusetts, with offices at 840 Memorial Drive, Cambridge, MA 02139, United States, represented by Nick Leschly, Chief Executive Officer

Hereinafter referred to as “Licensee”,

On the other hand,

Hereinafter mentioned as a Party or the Parties.

#### Recitals

1. Institut Pasteur has identified and patented a specific nucleotide sequence having a triplex structure, hereinafter referred to as “DNA flap”, covered by patents and patent applications.
2. Institut Pasteur has granted several exclusive or non exclusive licenses on the DNA flap under several fields to companies.
3. Licensee is a company developing innovative gene therapies for severe genetic disorders.
4. Licensee wishes to obtain a license of such patents and commercialize products for gene therapy.
5. Licensee and Institut Pasteur have decided to discuss terms of a license agreement according to the terms and conditions of this Agreement.

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**Now, therefore, the Parties hereby agree as follow:**

**Article 1. Definitions**

For the purpose of this Agreement, the terms used in this Agreement, in singular or in plural, shall have the respective meanings set forth below:

- “Affiliate” means with respect to Licensee any party which (directly or indirectly) is controlled by, controls, or is under common control with, Licensee. For the purposes of this definition, the terms “control” and “controlled” mean the direct or indirect ownership of more than fifty percent (50%) of the outstanding voting securities of an entity, or such other relationship as results in actual control over the management, assets, business and affairs of such entity.
- “Agreement” shall mean this license agreement together with its appendices which make integral part of it.
- “Confidential Information” shall mean any and all confidential information, whatever its nature or its format, which is disclosed by one Party to the other Party hereunder and that is marked confidential or with similar term, if disclosed in writing, or if disclosed orally, identified as confidential at the time of disclosure. Notwithstanding the foregoing, any information which, by its nature and under the circumstances surrounding its disclosure is generally considered proprietary and confidential shall be deemed Confidential Information regardless of whether it is properly marked with legends or properly reduced to writing.
- “Development Plan” shall mean a document defining the research and development of the Licensee and/or Affiliates as well as the commercial and financial development estimates of Licensee and/or Affiliates for its Product(s) using the Technology in the Field.
- “Effective Date” shall mean the date of the last signature of this Agreement by the Parties.
- “Ex vivo gene therapy” shall mean that cells are extracted from a patient, corrected by placing a healthy or functional gene(s) and transplanted back into patient.
- “Field” shall mean ex vivo gene therapy for human disorders limited to adrenoleukodystrophy (ALD) (including but not limited to AMN, CCALD, and all other variants of this disease caused by genetic mutations), beta hemoglobinopathies (including but not limited to beta-thalassemia and sickle cell anemia), [\*\*\*], the “Field” includes in vivo as well as ex vivo gene therapy. Licensee and/or Affiliates shall have the right to request Institut Pasteur to expand the definition of “Field” to include additional clinical areas. [\*\*\*] For clarity, the Field excludes any other fields and specifically prophylactic and therapeutic human and veterinary vaccination against all kind of pathogens, and the field of services of production and commercialisation of Good Manufacturing Practice (GMP) batches of lentiviral vectors for clinical trials. For clarity, the Field shall include production of GMP batches, by Licensee and its Affiliates [\*\*\*]. For clarity, Institut Pasteur has already granted exclusive rights for services of production and commercialisation of Good Manufacturing Practice (GMP) batches of lentiviral vectors for clinical trials. [\*\*\*]
- “Improvement” shall mean any new invention, patentable or not, patented or not, of the Technology, which under applicable law, depends on, at least, one claim of the Patents. For clarity, an Improvement does not include any Product.

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- “Net Sales” shall mean the gross amount, excluded taxes, invoiced for sale of Products manufactured or sold in the Territory, in finish or semi finish form by Licensee and/or Affiliates less the following items, consistent with U.S. GAAP:
  - a) trade, quantity and cash discounts actually allowed;
  - b) commissions, discounts, refunds, rebates, charge backs, retroactive price adjustments, and any other allowances paid to non-governmental Third Parties that effectively reduce net selling price;
  - c) credits, allowances and refunds for actual Product rejections, returns and allowances;
  - d) taxes, duties and other governmental charges on the sale, shipment or transfer of the Product; and
  - e) duly justified governmental discounts, refunds, rebates, charge backs, retroactive price adjustments and any other allowances that effectively reduce net selling price.

It is understood that deductions set forth in a), b) and c) herein above shall not exceed [\*\*\*] of gross revenue, excluded taxes, invoiced for sale of Products in the Territory.

- “Patents” shall mean the patents and patents applications listed in Appendix 1, along with all other patent rights (including but not limited to continuations, continuations-in-part (but only for those claims of such continuations-in-part that are fully supported by the patents and patent applications listed in Appendix 1 as of the Effective Date), divisionals, renewals, reissues, re-examinations, patent term extensions) that claim priority in whole or in part to any such patents and patent applications.
- “Product” shall mean all composition or product for gene therapy or method in the Field that incorporate the Technology.
- “Rare Diseases” shall include adrenoleukodystrophy (ALD) (including but not limited to AMN, CCALD, and all other variants of this disease caused by genetic mutations), beta hemoglobinopathies (including but not limited to beta-thalassemia and sickle cell anemia), [\*\*\*]. Whether in the case of [\*\*\*], such diseases shall be considered on a case-by-case basis and considered a Rare Diseases if the incidence or prevalence is similar to those diseases listed as Rare Diseases above. In these situations, Licensee and/or Affiliates shall provide justification as to whether such disease is a Rare Disease, in writing, prior to payment of the Milestone #4 or #5 as applicable.
- “Technology” shall mean lentivirus vector containing DNA flap sequence covered by whole or part of the claims of the Patents.
- “Territory” shall mean [\*\*\*].
- “Third Party” shall mean any party which is not Institut Pasteur or Licensee or its Affiliates.

## **Article 2. Scope**

2.1. Under this Agreement, Institut Pasteur grants to Licensee and its Affiliates, that Licensee and its Affiliates accepts at their own risks, a license under the Patents in the Field and in the Territory for research and development, and to manufacture, have manufactured, make, have made, use, have used, sell, have sold, offer for sale, have offered for sale, import or have imported the Products, to the exclusion of any other rights, which is:

- exclusive for Products containing human (HIV-1 and HIV-2) lentivirus vector, and ;
- non exclusive for Products containing non-human lentivirus vector.

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2.2. No right granted herein shall prevent Institut Pasteur or its licensees or research partners to conduct research in the Field.

2.3. This Agreement includes the right for Licensee and/or Affiliates to grant sublicenses in the Field and the Territory to and through multiple tier(s) of Third Party(ies).

### **Article 3. Licensee’s obligation**

3.1. Licensee shall make, or shall cause its Affiliates and sublicensee to make, all reasonable commercial efforts (by reference to a company of similar size and scope to Licensee as of the Effective Date) to develop and commercialize one or more Products in the Field and to obtain any necessary governmental approvals in respect of, and market the Product(s) in the Field, if any. It is expressly agreed that fulfillment of the above obligation is an absolute requirement for this Agreement to be maintained into force.

3.2. Licensee shall provide annually, upon each anniversary date of the Effective Date, to Institut Pasteur an updated Development Plan, which will be Confidential Information of Licensee. [\*\*\*]

3.3. Licensee is the sole responsible for securing the compliance of Products with applicable laws, rules and regulations, in particular, but without limitation, such as relating to ethics, the treatment of animals, and genetically modified organisms, if any.

### **Article 4. Intellectual property**

4.1. The provisions of this Agreement shall not modify the ownership of the Technology and Patents.

4.2. Any Improvement of the Technology made without Institut Pasteur by the Licensee shall belong to Licensee.

4.3. Any Improvement of the Technology made with the help of Institut Pasteur will be co-owned by Institut Pasteur and Licensee. A specific agreement shall be established between the co-owners within six (6) months following the identification of the joint Improvement.

4.4. Upon request of Institut Pasteur, Institut Pasteur and Licensee agrees to meet in view to determine the conditions under which Licensee shall grant Institut Pasteur, a non exclusive, free license on the Improvement if possible and available mentioned in articles 4.2 and 4.3 above, for internal research purpose. Licensee and/or Affiliates shall ask to Institut Pasteur to submit a supplementary protection certificate (SPC) for any Product. To this aim, Licensee shall provide Institut Pasteur all necessary information. This SPC shall automatically be part of the Agreement.

4.5. From the Effective Date, Licensee shall pay to Institut Pasteur [\*\*\*] of future external expenses engaged by Institut Pasteur for securing issuance of, and maintaining Patents or extending the duration of the Patents. Institut Pasteur shall not abandon any Patent without the prior notice of Licensee.

4.6. Upon written request, but at most once a year, Institut Pasteur shall keep Licensee informed of the status of issuance procedures of Patents, and shall update Appendix 1 accordingly.

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4.7. Licensee acknowledges that Institut Pasteur has expended significant resources and efforts to develop the Patents, and that the Patents represents highly valuable interests, [\*\*\*].

4.8. In the case a Patent is challenged (including but not limited to a re-examination, opposition or interference proceeding, but not including when part of an infringement action described above), the Parties shall make available to the other all information they have, and shall meet to decide the defense strategy. [\*\*\*]

**Article 5. Infringement**

5.1. Institut Pasteur and Licensee shall as soon as they become aware thereof mutually advise each other of any infringement of Patents by a Third Party in the Field. Institut Pasteur and Licensee shall make available to the other all information at their disposal on the basis of which nature and extend can be assessed.

5.2. [\*\*\*]

5.3. In the case Licensee is sued by a Third Party regarding Technology in a Product, the Parties shall make available to the other all information they have, and shall meet to decide the defense strategy, if any, with respect to such Technology.

**Article 6. Consideration**

6.1. Within thirty (30) days of the Effective Date, Licensee shall pay to Institut Pasteur a one-time, non-refundable license issuance fee [\*\*\*] exclusive of taxes. This amount cannot be set-off against future royalties.

6.2. For the development of each Product indication by indication, except in the case mentioned below, Licensee and/or Affiliates shall pay to Institut Pasteur the following milestones:

	[***]	[***]	[***]	[***]
<b>Milestone 1:</b> [***]	[***]	[***]	[***]	[***]
<b>Milestone 2:</b> [***]	[***]	[***]	[***]	[***]
<b>Milestone 3:</b> [***]	[***]	[***]	[***]	[***]
<b>Milestone 4:</b> [***]	[***]	[***]	[***]	[***]
<b>Milestone 5:</b> [***]	[***]	[***]	[***]	[***]
<b>Milestone 6:</b> [***]	[***]	[***]	[***]	[***]

For the foregoing table:

- [\*\*\*]



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- For each Product (including multiple indications for the same Product), only one column is applied and each milestone in such column is paid only once at the first occurrence of such event
- [\*\*\*]
- [\*\*\*]
- [\*\*\*]
- [\*\*\*]
- [\*\*\*]

For further clarity, with respect to the second tabbed paragraph above, if the same Product is developed or approved for more than one indication, the specified milestones for a column shall be paid one time only at the first occurrence of such event. As a specific example, [\*\*\*].

6.3. Until the expiration of the last Patent claiming a Product in the Territory, Licensee and/or Affiliates shall pay to Institut Pasteur the following yearly royalty fees:

- [\*\*\*] of Net Sales for a Product with an indication in a Rare Disease, without stacking clause, and
  - [\*\*\*] of Net Sales for a Product with an indication in a disease other than a Rare Disease, without stacking clause;
- [\*\*\*].

6.4. If the combined royalties Licensee and/or Affiliates would be required to pay to Institut Pasteur and Third Parties, is higher than [\*\*\*] for one Product, Licensee and/or Affiliates may ask Institut Pasteur to negotiate the royalty fees of the article 6.3.

6.5. Licensee and/or Affiliates shall pay to Institut Pasteur a minimum annual fee [\*\*\*] exclusive of taxes per twelve (12) month period and due at the end of such period which shall start from the fifth anniversary of the Effective Date of this Agreement for all Products. For clarity, such payment shall be offset by the royalties payments made to Institut Pasteur during such 12 month period. If no Product is on the market after the fifth anniversary of the Effective Date, this minimum annual fee shall be reduced [\*\*\*] exclusive of taxes until the first Product shall be on the market, date on which the minimum annual fee shall be again [\*\*\*] exclusive of taxes per twelve (12) month period.

6.6. Licensee and/or Affiliates shall pay to Institut Pasteur [\*\*\*] of all cash and cash-equivalent consideration, whatever its nature, and in particular without limitation, all sums, milestones, royalties, exchange value of any counterpart in kind or in industry (but not duly justified payments for research and development) received by Licensee and/or Affiliates from its all sublicenses agreements granted by Licensee and/or Affiliates on the sole Technology.

6.7. On a indication-by-indication basis, in case of sublicenses relating to a Product, Licensee and/or Affiliates shall pay to Institut Pasteur on any and all cash and cash-equivalent consideration, whatever its nature, and in particular without limitation, all sums, milestones, royalties, exchange value of any counterpart in kind or in industry (but not duly justified payments for research and development) received by Licensee and/or Affiliates from a sublicensee:

- [\*\*\*] if the sublicense is signed for Product(s) in a preclinical stage development, or,
- [\*\*\*] if the sublicense is signed for Product(s) in a clinical stage of development.

6.8. If the combined royalties Licensee and/or Affiliates would be required to pay to Institut Pasteur and Third Parties, is higher than [\*\*\*] for one Product, Licensee and/or Affiliates may ask Institut Pasteur to negotiate the royalty fees of the article 6.7.

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6.9. Notwithstanding the foregoing, in the case of a sublicense of the Technology for a Product for ALD (including but not limited to AMN, CCALD, and all other variants of this disease caused by genetic mutations), beta-thalassemia and/or sickle cell anemia, Institut Pasteur shall receive the same Milestones and Royalties as if Licensee and/or Affiliates itself were developing and commercializing such Product(s) (and thus no amounts shall be payable to Institut Pasteur under Article 6.7). Licensee and/or Affiliates shall be liable for ensuring such payment in accordance with the terms of this Agreement.

#### **Article 7. Payment**

7.1. Payment of royalties due under this Agreement shall be made within forty-five (45) days from the invoice date, after the end of each six-month period's Net Sales (ending 30 June and 31 December) for the sum corresponding to that period.

7.2. Any payment due by Licensee, pursuant to this Agreement, shall be made in euros by check or by wire transfer to a bank account as designated by Institut Pasteur from time to time.

7.3. Royalties arising out of Net Sales achieved in currencies other than the Euro shall be converted at the current average exchange rate one month prior to the date upon which the royalties report is due, and shall be borne by Licensee.

7.4. Notwithstanding the provisions of this Agreement, sums paid to Institut Pasteur shall in any event be retained by Institut Pasteur. Any VAT (Value Added Tax) due, if any, shall be added to the invoiced amount at the then current rate, and shall be borne by Licensee.

7.5. Any withholding tax payable by Licensee on royalties due hereunder shall be deducted from royalties due for the relevant country. Licensee shall be responsible for obtaining and providing to Institut Pasteur evidence of the payment of such withholding taxes. Licensee shall assist Institut Pasteur to prevent any double taxation and shall provide Institut Pasteur on request with any document necessary to that end.

7.6. The royalties and other payments set forth in this Agreement shall, if overdue, bear interest until paid at a per annum rate of [\*\*\*]. The payment of such interest shall not foreclose Institut Pasteur from exercising any other rights or actions it may have as a consequence of the lateness of any payment.

#### **Article 8. Accounts**

8.1. Licensee shall simultaneously with payments of royalties deliver to Institut Pasteur a report reflecting its accounts and sub-licenses accounts, pertaining to royalties calculated, on Net Sales, including:

- [\*\*\*];
- [\*\*\*]; and
- [\*\*\*].

Such report maybe delivered by email to the following email address (which address may be updated by written notice from Institut Pasteur to Licensee):  
Service de Transfert de Technologie, [\*\*\*].

8.2. When no royalty is payable, a report so attesting shall be submitted to Institut Pasteur. The aforesaid reports shall be treated as Confidential Information of Licensee.

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Such report maybe delivered by email to the following email address (which address may be updated by written notice from Institut Pasteur to Licensee): Service de Transfert de Technologie, [\*\*\*].

8.3. Licensee shall keep complete and accurate records of all Net Sales, allowing a computation and checking of the royalty amount due to Institut Pasteur hereunder. Once a year and upon prior notice to Licensee and/or Affiliates, Institut Pasteur shall, throughout the term of this Agreement, and for a period of three (3) years following the end of this Agreement, be entitled to have at its own expense and during regular business hours Licensee’s records pertaining to this Agreement checked by an independent certified public accountant chosen by Institut Pasteur and reasonably acceptable to Licensee, which accountant shall enter into a confidentiality agreement with Licensee. Such accountant shall be appointed for the sole purpose of determining the amount of royalties due to Institut Pasteur hereunder, covering a period not to exceed the past three (3) years, provided that [\*\*\*] such accountant shall report to Institut Pasteur only as to the accuracy of royalty statements and payments and that such reported information shall be considered to be Confidential Information of Licensee.

8.4. If, as a result of such audit, an adjustment is determined to be made in favor of Institut Pasteur, the accountant’s fees and expenses shall be borne by Licensee, if the sums underpaid by Licensee exceed [\*\*\*] of what was actually paid by Licensee to Institut Pasteur; otherwise such fees and expenses shall be paid by Institut Pasteur. Licensee shall pay any underpaid royalties to Institut Pasteur.

#### **Article 9. Confidentiality**

9.1. Confidential Information does not include information for which it is evidenced that:

- is publicly known and made generally available in the public domain prior to the time of disclosure by the providing Party,
- becomes publicly known and made generally available after disclosure by the providing Party to the receiving Party through no action or inaction of the receiving Party,
- is already in the possession of the receiving Party at the time of disclosure by the providing Party as shown by the receiving Party’s documentary evidence,
- is obtained by the receiving Party from a Third Party without breach of such Third Party’s obligations of confidentiality, as shown by the receiving Party’s documentary evidence,
- is required by law to be disclosed by the receiving Party.

9.2. In the event that the receiving Party is notified of a requirement to disclose the providing Party’s Confidential Information, the receiving Party shall notify the providing Party immediately upon receipt of such notice and not release the Confidential Information until such time as the providing Party has taken reasonable steps to seek an order of a court of competent jurisdiction to prevent the disclosure, or limit the extent of disclosure, of the providing Party’s Confidential Information.

9.3. During the term of this Agreement and five (5) years thereafter, the receiving Party agrees to keep confidential and cause its employees, consultants or students to keep confidential, all Confidential Information of the providing Party that is disclosed to it, or to any of its employees, consultants or students under or in connection with this Agreement.

9.4. Neither the receiving Party nor any of its respective employees, consultants or students, shall use Confidential Information for any other purpose whatsoever except as expressly permitted by this Agreement.

9.5. The receiving Party may not disclose providing Party’s Confidential Information to a Third Party without the prior written consent of the providing Party, other than for Licensee and/or Affiliates in connection with a proposed or actual sublicense or transaction permitted by Article 13.7 or for other reasonable business purposes, subject to the confidentiality protections stated above, for the purpose of this Agreement.

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9.6. Following expiration or termination of this Agreement, the receiving Party shall return all the Confidential Information to the providing Party, or destroy such Confidential Information at the providing Party request, with the exception that (1) one copy of the Confidential Information that may be retained by the receiving Party’s legal counsel for the purpose of verifying its obligations under this Agreement.

#### **Article 10. Representations and Warranties**

10.1. At the Effective Date, each Party represents and warrants to the other Party that it has the right to enter into this Agreement.

10.2. Licensee agrees that all Confidential Information or any other information or data communicated or provided by Institut Pasteur under this Agreement are communicated “as is”, without any warranty, expressed or implied, regarding accuracy, completeness, merchantability, fitness, patentability and/or performance. Any hazards, costs and risks that may be incurred by Licensee in connection with the use of all or part of the Products, resulting, in particular, from possible defects or from the eviction risk, are the sole responsibility of Licensee. Institut Pasteur shall not be liable for any consequential, indirect or punitive damages or lost profits of Licensee.

10.3. Institut Pasteur gives no warranty whatsoever express or implied, in respect of the Patents, in particular as regards of its usefulness, safety or fitness for a particular purpose. Institut Pasteur does not, either expressly or tacitly warrant that the use of the Patents granted under this Agreement shall allow the production of Product, as well as the manufacture, sale, use, importation, exportation and holding of Products shall not infringe a Third Party’s intellectual proprietary rights or violate any rights in particular license rights, already granted to a Third Party. Licensee undertakes not to enforce any remedy, including a claim under any guarantee against Institut Pasteur, for compensation of whatever damage which might arise out of or in connection with the use or non use of the Patents.

10.4. Nothing in the Agreement shall be construed as: (a) a warranty or representation by Institut Pasteur as to the validity or scope of any Patents; (b) a warranty or representation by Institut Pasteur that the practice under the Patents is or will be free from infringement of patents of any Third Party or rights granted to Third Party; (c) except as expressly set forth herein, an obligation to Institut Pasteur to sue Third Party for infringement; or (d) conferring by implication, estoppels or otherwise any license, immunity or right under any patent owned by or licensed to Institut Pasteur other than the Patents.

10.5. Institut Pasteur shall under no circumstances be held liable to Licensee, whether expressly or impliedly, for any direct, indirect, consequential or special damages in relation to the use or sale of Patents and/or Products by Licensee. Licensee shall indemnify and hold Institut Pasteur harmless from all costs and expenses of any kind, arising from or resulting of any Third Party claim against Institut Pasteur relating to the use, handling or storage by Licensee of the Patents or Confidential Information, as well as the manufacture, sale, use, importation, exportation and holding of Product, except where such claims arise from a finding of gross negligence or willful misconduct by Institut Pasteur only with respect to the Patents or Confidential Information, to the exclusion of Products. [\*\*\*]

10.6. Institut Pasteur may terminate this Agreement with immediate effect in the event that Licensee, either directly or indirectly, or its Affiliates challenges the validity of any of the Patents.

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**Article 11. Term and Termination**

11.1. This Agreement shall be effective from the Effective Date.

11.2. Unless sooner terminated under the articles below, this Agreement shall be effective until the last Patents to expire in the Territory.

11.3. This Agreement may be terminated without any indemnification, by either Party at any time during this Agreement if the other Party is in substantial breach of its obligations hereunder and has not cured such breach within sixty (60) days after a registered letter notifying such substantial breach, without prejudice of any right to pursue an action for damages as a result of such breach.

11.4. Institut Pasteur may also terminate this Agreement without fault where collective proceedings – bankruptcy, suspension of proceedings – are opened against Licensee and not dismissed within sixty (60) days thereafter.

11.5. Licensee may terminate this Agreement by a written notice sent ninety (90) days in advance.

11.6. Termination of this Agreement for any reason shall not affect each Party’s continuing obligations to the other Party under this Agreement or pursuing provisions. Upon termination of this Agreement, as long as there are always unexpired Patents under the Territory, this license shall automatically terminate and Licensee shall promptly cease any use of the Patents and shall cease manufacturing, importing, using and selling Products within [\*\*\*] form the effective date of the termination.

11.7. Upon termination of this Agreement, Institut Pasteur shall have the right to retain any sums already paid by Licensee hereunder, and Licensee shall pay all sums accrued hereunder which are then due, including all sums generated during the three month period mentioned in Article 11.6 of this Agreement.

11.8. Articles 1, 3.3, 4.1 to 4.3, 7 to 10, 11.6, 11.7, 11.8 and 12 shall survive any termination or expiration of this Agreement.

**Article 12. Litigation and governing law**

12.1. This Agreement shall be construed and governed by the Laws of France. The language of this Agreement shall be English.

12.2. The Parties shall attempt to settle any dispute relating to this Agreement, its validity and/or its interpretation and/or its enforceability and/or its termination, in an amicable way. Should such attempts fails, the litigation will be held in the court of the competent jurisdiction in France.

**Article 13. Miscellaneous**

13.1. This Agreement contains the entire understanding and agreement between the Parties hereto with respect to its subject matter, and except where otherwise provided herein, supersedes any prior or contemporaneous written or oral agreement between them relating to the subject matter hereof.

13.2. The Parties agree to keep the existence and the terms and conditions of this Agreement strictly confidential, and shall not disclose the existence and the terms and conditions of this Agreement to any Third

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Party, except as required by law (including but not limited to in connection with a public securities offering) or by Licensee in connection with a proposed or actual sublicense or transaction permitted by Article 13.7 or for other reasonable business purposes. Moreover, nothing contained in this Agreement shall grant to Licensee a right to use for advertising, publicity or any promotional activity whatsoever Institut Pasteur’s names, trademarks, logo or any other designations, including in contracted or abbreviated form or by imitation, subject to a prior express written consent of Institut Pasteur. Notwithstanding the foregoing, Licensee may disclose the existence of this Agreement and the fact that Institut Pasteur has granted an exclusive license under the Patents to Licensee.

13.3. This Agreement may be amended only by a written amendment signed by the Parties.

13.4. If any term, provision or condition of this Agreement shall be held by a court of competent jurisdiction to be invalid, unenforceable or void, the remainder of this Agreement shall remain in full force and effect.

13.5. Any notice required or permitted to be given under this Agreement shall be sufficient if sent by commercial courier or certified mail (return receipt requested), facsimile, or postage prepaid, addressed to the address mentioned in first page of this Agreement.

13.6. Neither Party shall be liable to the other for any default under this Agreement caused by war, riot, fire, flood, drought, act of God or any other cause which is beyond the reasonable control of the defaulting Party, as acknowledged by the court of competent jurisdiction.

13.7. This Agreement being entered into for the benefit of consideration of the Parties, shall not be assigned or transferred, whether in whole or in part, without the other Party’s prior written consent; provided that Licensee may assign this Agreement to an Affiliate or in connection with the merger, consolidation, or sale of all or substantially all of its assets or that portion of its business pertaining to the subject matter of this Agreement, and the assignee shall notify Institut Pasteur of such assignment and shall agree in writing to be bound to the terms of this Agreement as “Licensee” hereunder.

13.8. The relationship created by this Agreement shall be that of independent contractors.

13.9. The failure or neglect of a Party at any time, to require performance of the other Party of any provision hereof, shall not in any way affect the right to require such performance at any time thereafter. The waiver by a Party of any breach of any provision hereof shall not be held to be a waiver of any subsequent breach of the same provision or of any other provisions hereof.

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IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed by their authorized respective representative.

Made in Paris,  
In duplicate.

Date: 08 SEP. 2011

/s/ Christophe Mauriet  
INSTITUT PASTEUR  
Christophe Mauriet  
Senior Executive Vice-President

Date: 13-Sept 2011

/s/ Nick Leschly  
BLUEBIRDBIO INC.  
Nick Leschly  
Chief Executive Officer

Date: 26/8/11

/s/ Jean Derégnacourt  
INSTITUT PASTEUR  
Jean Derégnacourt  
Executive Vice-President Business Development

---

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Appendix 1

Patents

Invention  
[\*\*\*]

Priority/Filing date  
Extension/Filing date

Territories / Filing date  
Legal Status

Institut Pasteur hereby confirms that the foregoing is a complete and accurate list of all the Patents as the Date of August 11,2011.



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**AMENDMENT N°1 TO THE LICENSE AGREEMENT**

Between

**INSTITUT PASTEUR**, a non profit private foundation organized under the laws of France with offices at 25-28 rue du Docteur Roux, 75 724 Paris Cedex 15, France, VAT FR 65 775 684 897, represented by M. Christophe Mauriet, Senior Executive Vice-President for Administration

Hereinafter referred to as “Institut Pasteur”

On one hand,

And

**BLUEBIRDBIO INC.**, a company incorporated under the laws of Massachusetts, with offices at 840 Memorial Drive, Cambridge, MA 02139, United States, represented by Nick Leschly, Chief Executive Officer

Hereinafter referred to as “Licensee”,

On the other hand,

Hereinafter mentioned as a Party or the Parties.

**Recitals**

1. The Parties have signed a license agreement on September 13, 2011 on a patented specific nucleotide sequence having a triplex structure, referred to as “DNA flap”.
2. Institut Pasteur has granted several exclusive or non exclusive licenses on the DNA flap under several fields to companies, [\*\*\*] .
3. Institut Pasteur has negotiated with a licensee to obtain rights for the Licensee in this field of services of production and commercialization of Good Manufacturing Practice (GMP) batches of lentiviral vectors for clinical trials, according to the terms and conditions of this Amendment n°1.

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**Now, therefore, the Parties hereby agree as follow:**

**Article 1. Scope**

The scope of this Amendment n°1 is to extend the Field of the Agreement and the license grants by Institut Pasteur.

**Article 2. Modifications**

2.1. The definition of the Field in the article 1 of the Agreement is replaced by the following definition as from the effective date of this Amendment n°1:

- “Field” shall mean ex vivo gene therapy for human disorders limited to adrenoleukodystrophy (ALD) (including but not limited to AMN, CCALD, and all other variants of this disease caused by genetic mutations), beta hemoglobinopathies (including but not limited to beta-thalassemia and sickle cell anemia), [\*\*\*] for [\*\*\*] the “Field” includes in vivo as well as ex vivo gene therapy. [\*\*\*]

2.2. The article 2.1 of the Agreement is modified as follow, as from the effective date of this Amendment n°1:

“2.1. Institut Pasteur hereby grants to Licensee, its Affiliates, that Licensee, its Affiliates accept at their own risk, a license under the Patents in the Field and in the Territory for research and development, and to manufacture, have manufactured, make, have made, use, have used, sell, have sold, offer for sale, have offered for sale, import or have imported the Products, to the exclusion of any other rights, the said license being:

- exclusive for Products containing human (HIV-1 and HIV-2) lentivirus vector;
- nonexclusive for Products containing non-human lentivirus vector.

In addition, Institut Pasteur hereby grants to Licensee, its Affiliates and sublicensees, that Licensee, its Affiliates and sublicensees accept at their own risk, a nonexclusive license under the Patents in the Field and in the Territory to make or to have made by a Third Party Good Manufacturing Practice (GMP) batches of lentiviral vectors for its/their own clinical trials on Products, provided that such Third Party makes Good Manufacturing Practice (GMP) batches of lentiviral vectors solely for the Licensee, its Affiliates and sublicensees clinical trials of Products above mentioned.

2.3. The article 10.5 of the Agreement is modified as follow, as from the effective date of this Amendment n° 1: the last sentence of such article 10.5 is modified as follow: [\*\*\*]

**Article 3. Miscellaneous**

3.1. All the other provisions of the Agreement remain unchanged and fully applicable between the Parties.

3.2. This Amendment n°1 is effective from the date of signature by the Parties.

3.3. This Amendment n°1 makes integral part of the Agreement.

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IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed by their authorized respective representative.

Made in Paris,  
In duplicate.

Date: 27 AVR. 2012

/s/ Christophe Mauriet  
INSTITUT PASTEUR  
Christophe Mauriet  
Senior Executive Vice-President

Date:

/s/ Nick Leschly  
BLUEBIRDBIO INC.  
Nick Leschly  
Chief Executive Officer

---

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## AMENDMENT N°2 TO THE LICENSE AGREEMENT

Between

**INSTITUT PASTEUR**, a non profit private foundation organized under the laws of France with offices at 25-28 rue du Docteur Roux, 75 724 Paris Cedex 15, France, VAT FR 65 775 684 897, represented by M. Christophe Mauriet, Senior Executive Vice-President for Administration

Hereinafter referred to as “Institut Pasteur”

On one hand,

And

**BLUEBIRDBIO INC.**, a company incorporated under the laws of Delaware, with offices at 840 Memorial Drive, Cambridge, MA 02139, United States, represented by Nick Leschly, Chief Executive Officer

Hereinafter referred to as “Licensee”,

On the other hand,

Hereinafter mentioned as a Party or the Parties.

### Recitals

1. The Parties have signed a license agreement on September 13, 2011 on a patented specific nucleotide sequence having a triplex structure, referred to as “DNA flap”, modified by an amendment n°1 dated April 27, 2012 (the “Agreement”).
2. The Licensee has initiated a program to treat cancerous and/or pre-cancerous conditions by genetically modifying T cells to express antigen binding domain(s) on their surface that target tumor associated antigen(s).
3. Institut Pasteur agrees to extend the Field as follows, and the Parties agree to modify some definitions, according to the terms and conditions of this Amendment n°2.

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**Now, therefore, the Parties hereby agree as follow:**

**Article 1. Scope**

The scope of this Amendment n°2 is to extend the Field of the Agreement and the license granted by Institut Pasteur, and to make some modifications.

**Article 2. Modifications**

2.1. The following definitions shall replace the definitions of the Agreement:

- “Gene therapy” shall mean the use of a vector containing at least one DNA sequence that encodes at least one protein, in order to restore the functional activity of one or more resident non-functional gene copies, or provide for the introduction and expression of novel protein(s) not normally expressed in the cell type or expression of protein(s) that do not exist normally in nature. The introduced protein(s) are not intended to generate a prophylactic and/or therapeutic immune response against the protein encoded by the introduced DNA sequence of interest for use in Vaccination.
- “Ex vivo” shall mean that cells are extracted from a patient, corrected or otherwise modified by Gene Therapy, and transplanted or dosed back into patient.
- “Vaccination” shall mean the use of a vector containing at least one DNA sequence that encodes at least one protein with the intent to generate an immune response against the protein encoded by the DNA sequence of interest to cause a prophylactic or therapeutic effect in humans and other animals. The protein encoded by the DNA sequence of interest shall not restore an altered or non existing protein function or, modify existing protein function.
- “Field” shall mean ex vivo Gene therapy for human disorders limited to adrenoleukodystrophy (ALD) (including but not limited to AMN, CCALD, and all other variants of this disease caused by genetic mutations), beta hemoglobinopathies (including but not limited to beta-thalassemia and sickle cell anemia), [\*\*\*] leukemias, lymphomas, B-cell malignancies and solid tumors by producing chimeric antigen receptor T-cells [\*\*\*] for [\*\*\*] and [\*\*\*] the “Field” includes in vivo as well as ex vivo Gene therapy. [\*\*\*]

2.2. The following sentence is hereby added to the end of Article 2.1 of the Agreement: “At Licensee’s request, the Parties agree to discuss in good faith about the [\*\*\*].”

2.3. The following sentence is hereby added to the end of Article 4.4 of the Agreement: “Further, Licensee shall have the right to seek patent term extension according to the Drug Price Competition and Patent Term Restoration Act of 1984 (Hatch-Waxman) for any Patent based on Product(s) (in addition to an SPC(s) as provided in Article 4.4). Institut Pasteur will reasonably assist Licensee if Licensee elects to initiate to obtain any such patent term extension”.

**Article 3. Other Terms**

3.1. Upon signature of this Amendment 2 by the Parties, Licensee shall pay Institut Pasteur [\*\*\*] exclusive of taxes. This amount cannot be set-off against future royalties.

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**Article 4. Miscellaneous**

- 4.1. All the other provisions of the Agreement remain unchanged and fully applicable between the Parties.
- 4.2. This Amendment n°2 is effective from the date of signature by the Parties.
- 4.3. This Amendment n°2 makes integral part of the Agreement.

IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed by their authorized respective representative.

Made in Paris,  
In duplicate.

Date: 16 OCT. 2012

/s/ Christophe Mauriet  
INSTITUT PASTEUR  
Christophe Mauriet  
Senior Executive Vice-President

Date: 16 OCT. 2012

/s/ Nick Leschly  
BLUEBIRDBIO INC.  
Nick Leschly  
Chief Executive Officer

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**LICENSE AGREEMENT**  
**BETWEEN**  
**RESEARCH DEVELOPMENT FOUNDATION**  
**AND**  
**BLUEBIRD BIO, INC.**

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### **LICENSE AGREEMENT**

This License Agreement (hereinafter referred to as “Agreement”) is made and entered into as of the 7th day of December, 2011 (the “Effective Date” of this Agreement), by and between RESEARCH DEVELOPMENT FOUNDATION (hereinafter referred to as “Licensor”), a Nevada nonprofit corporation having its office at 402 North Division Street, Carson City, Nevada, 89703;

AND

BLUEBIRD BIO, INC., having an office at 840 Memorial Drive, Cambridge, Massachusetts, 02139 (hereinafter referred to as “Licensee”).

### **WITNESSETH:**

WHEREAS, Licensor is a nonprofit organization exempt from taxation under Section 501(c)(3) of the Internal Revenue Code of 1986;

WHEREAS, Licensor is the owner of “Licensed Patents” (as defined below);

WHEREAS, Licensor has determined that the grant of a license to Licensee is the only practicable manner in which the Licensed Patents can be utilized to benefit the public;

WHEREAS, Licensee desires to obtain a [\*\*\*] license from Licensor as described herein, and Licensor desires to grant such a license pursuant to the terms and conditions of this Agreement;

NOW, THEREFORE, in consideration of the above premises and the covenants herein, the parties agree as follows:

### ARTICLE I

#### Definitions

As used in this Agreement, the following capitalized terms shall have the following respective meanings:

1.1 The term “Licensed Patents” shall mean the United States and foreign patent applications and issued patents listed in Exhibit 1, including all continuations, continuations-in-part, divisionals, patents of addition, reissues, renewals or extensions (including supplementary protection certificates) and all foreign counterparts of the foregoing.

1.2 The term “Licensed Product” shall mean any process, method, material, composition, drug, or other product or portion of a product within a Valid Claim of the Licensed Patents.

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1.3 The term “Make, Use or Sell” shall mean to develop, have developed, produce, have produced, make, have made, manufacture, have manufactured, use, have used, offer for sale, have offered for sale, sell, have sold, rent, have rented, lease, have leased, import or have imported a Licensed Product.

1.4 The term “Affiliate” shall mean any present or future companies, corporations, partnerships, joint ventures, business trusts or other business entities organized under the laws of any nation (a) with respect to which: (i) at least fifty percent (50%) in value of the total equity interests, (ii) at least fifty percent (50%) of the total combined voting power of all classes of shares entitled to vote, or (iii) at least fifty percent (50%) of the profits interest in the case of a partnership, joint venture or other non-stock entity, is directly or indirectly under the control of Licensee, or (b) with respect to which Licensee has effective control, directly or indirectly. “Control” shall mean the possession of the power to direct or cause the direction of the management and the policies of an entity, whether through an ownership interest or by contract or otherwise. The term “Licensee” wherever used herein shall include any Affiliate of Licensee.

1.5 The term “Net Sales” shall mean the gross amount received with respect to the sale or other transfer of Licensed Product, less the following deductions for amounts actually incurred related to such sale or other transfer and included in the gross invoiced amount: (a) normal, customary trade discounts (including volume discounts), credits and rebates, and allowances and adjustments for rejections, recalls, returns or retroactive price reductions; and (b) freight, insurance, sales, use, excise, value-added and similar taxes or duties imposed on the sale.

No other allowance or deduction shall be made by whatever name known. For the avoidance of doubt, transfers of a Licensed Product between any of Licensee, an Affiliate or a sublicensee for sale by the transferee shall not be considered Net Sales hereunder.

1.6 The terms “commercialize” and “commercialization” shall mean the Making, Using, or Selling, licensing or other use by Licensee (or a sublicensee) of the Licensed Product under such circumstances as may be permitted by applicable international, federal, and state laws and regulation.

1.7 The term “Valid Claim” shall mean, in the country of manufacture or sale, (a) a claim of any issued and unexpired patent within the Licensed Patents that (i) has not been permanently revoked, nor held unenforceable, unpatentable or invalid by a decision of a court or other governmental agency of competent jurisdiction that is unappealable or unappealed in the time allowed for appeal, (ii) has not been admitted to be invalid or unenforceable through reissue or disclaimer or otherwise, and (iii) has not been lost through an interference, reexamination, post-grant review or reissue proceeding; or (b) a pending claim of a pending patent application included in the Licensed Patents that was filed and has been prosecuted in good faith and has not been (i) cancelled, withdrawn, abandoned or finally disallowed without the possibility of appeal or refiling of such application, or (ii) pending for more than [\*\*\*] since such claim was first presented; provided, however, that such claim pending for more than [\*\*\*] shall be a Valid Claim if and when it is issued as a claim of an issued and unexpired patent included within Licensed Patents, or if it is part of an opposition, interference, re-examination or other such administrative proceeding.

1.8 The term “Cover(s)” or “Covered” or “Covering” shall mean that a product, process, material, composition, drag, or other product or portion of a product would infringe a Valid Claim in a Licensed Patent (or in the case of a Valid Claim in a patent application within the Licensed Patents, would infringe such Valid Claim if it were in an issued patent) but for the exclusive license granted to Licensee hereunder.

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## ARTICLE II

### Grant of License

2.1 Scope of License. Licensors hereby grants and Licensee hereby accepts a [\*\*\*], exclusive license under the Licensed Patents to Make, Use or Sell Licensed Products. Except as provided herein, no other, further, or different license is hereby granted, either expressly or by implication.

2.2 Right to Sublicense. Licensors hereby grants and Licensee hereby accepts the right to grant sublicenses through multiple tiers to third parties to all or any portion of Licensee’s rights hereunder. Licensee shall, within thirty (30) days of the grant of each such sublicense, give written notice of such sublicenses to Licensors and provide Licensors with a copy thereof; provided that Licensee may redact those portions of such sublicenses that are not necessary for Licensors to determine whether Licensee is in compliance with its obligations under this Agreement. Licensee shall incorporate terms and conditions into its sublicense agreements sufficient to enable Licensee to comply with this Agreement. Upon termination of this Agreement for any reason, Licensors shall grant to a sublicensee that is not in material breach of its sublicense a direct license granting rights and terms equivalent to the sublicense rights and terms which Licensee previously granted to such sublicensee, provided that Licensors shall have no greater rights and obligations to any such sublicensee than Licensors has to Licensee under this Agreement.

2.3 Retained Rights. Licensors retains the right, on behalf of itself and other nonprofit academic research institutions, to practice and use the Licensed Patents for any academic, non-clinical research and educational purposes. Licensee agrees that, notwithstanding any other provision of this Agreement, it has no right to enforce the Licensed Patents against any such practice or use by any such institution.

2.4 Government Rights. If any invention described and claimed in the Licensed Patents is developed with the support of federal research funds, Title 35, Sections 200-212 of the United States Code (the “Bayh-Dole Act”) shall apply. Among other things, the provisions of the Bayh-Dole Act provide the United States Government with non-exclusive rights in any inventions arising from the use of federal funds and also generally impose the obligation that any products embodying the subject invention or produced through the use of such invention be manufactured substantially in the United States. If and to the extent the Bayh-Dole Act is

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applicable to any Licensed Patents, Licensee acknowledges and agrees that the rights granted under this Agreement are subject to the applicable terms and conditions of the Bayh-Dole Act and that Licensee will ensure that all relevant obligations under the Bayh-Dole Act provisions are met.

### ARTICLE III

#### Patents

3.1 Patent Applications. Licensor represents that it has timely filed patent applications relating to the Licensed Patents in the countries listed on Exhibit 1 hereto.

3.2 Patent Prosecution and Maintenance. Licensor will control the conduct, preparation, filing and prosecution of such patent applications within the Licensed Patents and will maintain any patents issued thereon, but will provide Licensee with copies of all office actions and responses thereto and will consider and take into account in good faith Licensee’s comments with respect thereto. Notwithstanding the foregoing sentence, in the event that Licensor within its sole judgment and discretion determines that prosecution or maintenance of a patent in a particular country is not economically viable or otherwise feasible, Licensor shall promptly notify Licensee of Licensor’s intention to abandon such patent application or patent. Upon receipt of such notice, Licensee, in its sole discretion, may elect to assume responsibility (and to pay associated fees and expenses) with respect to a patent application or patent which Licensor intends to abandon. Licensee may, in its sole discretion, abandon any patent application or patent for which it has previously assumed responsibility and will not be liable to Licensor in any way for such abandonment.

3.3 Patent Costs. Licensee shall reimburse Licensor for [\*\*\*] for filing, prosecuting and maintaining the Licensed Patents. Licensor shall invoice Licensee for such patent costs quarterly, and undisputed payments shall be made by Licensee within thirty (30) days after receipt of each invoice.

### ARTICLE IV

#### Royalties and Other Consideration

4.1 License Fee. Licensee shall pay Licensor an up-front non-refundable license fee of [\*\*\*] within ten (10) business days after the execution of this Agreement by the parties.

4.2 Royalty. Licensee shall pay Licensor an earned royalty of [\*\*\*] on Net Sales of Licensed Product, on a Licensed Product-by-Licensed Product and country-by-country basis, where there is at least one Valid Claim of a Licensed Patent Covering such Licensed Product in such country at the time of first marketing approval. Such royalty shall continue until the longer of: (a) expiry or end of the last Valid Claim within a Licensed Patent that Covers a Licensed Product in such country, or (b) ten (10) years from the first marketing approval; provided that the royalty shall be reduced by [\*\*\*] if payable under this clause (b) after the last Valid Claim

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expires or ends during such ten (10) year period. For clarity, no royalty shall be owed on any Licensed Product not Covered by a Valid Claim under a Licensed Patent at the time of first marketing approval in the country in question, and further, under no circumstances shall any royalty be owed (during the term of this Agreement or thereafter) if all the Valid Claim(s) that Cover a Licensed Product in a country are held not valid, unenforceable or otherwise unpatentable. For clarity, in such event, royalties already paid by Licensee shall not be refunded by Licensor. Only one (1) royalty shall be payable on a Licensed Product, regardless of the number of Valid Claims or the number of patent applications and patents within the Licensed Patents under which such Licensed Product has been Made, Used or Sold.

4.3 Basis of Royalty Obligation. Royalty payments to be paid at the applicable rate(s) hereunder shall be paid on a Licensed Product by Licensed Product and country-by-country basis.

4.4 Limitation on Deductions from Royalty Payments. Wherever this Agreement provides that Licensee may deduct expenses, payments or other amounts from royalties payable to Licensor, such deduction shall be prorated over such time as is necessary to assure that the royalties payable to Licensor in any period shall not be reduced by more than [\*\*\*].

4.5 Milestone Payments. In addition to the up-front license fee and royalties required under this Article IV, Licensee shall make milestone payments to Licensor as set forth in Exhibit 2 hereto. Such cash payments shall be delivered to Licensor within forty-five (45) days after the end of the calendar quarter in which each of the milestone payment events indicated on such exhibit occurs.

4.6 Marketing Arrangements. Where Licensed Products are sold by a third party other than Licensee or sublicensee under any type of commercial arrangement between Licensee (or sublicensee) and such third party (including, without limitation, a joint venture, distributorship, or collaboration agreement), Net Sales for earned royalty purposes shall be calculated based on the gross sales of Licensed Products by such third party.

## ARTICLE V

### Reports and Payments

5.1 Progress Reports. Licensee agrees to make an annual report to Licensor each March covering Licensee's (and its sublicensees', if applicable) progress during the previous calendar year toward research, development, commercialization and out-licensing of Licensed Products. Email communication shall suffice for the purpose of this reporting requirement.

5.2 Notice of Commercial Sale. Licensee shall notify Licensor, in writing, within thirty (30) days of the date of the first commercial sale of a Licensed Product to a third party by Licensee or a sublicensee.

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5.3 Royalty Reports and Payments. Licensee agrees that Licensor shall, if applicable, receive within forty-five (45) days after the end of each quarter of each calendar year:

- (a) a complete and accurate royalty report showing the information and basis on which such amounts have been calculated, including disclosure of at least the following information:
  - i. [\*\*\*];
  - ii. [\*\*\*];
  - iii. [\*\*\*];
  - iv. [\*\*\*]; and
- (b) payment of amounts due to Licensor pursuant to this Agreement, including, but not limited to, amounts pursuant to Articles IV and VI.

5.4 U.S. Dollars. All amounts payable by Licensee shall be paid in U.S. Dollars. Conversion from currencies other than U.S. Dollars shall be at the rate of exchange used by Licensee for its general accounting purposes, consistent with generally accepted accounting principles.

5.5 Report on Termination. Licensee also agrees to make a written report to Licensor within ninety (90) days after the expiration or termination of this Agreement, stating in such report the amounts payable hereunder and the basis therefor not previously reported to Licensor. In the event of a termination prior to expiration of the Term, Licensee shall also continue to make annual reports pursuant to the provisions of this Agreement covering sales, uses, or production and the applicable earned royalties and other amounts payable hereunder for Licensed Products made during the Term, but not used or sold until after termination thereof, until such time as all such makings, uses or sales shall have terminated. Concurrent with the submittal of such post-expiration or post-termination report, Licensee shall pay Licensor all applicable royalties and other amounts payable hereunder.

5.6 Books and Records. Licensee shall keep full, true, clear and accurate records and books of account with respect to the Licensed Products subject to royalty or other payments hereunder. Said records and books of account shall be kept by Licensee at the usual places where its like records and books are kept and shall be retained for a period of three (3) years following the end of the calendar year to which they pertain. Licensor shall have the right through an independent public accountant selected by Licensor and reasonably acceptable to Licensee to examine and inspect during normal business hours all such records and books of account and such other records and accounts as may under recognized accounting practices contain information reasonably bearing upon the amounts payable to it under this Agreement. Prompt adjustment shall be made by the proper party to compensate for any errors or omissions disclosed by such examination or inspection. In the event the examination or inspection results

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in a discrepancy in the correctness of the payments due under this Agreement in an amount in excess of [\*\*\*] of the payments due Licensor for any single quarter audited, Licensee shall reimburse Licensor for all reasonable out-of-pocket costs and fees associated with such examination or inspection, and all reasonable out-of-pocket costs and expenses required to collect the amount underpaid, including (but not limited to) reasonable attorneys’ fees incurred in connection therewith. Neither such right to examine and inspect nor the right to receive such adjustment shall be affected by any statement to the contrary appearing on checks or otherwise, unless such statements appear in a letter, signed by the party having such right and delivered to the other party, expressly waiving such right. Notwithstanding the foregoing, Licensor may require Licensee to furnish any other information reasonably requested to enable Licensor to evaluate Licensee’s performance in accordance with this Agreement.

5.7 Delinquent Payments. Payments provided for in this Agreement shall, when overdue, bear interest [\*\*\*] per annum until paid, but in no event shall such interest exceed the usury limit, if any, as may exist from time to time in the State of Nevada. Each such payment when made shall be accompanied by all interest so accrued. Said interest and the payment and acceptance thereof shall not negate or waive the right of Licensor to seek any other remedy, legal or equitable, to which it may be entitled because of the delinquency of any payment or any other breach of this Agreement by Licensee.

## ARTICLE VI

### Diligence; Minimum Royalties

Licensee shall undertake to use commercially reasonable and diligent efforts for a company of Licensee’s size and resources, directly or through a sublicensee, to develop or commercialize one or more Licensed Products, including its first Licensed Product by 2016 and a second Licensed Product by 2018. Licensee will be considered not to have utilized reasonable commercial efforts unless it (or a sublicensee) makes the following minimum annual royalty payments to Licensor:

- (a) [\*\*\*];
- (b) [\*\*\*]; and
- (c) [\*\*\*].

Such payments will be creditable against earned royalties otherwise due to Licensor for a given calendar year.



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## ARTICLE VII

### Protection of Patents

7.1 Protection. Each party agrees to cooperate fully in any action under this Article VII which is controlled by the other party, including by joining as a party to any proceeding if required by applicable law; provided that the controlling party reimburses the cooperating party promptly for any reasonable costs and expenses incurred by the cooperating party in connection with providing such assistance. [\*\*\*]

7.2 Notice of Infringement; Licensor Enforcement of Third Party Infringement. Licensor and Licensee shall each give prompt written notice to the other of any infringement of a Licensed Patent by any third party as may come to its knowledge. [\*\*\*]

7.3 Notice of Infringement; Claim of Licensee Infringement. Licensee shall promptly advise Licensor in writing of any notice or claim of any infringement and of the commencement against it of any suit or action for infringement of a third party patent made or brought against Licensee and based upon the use hereunder by Licensee of the Licensed Patents. [\*\*\*]

(a) [\*\*\*]

(b) [\*\*\*]

7.4 Reasonable Assistance. [\*\*\*]

7.5 Declaratory Judgment Actions. If a declaratory judgment action is brought naming Licensor or Licensee or any of its Affiliates or sublicensees as a defendant and alleging invalidity, unenforceability or non-infringement of any Licensed Patents, Licensee or Licensor, as the case may be, shall promptly notify the other party in writing. [\*\*\*]

7.6 Patent Certifications. Each party shall notify and provide the other with copies of any allegations of alleged patent invalidity, unenforceability or non-infringement of a Licensed Patent pursuant to the FDA’s Paragraph IV Patent Certification procedure by a third party filing an Abbreviated New Drug Application, an application under §505(b)(2) of the Federal Food, Drug and Cosmetics Act, or any other similar patent certification by a third party, and any foreign equivalent thereof. Such notification and copies shall be provided to the other party within five (5) business days after the party receives such certification.

## ARTICLE VIII

### Disclaimer of Liability and/or Warranty

8.1 No Warranty. Nothing in this Agreement shall be construed as:

(a) a warranty or representation by Licensor as to the validity or scope of any Licensed Patents; or

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- (b) a warranty or representation that anything Made, Used or Sold under any license granted in this Agreement is or will be free from infringement of patents, copyrights, and/or trademarks of third parties; or
- (c) an express or implied warranty of merchantability or fitness for a particular purpose.

8.2 No Damages. Neither party shall be liable to the other party for indirect, special, consequential or punitive damages under any circumstances.

8.3 No Warranty of Quality or Usefulness. Licensor shall have no responsibility for the ability of Licensee to use such information, the quality or performance of any process or any Licensed Product produced by Licensee with the aid of such information, or with respect to claims of third parties arising from Licensee’s use of such information.

8.4 Indemnification. Licensee shall assume all responsibility and liability for the sale, use, production, and/or commercialization of the Licensed Products, including, but not limited to, the safety, effectiveness, and reliability of the Licensed Products produced pursuant to this Agreement. Licensee further agrees to defend, indemnify, and hold harmless Licensor, its trustees, directors, officers, employees, agents, representatives, successors, assigns, affiliated entities and Other Corporations (as defined in Section 11.3 below) (collectively “Indemnitees”) from and against any and all liability, demands, damages, expenses and losses for death, personal injury, illness, or property damage, including the cost of defense against same, which may be asserted by third parties, or any third party claims which may arise from the sale, use, production, commercialization, or other disposition of Licensed Products pursuant to any right or license granted under this Agreement, except to the extent that such liability, demands, damages, expenses or losses relate to or arise out of Licensor’s material breach of its representations, warranties and covenants under this Agreement, or Licensor’s or Indemnitees’ gross negligence or willful misconduct.

8.5 Insurance. Licensee agrees to purchase and/or maintain insurance coverage sufficient, taking into account its other assets, to establish the ability of Licensee to honor the indemnity made herein, and Licensor shall be listed as an additional named insured on any such insurance coverage. Licensee shall furnish evidence satisfactory to Licensor of its insurance coverage upon request of Licensor. Upon Licensee’s, or any of its sublicensees’, Making, Use or Sale of Licensed Products commercially, the initial amount of insurance coverage required is in the face amount of [\*\*\*].

## ARTICLE IX

### Term; Termination

9.1 Term. The Term of this Agreement (“Term”) shall continue until its expiration upon the later of: (a) there being no more Valid Claims within the Licensed Patents, or (b) the expiration of Licensee’s royalty obligations on Licensed Products that are subject to an earned royalty, if such earned royalty is based on the minimum ten (10) year royalty period described in Section 4.2(b) above; unless this Agreement is earlier terminated as herein provided.

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9.2 Termination for Cause: Insolvency. If Licensee shall determine that it intends to file for bankruptcy or reorganization, it shall give prompt written notice to Licensor. Failure to give such notice shall cause immediate termination of this Agreement, and all rights of Licensee in the Licensed Patents shall automatically revert to Licensor. If Licensee shall become bankrupt; if the business or any assets or property of Licensee shall be placed in the hands of a receiver, assignee or trustee, whether by the voluntary act of Licensee or otherwise; if Licensee institutes or suffers to be instituted any procedure in bankruptcy court for reorganization or rearrangement of its financial affairs; if Licensee makes a general assignment for the benefit of creditors; or if Licensee or an Affiliate or a sublicensee (with the assistance, consent, approval or cooperation of Licensee) institutes or suffers to be instituted any procedure, administratively or in a court, challenging validity or patentability of any patent or patent application within the Licensed Patents, this Agreement shall immediately terminate, and all rights of Licensee in the Licensed Patents shall automatically revert to Licensor. Upon occurrence of any of the foregoing events, Licensee shall give prompt written notice thereof to Licensor.

9.3 Default. Upon any breach or default under this Agreement by Licensee, Licensor may give written notice thereof to Licensee, and Licensee shall have ninety (90) days thereafter to cure such breach or default, except in the event of a breach or default by non-payment, in which case the cure period shall be thirty (30) days. If such breach or default is not cured within such period, Licensor shall have the right in its sole option to cancel and terminate this Agreement and the licenses granted by it by giving written notice thereof to Licensee. In such event, Licensor may also seek such other relief as may be provided by law or in equity in such circumstances.

9.4 Commercialization Rights Upon Termination. Upon termination hereof under Section 9.2 or 9.3, all rights of Licensee in the Licensed Patents shall revert to Licensor, and Licensee agrees to execute appropriate written releases and/or assignments of such rights to Licensor; and further, Licensee agrees to discontinue the commercialization of the Licensed Patents. Upon expiration of the Term and Licensee's payment of all amounts due Licensor hereunder, Licensee will continue to have commercialization rights with respect to the Licensed Products with no further royalty obligation to Licensor, and Licensor will not license or otherwise grant rights to any third party inconsistent with such rights remaining exclusively in Licensee.

9.5 Provisions Surviving Termination. Articles X and XIII and Sections 8.1, 8.2, 8.3, 8.4, 9.4 and 11.3 of this Agreement shall survive expiration or termination of this Agreement.

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## ARTICLE X

### Representations and Warranties

10.1 Warranty to Title. Licensor represents and warrants that it owns the Licensed Patents and has the legal power and authority to extend the rights granted to Licensee pursuant to this Agreement, and that it has not assigned, licensed, pledged or compromised the Licensed Patents or made any commitments or offers inconsistent with or in derogation of the rights created by this Agreement.

10.2 Power and Authority. Licensee represents and warrants that (a) it has full power and authority to enter into this Agreement and to carry out the transactions contemplated hereby; and (b) this Agreement constitutes the legal, valid and binding obligation of Licensee, enforceable against Licensee in accordance with its terms.

10.3 Compliance with Laws. Licensee represents and warrants that it will comply with all applicable laws and regulations, including without limitation, all United States laws and regulations controlling the export of commodities and technical data. Licensee will be solely responsible for any violation of such laws or regulations by Licensee, and it will defend and hold Licensor harmless in the event of any legal action of any nature occasioned by such violation.

10.4 No Knowledge of Infringement. Licensor represents that it has no knowledge of any infringement of the Licensed Patents by any third party.

## ARTICLE XI

### Agency/Partnership/Use of Name

11.1 No Agency. Neither party shall be deemed to be an agent of the other party as a result of any transaction under or related to this Agreement, and shall not in any way pledge the other party's credit or incur any obligations on behalf of the other party.

11.2 No Partnership. This Agreement shall not constitute either a partnership or a joint venture, and neither party may be bound by the other to any contract, arrangement or understanding except as specifically stated herein.

11.3 Prohibition Against Use of Name. Except to the extent required to comply with applicable laws and regulations, without prior written consent obtained from Licensor, Licensee (including any Affiliate or sublicensee of Licensee) shall not use for purposes of sales, advertising, marketing, marking of goods, promotion to investors, press releases or other publicity, etc.: (i) the name of (or any other information which would identify) Licensor or any corporation which is controlled by the same persons who control Licensor (“Other Corporation”); (ii) the names of trustees, directors, officers, or employees of Licensor or an Other Corporation; or (iii) any trademarks (or adaptations thereof) of Licensor or an Other Corporation. The foregoing notwithstanding, without the consent of Licensor, Licensee may

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indicate other than in advertising that it is licensed by Licensor under the Licensed Patents and identify the inventors, their affiliation with Licensor, and their relationship to Licensor, and further, Licensee may comply with disclosure requirements of all applicable laws relating to its business, including, without limitation, United States and state securities laws.

## ARTICLE XII

### Marking

To the extent commercially feasible and consistent with prevailing business practices, Licensee agrees to apply or have applied to all articles and to all containers containing Licensed Products manufactured by it or any sublicensee(s) under this Agreement the number of each issued patent under the Patent Rights that applies to such Licensed Product.

## ARTICLE XIII

### Nondisclosure of Confidential Information

All confidential or proprietary business, scientific and technical information communicated by one party to the other party under this Agreement, including information contained in unpublished patent applications, shall be kept confidential by such other party. Notwithstanding the foregoing, either party shall be relieved of the confidentiality obligations herein and not be prevented by this Agreement from utilizing any information received by it from the other party if:

- (a) the information, at the time of disclosure, is in the public domain or, after disclosure, becomes part of the public domain through no act or omission of the receiving party;
- (b) the receiving party can show that the information was in its possession at the time of disclosure and was not acquired, directly or indirectly, from the disclosing party;
- (c) the information is lawfully obtained or received on a non-confidential basis from a third party, other than the disclosing party, having the legal right to transmit same; or
- (d) the disclosure of such information is essential for the commercial exploitation of the Licensed Patents under this Agreement, provided that such information is disclosed subject to a secrecy agreement.

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ARTICLE XIV

Miscellaneous

14.1 Captions. The captions herein are for convenience only and shall not be deemed to limit or otherwise affect the construction hereof.

14.2 Notices. Any notice or other communication hereunder must be given in writing and (a) delivered in person, (b) transmitted by telefax or other telecommunications mechanism, (c) mailed by certified or registered mail, postage prepaid, receipt requested, or (d) sent by overnight delivery with charges prepaid and receipt acknowledged, as follows:

If to Licensor, addressed to:

Research Development Foundation  
402 North Division Street  
Carson City, Nevada 89703  
Attn: Andrew MacKenzie, Esq.  
Phone: (775) 882-0202  
Fax: (775) 882-7918

If to Licensee, addressed to:

bluebird bio, inc.  
840 Memorial Drive  
Cambridge, Massachusetts 02139  
Attn: Head of Business Development  
Phone: (617) 873-0900  
Fax: (617) 576-2421

or to such other address or to such other person as the party shall have last designated by such notice to the other party. Each such notice or other communication shall be effective when actually received at such address.

14.3 Assignment. This Agreement, in whole or in part, shall not be assignable by either party without prior written consent of the other party (unless to a successor entity to such party by merger, acquisition, consolidation or other non-bankruptcy reorganization or sale of substantially all of its assets or that portion of its business to which this Agreement relates), and any attempted assignment without such consent shall be void.

14.4 No Waiver. The failure of either party to enforce at any time any of the provisions of this Agreement, or any rights in respect thereto, or to exercise any election herein provided, shall in no way be considered to be a waiver of such provisions, rights, or elections, or in any way to affect the validity of this Agreement. The exercise by either party of any of its rights herein or any of its elections under the terms or covenants herein shall not preclude either party from exercising the same or any other rights it may have under this Agreement, irrespective of any previous action or proceeding taken by either party hereunder.

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14.5 Choice of Law and Jurisdiction. This Agreement shall be governed and construed in accordance with the laws of the State of Nevada, U.S.A. applicable to contracts made in such State without regard to conflicts of law doctrines, and the parties agree that jurisdiction and venue for any dispute regarding this Agreement will be in such State.

14.6 Severability. If any provision of this Agreement is judicially determined to be void or unenforceable, such provision shall be construed to be severable from the other provisions of this Agreement, which shall retain full force and effect.

14.7 Further Acts. The parties hereto agree promptly to execute, forward, or otherwise provide all documents and material necessary or desirable to effectuate this Agreement.

14.8 Entire Agreement. This Agreement constitutes the entire agreement between the parties with respect to the subject matter hereof and shall supersede all previous communications, either oral or written, between the parties hereto with respect to the subject matter hereof. No agreement or understanding bearing on the same shall be binding upon either party hereto unless it shall be in writing and signed by the duly authorized officer or representative of each of the parties and shall expressly refer to this Agreement.

14.9 Successors and Assigns. This Agreement shall be binding on and shall inure to the benefit of the parties hereto, and their respective successors and assigns.

IN WITNESS WHEREOF, the parties hereto have caused this Agreement to be duly executed in multiple originals by their duly authorized representatives.

RESEARCH DEVELOPMENT FOUNDATION

By: /s/ Andrew MacKenzie

Print Name: Andrew MacKenzie

Title: Vice President

BLUEBIRD BIO, INC.

By: /s/ Nick Leschly

Print Name: Nick Leschly

Title: CEO

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EXHIBIT 1

Licensed Patents

[See attached insert from F&J Master Listings]



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**Research Development Foundation by Alias**

**Master Listing of Technologies**

**10/04/2011**

**CLFR: 010**      TITLE: [\*\*\*]  
                    **Summary:** [\*\*\*]

Client Reference No.: NULL

[\*\*\*]

<u>Country</u>	<u>Case</u>	<u>Status</u>	<u>Application No.</u>	<u>Filing Date</u>	<u>Patent No.</u>	<u>Granted</u>
[***]						

**CLFR: 011**      TITLE: [\*\*\*]  
                    **Summary:** [\*\*\*]

Client Reference No.: CLFR:011

[\*\*\*]

<u>Country</u>	<u>Case</u>	<u>Status</u>	<u>Application No.</u>	<u>Filing Date</u>	<u>Patent No.</u>	<u>Granted</u>
[***]						

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**Research Development Foundation by Alias**

**Master Listing of Technologies**

**10/04/2011**

**CLFR: 014**      **TITLE:** [\*\*\*]  
**Summary:** [\*\*\*]

Client Reference No.: NULL

[\*\*\*]

<u>Country</u>	<u>Case</u>	<u>Status</u>	<u>Application No.</u>	<u>Filing Date</u>	<u>Patent No.</u>	<u>Granted</u>
[***]						

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EXHIBIT 2

Milestone Payments

Except as provided herein, milestone payments in the following amounts shall be paid for each Licensed Product requiring marketing approval, payable on a product-by-product basis:

[\*\*\*]

Each milestone shall be payable one time only, on a Licensed Product-by-Licensed Product basis.

In the event that a Licensed Product achieves the milestone on more than one occasion, only the first achievement of such shall be subject to milestone consideration. For example, [\*\*\*].

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### NOVATION AGREEMENT

This Agreement between THE BOARD OF TRUSTEES OF THE LELAND STANFORD JUNIOR UNIVERSITY (“Stanford”), an institution of higher education having powers under the laws of the State of California, and bluebird bio, Inc., a corporation having a principal place of business at 840 Memorial Drive, Cambridge, MA 02139 is effective on the 2<sup>nd</sup> day of April, 2012 (“Effective Date”).

#### 1 BACKGROUND

Stanford has rights to biological material known as 293T Cell Line. It was developed in the laboratory of Dr. Michele Calos, and is described in Stanford Docket S97-079. The biological material was developed in the course of research supported by the National Institutes of Health. Stanford wants to have the biological material developed and marketed as soon as possible so that resulting products may be available for public use and benefit.

Stanford and BBB (formerly Genetix Pharmaceuticals Inc.) are parties to a previous agreement concerning the Biological Material dated July 11, 2002, hereafter “Prior Agreement”. Stanford and BBB wish to amend and restate said Prior Agreement by this Novation Agreement (“Agreement”), beginning as of the Effective Date of this Agreement.

#### 2 DEFINITIONS

- 2.1 “BBB” means bluebird bio, Inc. and its Affiliates. “Affiliates” any person, corporation, or other business entity which controls, is controlled by, or is under common control with BBB; and for this purpose, “control” of a corporation means the direct or indirect ownership of more than fifty percent (50%) of its voting stock, and “control” of any other business entity means the direct or indirect ownership of greater than a fifty percent (50%) interest in the income of such entity.
- 2.2 “Biological Material” means the 293T cell line previously provided to BBB under the Prior Agreement.
- 2.3 “Licensed Field of Use” means any commercial and/or non-commercial use of Biological Material for:
  - research, and non-clinical and clinical development purposes; and
  - human and animal gene therapy products.
- 2.4 “Licensed Product” means a product or part of a product in the Licensed Field of Use containing, derived from, or made using Biological Material.
- 2.5 “Licensed Territory” means [\*\*\*].

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2.6 “Net Sales” means all gross revenue actually received by BBB, from the sale, transfer or disposition of Licensed Product to a third party. Net Sales excludes the following items (but only as they pertain to the making, using, importing or selling of Licensed Products, are included in gross revenue, and are separately billed):

- (A) import, export, excise and sales taxes, and custom duties;
- (B) costs of insurance, packing, and transportation from the place of manufacture to the customer’s premises or point of installation;
- (C) credit for returns, allowances, or trades; and
- (D) cash, trade or quantity discounts actually granted to third parties.

For clarity, Net Sales does not include (i) any payments received by BBB, its affiliates, or sublicensees in consideration of a Sublicense; or (ii) transfers of a Licensed Product between any of BBB, an affiliate or a sublicensee for sale by the transferee.

2.7 “Stanford Indemnitees” means Stanford and Stanford Hospitals and Clinics, and their respective trustees, officers, employees, students, and agents.

### 3 GRANT

3.1 **Grant.** Subject to the terms and conditions of this Agreement, Stanford hereby grants BBB a license under the Biological Material in the Licensed Field of Use to make, have made, use, have used, import, have imported, offer to sell, have offered to sell, sell and have sold Licensed Product in the Licensed Territory.

3.2 **Nonexclusivity.** The license is nonexclusive in the Licensed Field of Use beginning on April 2, 2012 and expiring April 2, 2037. BBB may elect to extend the term of this Agreement for additional twenty five (25) year periods upon written notice to Stanford, without further consideration or amendment to this agreement; provided however, that i) such notice of extension must include verification by BBB that it has a commercial product on the market at that time, and ii) BBB is in material compliance with this Agreement at that time. If either of these conditions is not met, BBB and Stanford may elect to extend the term of this Agreement by mutual consent.

3.3 **Retained Rights.** Stanford retains title to all Biological Materials.

3.4 **Specific Exclusion.** Stanford does not:

- (A) grant to BBB any other licenses, implied or otherwise, to any patents or other rights of Stanford regardless of whether the patents or other rights are required to exploit any Biological Material; and
- (B) agree to furnish to BBB any technology or technological information other than the Biological Material or to provide BBB with any assistance.

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#### 4 SUBLICENSING

- 4.1 **Permitted Sublicensing.** BBB may grant sublicenses to its rights under this Agreement in the Licensed Field of Use to third parties. Such Sublicensees may then grant further sublicenses to other third parties for the purpose of the development, manufacturing, marketing, selling, importing and distribution of Licensed Products; provided however that (i) Sublicensees shall first submit to Stanford the identity of such other third party, along with a summary of the purpose for such further sublicense and (ii) Stanford shall then provide Sublicensee its prompt, prior written consent, which shall not be unreasonable withheld or conditioned.
- 4.2 **Sublicense Requirements.** Any Sublicense:
- (A) is subject to this Agreement;
  - (B) will expressly include the provisions of Articles 8, 9, and 10 for the benefit of Stanford; and
  - (C) will require the transfer of all the sublicensee's obligations to BBB specifically relating to the sublicense, including the payment of royalties related to the Biological Material specified in the sublicense, to Stanford or its designee, if this Agreement is terminated.
  - (D) BBB will submit to Stanford a copy of each sublicense promptly following execution.
- 4.3 **Sublicense Consideration.** BBB will pay Stanford a [\*\*\*] payment in consideration of said sublicense, unless such sublicense is to a collaborating partner, contract manufacturer or contract research organization during the term of the agreement between BBB and such partner/contractor. If sublicensee already has a license for the research use of the Biological Material, said milestone payment will be reduced to [\*\*\*]. If the sublicensee already has a license for the commercial use of the Biological Material, said milestone payment will not be due.

#### 5 GOVERNMENT RIGHTS

This Agreement is subject to Title 35 Sections 200-204 of the United States Code. Among other things, these provisions provide the United States Government with nonexclusive rights to the Biological Material. BBB will ensure all obligations of these provisions are met.

#### 6 ROYALTIES

- 6.1 **Issue Royalty.** BBB will pay to Stanford a noncreditable, nonrefundable license issue royalty of [\*\*\*] within thirty (30) days after signing this Agreement. Upon receipt of payment, Stanford will send Biological Material to BBB.
- 6.2 **License Maintenance Fee.** Beginning April 2, 2013, and each April 2, thereafter, BBB will pay Stanford a yearly license maintenance fee based on the Net Sales of Licensed Products as follows:  
[\*\*\*];

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[\*\*\*]

[\*\*\*]

[\*\*\*]

Yearly maintenance payments are nonrefundable, but they are creditable each year as described in Section 6.4.

6.3 **Earned Royalty.** BBB will pay Stanford earned royalties on Net Sales as follows:

Licensed Products for Research Use [\*\*\*] of Net Sales

Licensed Products for human and animal lentivirus based gene therapy products:

[\*\*\*]

[\*\*\*]

These percentages shall be reduced by [\*\*\*] for each third party license that requires payment(s) by BBB with respect to a Licensed Products; provided however, the royalty owed to Stanford under this Section 6.3 shall not be less than [\*\*\*] on this basis.

6.4 **Creditable Payments.** The license maintenance fee for a year may be offset against earned royalty payments due on Net Sales occurring in that year.

For example:

(A) if BBB pays Stanford a \$10 maintenance payment for year Y, and according to Section 6.3 \$15 in earned royalties are due Stanford for Net Sales in year Y, BBB will only need to pay Stanford an additional \$5 for that year’s earned royalties due on Net Sales.

(B) if BBB pays Stanford a \$10 maintenance payment for year Y, and according to Section 6.3 \$3 in earned royalties are due Stanford for Net Sales in year Y, BBB will not need to pay Stanford any earned royalty payment due on Net Sales for that year. BBB will not be able to offset the remaining \$7 against a future year’s earned royalties.

6.5 **No Escrow.** BBB shall not pay royalties into any escrow or other account.

6.6 **Currency.** BBB will calculate the royalty on sales in currencies other than U.S. Dollars using the appropriate foreign exchange rate for the currency quoted by the Bank of America (San Francisco) foreign exchange desk, on the close of business on the last banking day of each calendar quarter. BBB will make royalty payments to Stanford in U.S. Dollars.

6.7 **Non-U.S. Taxes.** BBB will pay all non-U.S. taxes related to royalty payments. These payments are not deductible from any payments due to Stanford.

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6.8 **Interest.** Any payments not made when due will bear interest at the lower of (a) [\*\*\*] or (b) the maximum rate permitted by law.

## 7 ROYALTY REPORTS, PAYMENTS, AND ACCOUNTING

- 7.1 **Earned Royalty Payment and Report.** Beginning with the first commercial sale of a Licensed Product to a third party, BBB will submit to Stanford a written report (even if there are no commercial sales) and an earned royalty payment within sixty (60) days after the end of each calendar year [\*\*\*], and within sixty (60) days after the end of each calendar quarter thereafter. This report will be in the form of Appendix A and will state the number, description, and aggregate Net Sales of Licensed Product during the completed calendar quarter. With each report BBB will include any earned royalty payment due Stanford for the completed calendar quarter (as calculated under Section 6.3 and subject to Section 6.4.)
- 7.2 **Termination Report.** BBB will pay to Stanford all applicable royalties and submit to Stanford a written report within ninety (90) days after the license terminates. BBB will continue to submit earned royalty payments and reports to Stanford after the license terminates, until all Licensed Products made or imported under the license have been sold.

## 8 EXCLUSIONS AND NEGATION OF WARRANTIES

- 8.1 **Representation.** Stanford represents that it has the right to enter into this Agreement and grant the rights and licenses hereunder.
- 8.2 **Negation of Warranties.** Except as provided in Section 8.1, Stanford provides BBB the rights granted in this Agreement AS IS and WITH ALL FAULTS. Stanford makes no representations and extends no warranties of any kind, either express or implied. Among other things, Stanford disclaims any express or implied warranty:
- (A) of merchantability, of fitness for a particular purpose,
  - (B) of non-infringement or
  - (C) arising out of any course of dealing.
- 8.3 **No Representation of Biological Material.** BBB also acknowledges that Stanford does not represent or warrant that the exploitation of Biological Material will be successful.

## 9 INDEMNITY

- 9.1 **Indemnification.** BBB will indemnify, hold harmless, and defend all Stanford Indemnitees against any third party claim of any kind arising out of or related to the exercise of any rights granted BBB under this Agreement or the breach of this Agreement by BBB.



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- 9.2 **No Indirect Liability.** Neither party will be liable to the other for any indirect, special, consequential, or other damages whatsoever, whether grounded in tort (including negligence), strict liability, contract, or otherwise arising out of or in connection with solely this Agreement under any theory of liability, provided, however, that the foregoing will not apply to any right of action for infringement, contributory infringement or inducement of infringement Stanford may have under any applicable law. Stanford will not have any responsibilities or liabilities whatsoever with respect to Licensed Product(s).
- 9.3 **Workers’ Compensation.** BBB will comply with all statutory workers’ compensation and employers’ liability requirements for activities performed under this Agreement.
- 9.4 **Insurance.** During the term of this Agreement, BBB will maintain Comprehensive General Liability Insurance, including Product Liability Insurance, with a reputable and financially secure insurance carrier to cover the activities of BBB. The insurance will provide minimum limits of liability of [\*\*\*]. Insurance must cover claims incurred, discovered, manifested, or made during or after the expiration of this Agreement and must be placed with carriers with ratings of at least A- as rated by A.M. Best. Within 15 days of the Effective Date of this Agreement, BBB will furnish a Certificate of Insurance evidencing primary coverage and additional insured requirements. BBB will provide to Stanford 30 days prior written notice of cancellation or material change to this insurance coverage. BBB will advise Stanford in writing that it maintains excess liability coverage (following form) over primary insurance for at least the minimum limits set forth above. All insurance of BBB will be primary coverage; insurance of Stanford and Stanford Hospitals and Clinics will be excess and noncontributory.

## 10 STANFORD NAMES AND MARKS

BBB will not identify Stanford in any promotional statement, or otherwise use the name of any Stanford faculty member, employee, or student, or any trademark, service mark, trade name, or symbol of Stanford or Stanford Hospitals and Clinics, including the Stanford name, unless BBB has received Stanford’s prior written consent. Permission may be withheld at Stanford’s sole discretion. Notwithstanding the foregoing, without the consent of Stanford, BBB may state to its actual and prospective investors, strategic partners and sublicensees that it is licensed under the Biological Material, and further BBB may comply with disclosure requirements of all applicable laws relating to its business, including, without limitation, United States and state securities laws.

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## **11 TERMINATION**

### **11.1 Termination by BBB.**

- (A) BBB may terminate this Agreement by giving Stanford written notice at least 30 days in advance of the effective date of termination selected by BBB.
- (B) As of the effective date of termination, BBB will:
  - (1) cease use and sale of Biological Material and any Licensed Products; and
  - (2) return to Stanford or destroy all Biological Material.

### **11.2 Termination by Stanford.**

- (A) Stanford may also terminate this Agreement if BBB:
  - (1) is delinquent on any report or payment;
  - (2) is not using commercially reasonable efforts to develop, manufacture and/or commercialize one or more Licensed Products or to enter into a partnering or collaboration agreement or corporate transaction with respect to Licensed Products;
  - (3) is in material breach of any provision; or
  - (4) provides any false report.
- (B) Termination under this Section 11.2 will take effect 30 days after written notice by Stanford unless BBB remedies the problem in that 30-day period, except for Sections 11.2(A)(2), in which case termination will take place 120 days after written notice by Stanford unless BBB remedies the problem in that 120-day period.
- (C) As of the effective date of termination, BBB will:
  - (1) cease use and sale of Biological Material and any Licensed Products; and
  - (2) return to Stanford or destroy all Biological Material.

### **11.3 Surviving Provisions.** Surviving any termination or expiration are:

- (A) BBB’s obligation to pay royalties accrued or accruable;
- (B) any claim of BBB or Stanford, accrued or to accrue, because of any breach or default by the other party; and
- (C) the provisions of Articles 6.4, 7, 8, and 9, and any other provision that by its nature is intended to survive.

## **12 ASSIGNMENT**

The rights and obligations of the parties under this Agreement may not be assigned or otherwise transferred without the written consent of Stanford and BBB; however, no consent is needed for an assignment to an entity which acquires a party (which

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acquisition may be by merger, purchase of assets, or change of control), so long as the acquiring entity agrees in writing to be bound by the terms of this Agreement. In the event of any acquisition of BBB, Stanford agrees to execute whatever agreements are necessary, if any, to assure the transfer of the rights and obligations herein to the succeeding entity.

### **13 EXPORT**

BBB and its affiliates and sublicensees shall comply with all United States laws and regulations controlling the export of licensed commodities and technical data. (For the purpose of this paragraph, “licensed commodities” means any article, material or supply but does not include information; and “technical data” means tangible or intangible technical information that is subject to US export regulations, including blueprints, plans, diagrams, models, formulae, tables, engineering designs and specifications, manuals and instructions.) These laws and regulations may include, but are not limited to, the Export Administration Regulations (15CFR 730-774), the International Traffic in Arms Regulations (22 CFR 120-130) and the various economic sanctions regulations administered by the US Department of the Treasury (31 CFR 500-600).

Among other things, these laws and regulations prohibit or require a license for the export or retransfer of certain commodities and technical data to specified countries, entities and persons. BBB hereby gives written assurance that it will comply with, and will cause its affiliates and sublicensees to comply with all United States export control laws and regulations, that it bears sole responsibility for any violation of such laws and regulations by itself or its affiliates or sublicensees, and that it will indemnify, defend and hold Stanford harmless for the consequences of any such violation.

### **14 ARBITRATION**

Any dispute between the parties regarding any payments made or due under this Agreement will be settled by arbitration in accordance with the JAMS Arbitration Rules and Procedures. The parties are not obligated to settle any other dispute that may arise under this Agreement by arbitration.

### **15 NOTICES**

All notices under this Agreement are deemed fully given when written, addressed, and sent as follows:

All general notices to BBB are mailed to:

Head of Business Development  
Bluebird Bio, Inc.  
840 Memorial Drive.  
Cambridge, MA 02139

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All financial invoices to BBB (i.e., accounting contact) are e-mailed to:

[\*\*\*]

With a copy to: [\*\*\*]

All progress report invoices to BBB (i.e., technical contact) are e-mailed to:

[\*\*\*]

All general notices to Stanford are e-mailed or mailed to:

Office of Technology Licensing

1705 El Camino Real

Palo Alto, CA 94306-1106

[\*\*\*]

All payments to Stanford are mailed to:

Stanford University

Office of Technology Licensing

Department #44439

P.O. Box 44000

San Francisco, CA 94144-4439

All progress reports to Stanford are e-mailed or mailed to:

Office of Technology Licensing

1705 El Camino Real

Palo Alto, CA 94306-1106

[\*\*\*]

Either party may change its address with written notice to the other party.

## 16 MISCELLANEOUS

16.1 **Waiver.** No term of this Agreement can be waived except by the written consent of the party waiving compliance.

16.2 **Choice of Law.** This Agreement and any dispute arising under it is governed by the laws of the State of California, United States of America, applicable to agreements negotiated, executed, and performed within California, without reference to its conflicts of laws principles.

16.3 **Exclusive Forum.** Subject to Section 14, the state and federal courts having jurisdiction over Stanford, California, United States of America, provide the

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exclusive forum for any court action between the parties relating to this Agreement. BBB submits to the jurisdiction of such courts, and waives any claim that such a court lacks jurisdiction over BBB or constitutes an inconvenient or improper forum.

16.4 **Headings.** No headings in this Agreement affect its interpretation.

16.5 **Electronic Copy.** The parties to this document agree that a copy of the original signature (including an electronic copy) may be used for any and all purposes for which the original signature may have been used. The parties further waive any right to challenge the admissibility or authenticity of this document in a court of law based solely on the absence of an original signature.

The parties execute this Agreement in duplicate originals by their duly authorized officers or representatives.

THE BOARD OF TRUSTEES OF THE LELAND  
STANFORD JUNIOR UNIVERSITY

Signature: /s/ Katharine Ku

Name: Katharine Ku

Title: Director, Technology Licensing

Date: April 12, 2012

BLUEBIRD BIO

Signature: /s/ Nick Leschly

Name: Nick Leschly

Title: Chief Executive Officer

Date: 4/2/12

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**APPENDIX A**

SAMPLE REPORTING FORM

Stanford Docket No. S97-079

This report is provided pursuant to the license agreement between Stanford University and bluebird bio, Inc.

License Agreement Effective Date: April 2, 2012

Report Covering Period	
Yearly Maintenance Fee	\$
Net Sales	\$
Royalty Calculation	
Royalty Subtotal	\$
Credit	\$
Royalty Due	\$

Comments:

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**Master Collaboration Agreement**

**by and between**

**bluebird bio, Inc.**

**and**

**Celgene Corporation**

**March 19, 2013**

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**List of Exhibits**

Exhibit A License Agreement

Exhibit B Co-Development, Co-Promote and Profit Share Agreement

[\*\*\*]

[\*\*\*]

[\*\*\*]

Exhibit F Additional Celgene Option Information

[\*\*\*]

Exhibit H Redacted Master Collaboration Agreement

Exhibit I Press Release

[\*\*\*]

[\*\*\*]

Exhibit L Call Option

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### Master Collaboration Agreement

This Master Collaboration Agreement (this “Agreement”), dated as of March 19, 2013, 2013 (the “Effective Date”), is made by and between bluebird bio, Inc., a Delaware corporation (“Bluebird”), and Celgene Corporation, a Delaware corporation (“Celgene”). Each of Bluebird and Celgene may be referred to herein as a “Party” or together as the “Parties.”

WHEREAS, Bluebird has developed and owns or has rights to certain Patents and technology relating to developing innovative gene therapies for genetic disorders;

WHEREAS, Celgene is a biopharmaceutical company focused on acquiring, Developing and Commercializing innovative anti-cancer agents; and

WHEREAS, the Parties are interested in collaborating together to research, develop and commercialize therapeutic products in the Field, all in accordance with the terms and conditions set forth below;

NOW, THEREFORE, in consideration of the mutual covenants contained herein, and for other good and valuable consideration, the amount and sufficiency of which are hereby acknowledged, the Parties hereby agree as follows:

#### 1. Definitions.

The following terms and their correlatives will have the following meanings:

1.1 “Affiliate” of a Person means any other Person which (directly or indirectly) is controlled by, controls or is under common control with such Person. A Person will be deemed to “control” another Person if it: (a) with respect to such other Person that is a corporation, owns, directly or indirectly, beneficially or legally, at least fifty percent (50%) of the outstanding voting securities or capital stock (or such lesser percentage which is the maximum allowed to be owned by such Person in a particular jurisdiction) of such other Person, or, with respect to such other Person that is not a corporation, has other comparable ownership interest; or (b) has the power, whether pursuant to contract, ownership of securities or otherwise, to direct the management and policies of such other Person.

1.2 “Baylor” means Baylor College of Medicine.

1.3 “Baylor Agreements” means (i) the Research and Collaboration Agreement (dated as of the date hereof) by and between Baylor and Celgene (“Baylor Research Agreement”), (ii) the Platform Technology License Agreement (dated as of the date hereof) by and between Baylor and Celgene (“Baylor Platform License”), and (iii) any Product License Agreement (“Baylor Product License”), in each case ((i) – (iii)) as may be amended or restated.

1.4 “Biologics License Application” or “BLA” means, with respect to a country or extra-national territory, a request for permission to introduce, distribute, sell or market a biologic product in such country or some or all of such extra-national territory, including pursuant to 21 CFR 601.2 in the U.S.

1.5 “Bluebird In-Licensed IP” means all Patents, Materials and Know-How in-licensed by Bluebird or its Affiliates during the Collaboration Program Term pursuant to Bluebird In-Licenses that are necessary or useful to perform the Collaboration Program.

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1.6 “Bluebird In-Licenses” means Pre-Existing In-Licenses and Bluebird Collaboration In-Licenses.

1.7 “Bluebird IP” means (i) Collaboration IP solely owned by Bluebird pursuant to Section 2.1(f), (ii) Bluebird In-Licensed IP and (iii) all Patents, Materials and Know-How Controlled by Bluebird or its Affiliates (other than Bluebird In-Licensed IP), in each case that is necessary or useful to perform the Collaboration Program. For avoidance of doubt, Collaboration IP jointly owned by the Parties pursuant to Section 2.1(f) will not be deemed Bluebird IP.

1.8 “Bluebird New In-License” means a New In-License between Bluebird or any of its Affiliates and a Third Party.

1.9 “Business Combination” means with respect to a Party, any of the following events: (i) any Third Party (or group of Third Parties acting in concert as a “group” within the meaning of Section 13(d) of the Exchange Act) acquires (including by way of a tender or exchange offer or issuance by such Party), directly or indirectly, beneficial ownership or a right to acquire beneficial ownership of shares of such Party representing fifty percent (50%) or more of the voting shares (where voting refers to being entitled to vote for the election of directors) then outstanding of such Party, but excluding for such purposes any transaction or series of transactions with Financial Investors made for bona fide equity financing purposes in which cash is received by Bluebird or indebtedness of Bluebird is cancelled or converted or a combination thereof; (ii) such Party consolidates with or merges into another corporation or entity which is a Third Party, or any corporation or entity which is a Third Party consolidates with or merges into such Party, in either event pursuant to a transaction in which more than fifty percent (50%) of the voting shares of the acquiring or resulting entity outstanding immediately after such consolidation or merger is not held by the holders of the outstanding voting shares of such Party immediately preceding such consolidation or merger; or (iii) such Party sells, transfers, leases or otherwise disposes of all or substantially all of its assets to a Third Party. “Financial Investor” means any investor or series of Affiliated investors whose primary business is the investment of capital for financial gain (including venture capital funds, private equity funds, pension funds and so-called “angel investors”), but in all cases excluding so-called “strategic investors” such as biotechnology companies, specialty pharmaceutical companies, pharmaceutical companies, generic pharmaceutical companies, and medical device companies and their Affiliates such as strategic venture arms.

1.10 “CAR” means chimeric antigen receptor.

1.11 “Celgene In-Licensed IP” means [\*\*\*]

1.12 “Celgene In-Licenses” means [\*\*\*]

1.13 “Celgene IP” means, collectively:

(a) “Celgene Know-How,” which means [\*\*\*]

(b) “Celgene Patents,” which means [\*\*\*]

1.14 “Celgene New In-License” means a New In-License between Celgene or any of its Affiliates and a Third Party.

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1.15 “Celgene Pre-Existing In-Licenses” means [\*\*\*]

1.16 “cGMP” means all applicable standards relating to manufacturing practices for pharmaceutical products, including (a) all applicable requirements detailed in the FDA’s current Good Manufacturing Practices regulations, 21 CFR Parts 210 and 211 and The Rules Governing Medicinal Products in the European Community, Volume IV, Good Manufacturing Practice for Medicinal Products, as each may be amended from time to time, and (b) all applicable Laws promulgated by any governmental authority having jurisdiction over the Manufacture of a Compound, Licensed Compound or Licensed Product, as applicable.

1.17 “Collaboration IP” means all Collaboration Know-How and Patents arising therefrom that Cover the Collaboration Know-How.

1.18 “Collaboration Know-How” means all Know-How and Materials discovered, created, conceived, developed or reduced to practice in the course of performing activities under the Collaboration Program (whether solely by one Party or jointly by the Parties, in each case with their Affiliates or any Third Parties or any employees, consultants or agents of any of the foregoing which perform activities under the Collaboration Program).

1.19 “Collaboration Program” means the program of research and Development in the Field that is engaged in by or on behalf of the Parties under this Agreement during the Collaboration Program Term.

1.20 “Commercially Reasonable Efforts” means, with respect to the research and Development of Product Candidates, that level of efforts and resources that such Party would normally devote to the research or Development, as the case may be, of a product owned by it or to which it has rights of the type it has hereunder, which is of a similar commercial potential at a similar stage in its lifecycle, in each case taking into account issues of safety and efficacy, product profile, the proprietary position, the then current competitive environment for such product and the likely timing of such product’s entry into the market, the pricing and launching strategy for the respective product, the regulatory environment and status of such product, and other relevant scientific, technical and commercial factors.

1.21 “Control” or “Controlled” means, with respect to any Know-How, Material or Patent, the possession (whether by ownership or license or sublicense) by a Party of the ability to use or practice such Know-How, Material, Patent, Regulatory Data, Regulatory Filings or Regulatory Approvals to perform the Collaboration Program or otherwise to grant to the other Party a license or access as provided herein to such item, without violating the terms of any agreement or other arrangement with any Third Party or, other than under the Bluebird In-Licenses, being obligated to pay any royalties or other consideration therefor (“Additional Payments”). For clarity, Bluebird New In-Licenses are not “Controlled” for purposes of this Agreement, unless and only after such Bluebird New In-License is converted into a Bluebird Collaboration In-License pursuant to Sections 4.1(b) or 4.1(d) and all required payments thereunder have been made by Celgene to Bluebird. For clarity, Celgene In-Licenses are not “Controlled” for purposes of this Agreement, unless and only after the Parties mutually agree to include such Celgene In-License in the Collaboration Program pursuant to Section 4.1(c). Notwithstanding the foregoing, if on or after the Effective Date and for such time as the other Party agrees to pay and does in fact pay all

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Additional Payments with respect to such Party’s access or license to such Know-How, Material, Patent, Regulatory Data, Regulatory Filings and Regulatory Approvals (other than that in-licensed by Bluebird pursuant to a Bluebird In-License), such Know-How, Material, Patent, Regulatory Data, Regulatory Filings and Regulatory Approvals will be deemed to be included in the definition of “Control”.

1.22 “Covers”, with reference to (i) a Patent, means that the making, using, selling, offering for sale or importing of a product or practice of a method would infringe a Valid Claim of such Patent in the country in which such activity occurs, and (ii) Materials or Know-How, means that the Manufacture, Development or Commercialization of a product incorporates, embodies or otherwise makes use of such Know-How.

1.23 “Declined Product Candidate Study” means (i) a Phase 3 Study that is intended by Bluebird (consistent with industry practice) to be submitted (together with any other registration trials that are prospectively planned when such Phase 3 Study is initiated) for Regulatory Approval of a Declined Product Candidate in the U.S. or the EU, (ii) a Phase <sup>2</sup>/<sub>3</sub> Study that is intended by Bluebird (consistent with industry practice) to be submitted (together with any other registration trials that are prospectively planned when such Phase <sup>2</sup>/<sub>3</sub> Study is initiated) for Regulatory Approval of a Declined Product Candidate in the U.S. or the EU, at such time when Bluebird obtains data from the Phase 2 portion of such Phase <sup>2</sup>/<sub>3</sub> Study and commences the Phase 3 portion of such Phase <sup>2</sup>/<sub>3</sub> Study, or (iii) any other clinical study that is designed to establish that a pharmaceutical product is safe and efficacious for its intended use, and to determine warnings, precautions, and adverse reactions that are associated with such pharmaceutical product in the dosage range to be prescribed, which clinical study is a registration trial intended to be sufficient for filing an application for a Regulatory Approval for the Declined Product Candidate in the U.S. or another country or some or all of an extra-national territory, as evidenced by the acceptance for filing for a Regulatory Approval for such product after completion of such study. For purposes of this Agreement, “completion of a Declined Product Candidate Study” means the date on which a final and complete clinical study report for the Declined Product Candidate Study, based on the complete and cleaned dataset from such Declined Product Candidate Study, which dataset includes a minimum of three (3) months follow-up of all patients in such Declined Product Candidate Study, is provided to Celgene.

1.24 “Declined Product Candidate Development Costs” means, with respect to a Declined Product Candidate, Bluebird’s FTE Costs and out-of-pocket costs directly identifiable or allocable to the Development of such Declined Product Candidate by Bluebird, its Affiliates or others working on their behalf, as applicable, following the date of Bluebird’s delivery of a Bluebird Development Notice for such Declined Product Candidate. Such costs will be calculated and allocated in accordance with methodologies based on Bluebird’s then current internal accounting systems, consistently applied, and in accordance with U.S. generally accepted accounting principles or International Financial Reporting Standards, consistently applied. For clarity, Declined Product Candidate Development Costs will include amounts paid by Bluebird to Celgene with respect to payments due under any Applicable Celgene In-License attributable to Bluebird as a sublicensee thereunder with respect to the Development of such Declined Product Candidate.

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1.25 “Development” means preclinical and clinical drug development activities, including: test method development and stability testing, toxicology, formulation, process development, qualification and validation, Manufacture scale-up, development-stage Manufacturing, quality assurance/quality control, clinical studies, statistical analysis and report writing, the preparation and submission of BLAs and MAAs, regulatory affairs with respect to the foregoing and all other activities necessary or useful or otherwise requested or required by a Regulatory Authority as a condition or in support of obtaining or maintaining a Regulatory Approval.

1.26 “Development & Commercialization Agreements” means the License Agreement attached hereto as Exhibit A and the Co-Development, Co-Promote and Profit Share Agreement attached hereto as Exhibit B.

1.27 “EMA” means the Regulatory Authority known as either the European Medicines Agency or the European Agency for the Evaluation of Medicinal Products and any successor agency thereto.

1.28 “FDA” means the United States Food and Drug Administration and any successor agency thereto.

1.29 “Field” means the use of (i) T-Cells expressing a CAR (with or without other engineering to enhance functionality and/or safety), including virus specific genetically modified T-Cells expressing a synthetic CAR, and (ii) T-Cells expressing native-virus antigen receptors or tumor-specific antigen receptors in which the T-Cells are genetically modified to enhance their performance, persistence or safety, in each case under (i) and (ii) for the treatment, modulation, palliation or prevention of cancer in humans.

1.30 “FTE” means a full-time scientific or technical person, or in the case of less than a full-time scientific or technical person, a full-time equivalent scientific or technical person year, carried out by an appropriately qualified employee of Bluebird or its Affiliates, based on 1,920 person-hours or greater per year.

1.31 “FTE Costs” means the actual FTEs employed by Bluebird or its Affiliates in the conduct of Development activities multiplied by the FTE Rate.

1.32 “FTE Rate” means [\*\*\*]

1.33 “IND” means an investigational new drug application filed with the FDA for authorization to commence clinical studies, and its equivalent in a foreign country.

1.34 “IND Product Candidate” means any Product Candidate for which an IND has been filed but for which an initial Phase 1 Study has not been completed as of the effective date of any termination or expiration of the Collaboration Program Term. For clarity, “IND Product Candidates” excludes Optioned Candidates.

1.35 “Know-How” means all commercial, technical, scientific and other know-how and information, trade secrets, knowledge, technology, methods, processes, practices, formulae, instructions, skills, techniques, procedures, experiences, ideas, technical assistance, designs, drawings, assembly procedures, computer programs, specifications, data and results (including biological, chemical, pharmacological, toxicological, pharmaceutical, physical and analytical, preclinical, clinical, safety, manufacturing and quality control data and know-how, including

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Regulatory Data, study designs and protocols), in all cases, whether or not confidential, proprietary, patented or patentable, in written, electronic or any other form now known or hereafter developed.

1.36 “Knowledge” means the actual knowledge or good faith understanding of the vice presidents, senior vice presidents, president or chief executive officer of a Party of the facts and information then in their possession.

1.37 “Law” or “Laws” means all laws, statutes, rules, regulations, orders, judgments, or ordinances having the effect of law of any federal, national, multinational, state, provincial, county, city or other political subdivision.

1.38 “license” means license or sublicense, as applicable.

1.39 “Manufacturing” means the production, manufacture, processing, filling, finishing, packaging, labeling, shipping and holding of product or any intermediate thereof, including process development, process qualification and validation, scale-up, commercial manufacture and analytic development, product characterization, stability testing, quality assurance and quality control. With reference to any Product Candidate, Manufacturing includes Vector and associated Payload supply.

1.40 “Materials” means any tangible chemical or biological material, including any compounds, DNA, RNA, clones, Vectors, Payloads, cells, and any expression product, progeny, derivative or other improvement thereto, along with any tangible chemical or biological material embodying any Know-How.

1.41 “MAA” means an application for the authorization to market a product in any country or group of countries outside the United States, as defined in the applicable Laws and filed with the Regulatory Authority of a given country or group of countries.

1.42 [\*\*\*]

1.43 “Option Fees” means the Initial Option Fee and the Additional Option Fee.

1.44 “Optioned Candidate” means a Product Candidate for which Celgene has exercised its option pursuant to Sections 5.1 or 5.7.

1.45 “Other In-Licenses” means Bluebird Collaboration In-Licenses that Celgene does not elect to include within the definition of Applicable New In-Licenses in an applicable Development & Commercialization Agreement in accordance with Section 5.8.

1.46 “Patent” means a patent or a patent application, including any additions, divisions, continuations, continuations-in-part, invention certificates, substitutions, reissues, reexaminations, extensions, registrations, supplementary protection certificates and renewals, including all U.S. and foreign counterparts thereof, but not including any rights that give rise to regulatory exclusivity periods (other than supplementary protection certificates, which will be treated as “Patents” hereunder).

1.47 “Patent Costs” means the out-of-pocket costs and expenses paid to outside legal counsel and other Third Parties (including to any licensor pursuant to any in-license), and filing and maintenance expenses, incurred in Prosecuting and Maintaining Patents and enforcing and defending them.

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1.48 “Payload” means [\*\*\*]

1.49 “Person” means any individual, partnership, joint venture, limited liability company, corporation, firm, trust, association, unincorporated organization, governmental authority or agency, or any other entity not specifically listed herein.

1.50 “Phase 1 Study” means a clinical trial of a product, the principal purpose of which is preliminary determination of safety in healthy individuals or patients as described under 21 C.F.R. §312.21(a) (as amended or any replacement thereof), or a similar clinical study prescribed by the Regulatory Authorities in a foreign country. For purposes of this Agreement, “completion of Phase 1 Study” means the date on which a final and complete clinical study report for the Phase 1 Study, based on an Initial Primary Analysis, is provided to Celgene. “Initial Primary Analysis” means, with respect to a Phase 1 Study, an analysis performed on the complete and cleaned dataset from such Phase 1 Study, which dataset includes a minimum of three (3) months follow-up of all patients in such Phase 1 Study.

1.51 “Phase 2/3 Study” means a clinical trial of a product that is (i) initiated to determine the safety and efficacy in the target patient population, as described in 21 C.F.R. 312.21(b) (as amended or any replacement thereof), or a similar clinical study prescribed by the Regulatory Authorities in a foreign country and (ii) converted to a Phase 3 Study following an interim analysis of safety and efficacy data generated from the initial patents enrolled in such clinical trial.

1.52 “Phase 3 Study” means a clinical trial of a product on a sufficient number of subjects that is designed to establish that a pharmaceutical product is safe and efficacious for its intended use, and to determine warnings, precautions, and adverse reactions that are associated with such pharmaceutical product in the dosage range to be prescribed, which trial is intended to support Regulatory Approval of such product, as described in 21 C.F.R. 312.21(c) (as amended or any replacement thereof), or a similar clinical study prescribed by the Regulatory Authorities in a foreign country. For purposes of this Agreement and the Development & Commercialization Agreements, (i) “commencement of Phase 3 Study” for a product means (a) the first dosing of such product in a human patient in a Phase 3 Study, or (b) the date on which the sponsor elects to continue enrollment of patients in a Phase 2/3 Study following an interim analysis of safety and efficacy data generated from the initial patents enrolled in such Phase 2/3 Study, and (ii) “completion of Phase 3 Study” means the final dosing of the last patient to be dosed in such Phase 3 Study.

1.53 “Pre-Existing In-Licenses” means the agreements listed in Exhibit C.

1.54 “Product Candidate” means a therapeutic candidate designed, discovered or developed as part of the Collaboration Program that comprises a T-Cell transduced with recombinant viral agent(s) encoding CAR(s) with targeting domain(s) that specifically target Target Antigen(s) and optionally encoding additional protein(s) that may modulate the efficacy and safety of such therapeutic candidate.



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1.55 “Prosecution and Maintenance” means, with regard to a particular Patent, the preparation, filing, prosecution and maintenance of such Patent, as well as re-examinations, reissues and the like with respect to that Patent, together with the conduct of interferences, the defense of oppositions and other similar proceedings with respect to that Patent.

1.56 “Regulatory Approval” means, with respect to a country or extra-national territory, any and all approvals (including BLAs and MAAs), licenses, registrations or authorizations of any Regulatory Authority necessary in order to commercially distribute, sell or market a product in such country or some or all of such extra-national territory, excluding any pricing or reimbursement approvals.

1.57 “Regulatory Authority” means any national (*e.g.*, the FDA), supra-national (*e.g.*, the EMA), regional, state or local regulatory agency, department, bureau, commission, council or other governmental authority, in any jurisdiction in the world, involved in the granting of Regulatory Approval.

1.58 “Regulatory Data” means all information with respect to a product made, collected or otherwise generated under or in connection with clinical studies and such other tests and studies in patients that are (i) required by applicable Law, or otherwise recommended by Regulatory Authorities, to obtain or maintain Regulatory Approvals, or (ii) conducted solely in support of pricing or reimbursement for such product or are not otherwise strictly required in order to obtain or maintain Regulatory Approval for such product (including epidemiological studies, modeling and pharmacoeconomic studies, post-marketing surveillance studies, investigator sponsored studies and health economics studies).

1.59 “Regulatory Filings” means any submission to a Regulatory Authority of any appropriate regulatory application together with any related correspondence and documentation, and will include any submission to a regulatory advisory board, marketing authorization application, and any supplement or amendment thereto. For the avoidance of doubt, Regulatory Filings will include any IND, BLA, MAA or the corresponding application in any other country or group of countries.

1.60 “Target Antigen” means any and all oncology associated antigens.

1.61 “T-Cell” means any of the lymphocytes that mature in the thymus and have the ability to recognize specific peptide antigens presented by major histocompatibility complex antigens through the receptors on their cell surface.

1.62 “Third Party” means any Person other than Bluebird, Celgene and their respective Affiliates.

1.63 [\*\*\*]

1.64 [\*\*\*]

1.65 “United States” or “U.S.” means the United States of America, including its territories and possessions, the District of Columbia and Puerto Rico.

1.66 “Vector” means [\*\*\*]

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Definitions for each of the following terms are found in the body of this Agreement as indicated below:

<u>Defined Term</u>	<u>Location</u>
Additional Option Fee Agreement [***]	Section 8.5 Preamble
Baylor Field [***]	Section 2.1(f)(ii)
Baylor Platform License	Section 1.3
Baylor Product License	Section 1.3
Baylor Research Agreement	Section 1.3
Bluebird	Preamble
Bluebird Acquisition	Section 2.1(e)(i)
Bluebird Business Program [***]	Section 2.1(e)(i)
Bluebird Collaboration In-License	Section 4.1(b)
Bluebird Development Notice	Section 5.7(a)
Bluebird Indemnitees	Section 11.6(a)
Bluebird Option Notice	Section 5.3
Bluebird Program Director	Section 3.1
Call Option	Section 6.9
Celgene [***] [***]	Preamble
Celgene Indemnitees	Section 11.6(b)
Celgene New In-License	Section 1.14
Celgene Option Notice	Section 5.1
Celgene Option Period	Section 5.1
Celgene Program Director	Section 3.1
Collaboration Plan	Section 2.1(a)
Collaboration Program Advisory Committee [***] [***]	Section 3.2(c)(xi)
Confidential Information	Section 10.1(a)
Corporate Event [***] [***]	Section 6.8
Declined Product Candidate	Section 5.7(a)
Disclosing Party	Section 10.1(a)
Effective Date [***]	Preamble
Financial Investor	Section 1.9

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<u>Defined Term</u>	<u>Location</u>
First Collaboration Extension Term	Section 2.1(d)
Follow-On Product Candidate	Section 5.6
HSR Act	Section 5.9(a)
HSR Clearance Date	Section 5.9(a)
HSR Filing	Section 5.9(a)
Implementation Date	Section 5.9(a)
Indemnification Claim Notice	Section 11.6(c)
Indemnified Party	Section 11.6(c)
Industry Transaction	Section 13.3
Initial Collaboration Term	Section 2.1(d)
Initial Option Fee	Section 8.4
[***]	
Issuing Party	Section 10.3(b)
JSC	Section 3.2(a)
Litigation Conditions	Section 11.6(d)(i)
Losses	Section 11.6(a)
[***]	
[***]	
New In-Licenses	Section 4.1(a)
[***]	
Party	Preamble
Patent Liaisons	Section 3.3(a)
Patent Committee	Section 3.3(a)
Phase 1 Study Data	Section 5.1
Pre-IND Product Candidate	Section 12.4(c)
Product Candidate In-License	Section 4.2
Program Directors	Section 3.1
Public Offering Submission	Section 6.6
Receiving Party	Section 10.1(a)
Release	Section 10.3(b)
[***]	
Reviewing Party	Section 10.3(b)
Second Collaboration Extension Term	Section 2.1(d)
Securities Act	Section 6.6
[***]	
Sub-Committees	Section 3.2(c)(xi)
Term	Section 12.1
Third Party Claims	Section 11.6(a)

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## **2. Collaboration Program.**

### **2.1 Collaboration Program.**

(a) *General.* During the Collaboration Program Term, the Parties will conduct the Collaboration Program on the terms and conditions set forth in this Agreement to identify, research and Develop Product Candidates. [\*\*\*] Under the Collaboration Program, Bluebird will be responsible for all research and Development activities performed through completion of the initial Phase 1 Study with respect to each Product Candidate, and Celgene will be a critical advisor for oncology drug development, ex vivo human cell processing, assay development and release testing. Bluebird will keep Celgene reasonably informed of Bluebird’s research and Development activities and will reasonably consult with Celgene and reasonably consider Celgene’s comments and advice with respect to all material decisions relating to such activities. Research and Development activities of the Parties with respect to the Collaboration Program will be described in a “Collaboration Plan,” an initial version of which is attached hereto as Exhibit D. Any modifications or amendments to the Collaboration Plan that are proposed by either Party will be subject to review by the JSC pursuant to and in accordance with the terms of Section 3.2(d) and to the prior written approval of both Parties. The specific Target Antigens that will be the focus of the Collaboration Program will be defined as soon as practicable [\*\*\*], and will be set forth in a Collaboration Plan amendment. The selection of Product Candidates for additional work under the Collaboration Program will be subject to the oversight and supervision of the JSC, provided that if the JSC is unable to unanimously agree with respect to the selection of a Product Candidate for additional work under the Collaboration Program, either Party may, by written notice to the other Party, have such dispute referred to the Bluebird CEO and the Celgene CEO or in either case his or her designee (who will be a senior executive), who will attempt in good faith to resolve such dispute by negotiation and consultation [\*\*\*], and if not so resolved, Bluebird will have the tie-breaking vote, provided that if a Business Combination has occurred with respect to Bluebird, Celgene will have the tie-breaking vote.

(b) *Obligations Under the Collaboration Plan.* Each Party will use Commercially Reasonable Efforts to perform (itself or through its Affiliates or by permitted subcontracting pursuant to Section 2.4) its respective obligations under the Collaboration Plan, and will cooperate with and provide reasonable support to the other Party in such other Party’s performance of its responsibilities under the Collaboration Plan. The Collaboration Plan will not assign to Celgene, and Bluebird will not request that Celgene perform, any research or Development activity that would require a sublicense under any Bluebird In-License. If, notwithstanding the foregoing, the Collaboration Plan assigns to Celgene, or Bluebird requests that Celgene perform, any such research or Development activity, Bluebird will be responsible for any and all obligations to its licensors under any Bluebird In-License that arise out of such research or Development. The Parties acknowledge and agree, however, that no outcome or success is or can be assured and that failure to achieve desired results will not in and of itself constitute a breach or default of any obligation in this Agreement (notwithstanding the focus of the Collaboration Program described above).

(c) *Celgene Manufacturing.* In the event the Parties mutually agree that, as a part of the Collaboration Program, Celgene will build and operate a cGMP suite for the processing of Product Candidates which incorporate Vectors and associated Payloads supplied by Bluebird, the

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Parties will enter into such additional agreements as may be necessary for Celgene to do so, including a Vector and Payload supply agreement. Prior to or during initial proof of concept studies, Celgene and Bluebird will mutually assess the capability for sole supply manufacture of Vector Supply, and agree to provisions to ensure the Manufacture and distribution, of Vector Supplies, in adequate quantities, of adequate quality, and in acceptable timeframes so as to not delay clinical Development and Commercialization of Product Candidates. Multiple sites may be required to supply and store inventories of Vector Supplies.

(d) *Collaboration Program Term.* Unless terminated or extended pursuant to the terms hereof, the term of the Collaboration Program will commence on the Effective Date and continue for an initial period of three (3) years (the “Initial Collaboration Term”). Celgene may elect to extend the Collaboration Program for (i) first, one additional two (2) year term (the “First Collaboration Extension Term”) and (ii) then next for one additional [\*\*\*] term (the “Second Collaboration Extension Term”, and together with the Initial Collaboration Term and the First Collaboration Extension Term, if any, the “Collaboration Program Term”) by providing written notice to Bluebird of such election at least [\*\*\*] prior to the expiration of the Initial Collaboration Term or First Collaboration Extension Term, as applicable, and payment of the applicable extension fees set forth in Section 8. Absent further agreement of the Parties, the maximum Collaboration Program Term (assuming Celgene elects both of the foregoing extensions) is [\*\*\*].

(e) *Relationship.*

(i) During the Collaboration Program Term, neither Bluebird nor its Affiliates (nor any others on behalf of or with, or under license (including a covenant not to sue) or sublicense from, Bluebird or any its Affiliates) will research, Develop, Manufacture or commercialize any actual or potential products (including Vectors and associated Payloads) to be used in the Field other than as a part of the Collaboration Program, and other than with respect to Declined Product Candidates in accordance with Section 5.7. Notwithstanding this Section 2.1(e)(i), if (i) a Business Combination occurs with respect to Bluebird with a Third Party or (ii) Bluebird acquires a Third Party (including by a merger or consolidation) so that such Third Party becomes an Affiliate over which Bluebird has control (as defined in Section 1.1), or (iii) Bluebird acquires all or substantially all of the assets of a Third Party (including any subsidiaries or divisions thereof) (each of (i), (ii) and (iii), a “Bluebird Acquisition”), and, in each case, the Third Party (or any of such Third Party’s Affiliates or any successors or assigns of such Third Party or such Third Party’s Affiliates, other than Bluebird and its Affiliates as of the Bluebird Acquisition) (1) already has, or the acquired assets contain, as applicable, a program that existed prior to, or was planned prior to and is demonstrably to be implemented shortly after, the Bluebird Acquisition or (2) initiates and pursues a new program following such Bluebird Acquisition, in each case that would otherwise violate this Section 2.1(e)(i) (a “Bluebird Business Program”), then such Third Party (or any of such Third Party’s Affiliates or any successors or assigns of such Third Party or such Third Party’s Affiliates, other than Bluebird and its Affiliates as of the Bluebird Acquisition), as applicable, will be permitted to initiate, pursue and continue such Bluebird Business Program after such Bluebird Acquisition and such initiation, pursuit and continuation will not constitute a violation of this Section 2.1(e)(i); provided that (A) none of the Collaboration IP or other Patents, Materials or Know-How

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Controlled by the other Party and, in each case, licensed to Bluebird will be used in the Bluebird Business Program, and (B) the research or Development activities required under this Agreement will be conducted separately from any research or Development activities directed to such Bluebird Business Program, including the maintenance of separate lab notebooks and records (password-protected to the extent kept on a computer network) and separate personnel working on each of the activities under this Agreement and the activities covered under such Bluebird Business Program.

(ii) [\*\*\*]

(f) *Collaboration Know-How and IP.*

(i) Each Party will promptly (and at least on a calendar quarterly basis) disclose to the other Party any Collaboration Know-How discovered, created, conceived, developed or reduced to practice by or on behalf of such Party, and will provide the other Party such documentation regarding the same as the other Party may reasonably request.

(ii) Except as set forth in this Section 2.1(f)(ii) and in Section 2.1(f)(iv) below, each Party will solely own all right, title and interest in and to all Collaboration IP that is discovered, created, conceived, developed or reduced to practice solely by or on behalf of such Party, and all right, title and interest in and to all Collaboration IP will automatically vest solely in such Party. [\*\*\*] Each Party agrees to execute such written assignments and confirmations as are necessary to effect the allocation of ownership of Patents, Know-How and Materials as provided in the immediately preceding sentence, and any Patents, Know-How and Materials addressed by the immediately preceding sentence (other than clause (C)(2)) shall be considered Collaboration IP. [\*\*\*]

(iii) Except as set forth in Section 2.1(f)(iv) below, the Parties will jointly own any and all Collaboration IP that is discovered, created, conceived, developed or reduced to practice jointly by or on behalf of the Parties. Each Party will have an undivided one-half interest in and to such jointly-owned Collaboration IP. Each Party will exercise its ownership rights in and to such jointly-owned Collaboration IP, including the right to license and sublicense or otherwise to exploit, transfer or encumber its ownership interest, without an accounting or obligation to, or consent required from, the other Party, but subject to the licenses hereunder and the other terms and conditions of this Agreement, including Section 2.1(e). At the reasonable written request of a Party, the other Party will in writing grant such consents and confirm that no such accounting is required to effect the foregoing regarding jointly-owned Collaboration IP. Each Party, for itself and on behalf of its Affiliates, licensees and sublicensees, and employees, subcontractors (subject to Section 2.4), consultants and agents of any of the foregoing, hereby assigns (and to the extent such assignment can only be made in the future hereby agrees to assign), to the other Party a joint and undivided interest in and to all jointly-owned Collaboration IP.

(iv) Notwithstanding the first sentence of Section 2.1(f)(ii) and notwithstanding Section 2.1(f)(iii), but subject to the second sentence of Section 2.1(f)(ii), (A) Celgene will solely own any Collaboration IP that is an improvement to, or modification or derivative work of, any Celgene IP, and Bluebird, for itself and on behalf of its Affiliates, licensees and sublicensees, and employees, subcontractors (subject to Section 2.4), consultants

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and agents of any of the foregoing, hereby assigns (and to the extent such assignment can only be made in the future hereby agrees to assign), all of its rights, title and interest in such Collaboration IP to Celgene, and (B) Bluebird will solely own any Collaboration IP that is an improvement to, or modification or derivative work of, any Bluebird IP, and Celgene, for itself and on behalf of its Affiliates, licensees and sublicensees, and employees, subcontractors (subject to Section 2.4), consultants and agents of any of the foregoing, hereby assigns (and to the extent such assignment can only be made in the future hereby agrees to assign), all of its rights, title and interest in such Collaboration IP to Bluebird. To the extent that a particular item of Collaboration IP constitutes an improvement to, or modification or derivative work of, both Celgene IP and Bluebird IP, the Parties will jointly own such particular item of Collaboration IP pursuant to Section 2.1(f)(iii).

(v) Invention determination for all Patents worldwide arising from any Know-How or Material discovered, created, conceived, developed or reduced to practice by or on behalf of the Parties under or in connection with this Agreement and thus the ownership thereof will be made in accordance with applicable United States patent laws.

(g) *Regulatory*. Bluebird will exclusively own the INDs for the Development of Product Candidates and will, after reasonable consultation with Celgene under the oversight of the JSC: (i) determine the regulatory plans and strategies for Product Candidates, (ii) prepare and file all Regulatory Filings with respect to Product Candidates, and (iii) be responsible for conducting all meetings with Regulatory Authorities in connection with the Development of Product Candidates, in each case unless and until such time that such Product Candidate becomes an Optioned Candidate. Bluebird will provide Celgene with reasonable prior notice of all such meetings with Regulatory Authorities, and Celgene will have the right to participate in such meetings.

(h) *Licenses*.

(i) During the Term, Bluebird hereby grants to Celgene the co-exclusive (with Bluebird and its Affiliates), worldwide, royalty-free right and license in the Field, without the right to grant sublicenses (other than to permitted subcontractors under Section 2.4), under Collaboration IP solely owned by Bluebird pursuant to Section 2.1(f) and Bluebird’s interest in jointly owned Collaboration IP, in each case solely to conduct research and Development under the Collaboration Plan as part of the Collaboration Program in accordance with the terms of this Agreement. Except as set forth in Section 12.4(c) or as may be permitted under an applicable Development & Commercialization Agreement, Celgene will not practice or otherwise use any Collaboration IP solely owned by Bluebird pursuant to Section 2.1(f) other than in accordance with the license granted in this Section 2.1(h)(i).

(ii) Subject to the terms and conditions of this Agreement, during the Term and thereafter, Celgene hereby grants to Bluebird a worldwide, fully paid-up, non-exclusive license, with the right to sublicense through multiple tiers, under (A) Collaboration IP solely owned by Celgene pursuant to Section 2.1(f), (B) all improvements to, or modifications or derivative works of, any Bluebird IP that are discovered, created, conceived, developed or reduced to practice by or on behalf of Celgene or its Affiliates during the Collaboration Program Term in the course of Developing an Optioned Candidate under a Development &

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Commercialization Agreement, and [\*\*\*], in each case of (A) through (C), that are related to the Manufacture of Vectors, solely to research, Develop, Manufacture and commercialize Vectors, provided that (w) the foregoing license does not include any Patents and Know-How for Manufacturing (other than Manufacturing of Vectors), (x) [\*\*\*] (y) during the Term and the term of any applicable Development & Commercialization Agreement, the foregoing license does not include the right to research, Develop, Manufacture or commercialize any Vectors that are used in connection with Optioned Candidates or Elected Candidates or Licensed Products under such Development & Commercialization Agreement, other than with and for Celgene, and (z) [\*\*\*]. Further, the Parties acknowledge and agree that, upon written notice to Celgene, Bluebird may decline the taking of or terminate such sublicense from Celgene with respect to any Patents, Know-How or Materials that are in-licensed by Celgene pursuant to a Celgene New In-License that is an Applicable Celgene In-License. [\*\*\*]

(i) *Celgene IP*. If either Party desires that Celgene make available any Patents, Know-How or Material Controlled by Celgene or its Affiliates (other than pursuant to a Celgene In-License, which is governed by Section 4.1(c), and other than Collaboration IP) for use in the Collaboration Program, such Party will notify the JSC and the JSC will discuss whether or not such Patents, Know-How or Materials would be useful for the Collaboration Program. If the JSC concludes that such Patents, Know-How or Materials would be useful for the Collaboration Program, the JSC will invite Celgene to make such intellectual property available to the Collaboration Program. Celgene will have sole discretion whether or not to make such intellectual property available to the Collaboration Program, and if Celgene so elects it will make such intellectual property available by providing the JSC with written notice specifying the Patents, Know-How and/or Materials that will be made available to the Collaboration Program as “Celgene IP”. Except by such written notice provided to the JSC, no Patents, Know-How or Materials Controlled by Celgene or its Affiliates (other than pursuant to a Celgene In-License, which is governed by Section 4.1(c) and other than Collaboration IP) will be made available for, or used in, the Collaboration Program, and no such Patents, Know-How or Materials shall be considered “Celgene IP”.

2.2 Collaboration Program Expenses. Except for any amounts that may be payable by Celgene under a Vector and associated Payload supply agreement described in Section 2.1(c), each of Bluebird and Celgene is and will remain solely responsible for all of its internal costs and expenses that are incurred by or on its behalf in connection with the performance of the Collaboration Plan. Subject to Sections 4.1, 4.2, 8.6 and 9.2, and except for any amounts that may be payable by Celgene under a Vector and associated Payload supply agreement described in Section 2.1(c) or a Celgene In-License, Bluebird will be responsible for all out-of-pocket costs and expenses payable to Third Parties in connection with the performance of the Collaboration Plan.

2.3 Collaboration Program Records, Reports and Materials.

(a) *Records*. Each Party will maintain, or cause to be maintained, records of its activities under the Collaboration Program in sufficient detail and in good scientific manner appropriate for scientific, Patent and regulatory purposes, that will properly reflect all work included in the Collaboration Program, for a period of at least ten (10) years after the creation of such records, but in no event less than required by applicable Laws. Each Party will have the right to request and receive a copy of any such records.



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(b) *Collaboration Program Reports*. Each Party will furnish to the JSC a high-level summary written report within thirty (30) days after each June 30<sup>th</sup> and December 31<sup>st</sup> occurring during the Collaboration Program Term, describing its progress under the Collaboration Plan as part of the Collaboration Program during the previous six (6) month period. Each Party agrees that it will promptly respond to the other Party’s reasonable questions regarding any of such Party’s reports.

(c) *Materials*.

(i) Each Party will, during the Collaboration Program Term, as a matter of course as described in the Collaboration Plan or upon the other Party’s reasonable written request, furnish to each other samples of Materials that are in such Party’s Control and are necessary for the other Party to carry out its responsibilities under the Collaboration Plan, provided that, prior to Celgene providing any Materials to Bluebird, Celgene will notify Bluebird of the cost of such Materials and Bluebird may elect whether or not to receive such Materials from Celgene. Subject to the foregoing, after Celgene has provided Materials costing more than [\*\*\*], Bluebird will reimburse Celgene for the costs of any additional Materials.

(ii) Each Party will use such Materials only in accordance with the Collaboration Plan and otherwise in accordance with the terms and conditions of this Agreement and any instructions provided by the Party furnishing the Materials. Except with the prior written consent of the supplying Party (such consent not to be unreasonably withheld, delayed or conditioned), the Party receiving any Materials will not distribute or otherwise allow the release of Materials to any Affiliate (other than wholly-owned subsidiaries) or Third Party, except for subcontracting as permitted hereunder. All Materials delivered to the receiving Party will remain the sole property of the supplying Party and will be used in compliance with all applicable Law. The Materials supplied under this Agreement will be used with prudence and appropriate caution in any experimental work because not all of their characteristics may be known.

2.4 Permitted Subcontracting. Each Party may subcontract any of its activities to be performed under the Collaboration Plan to an Affiliate or Third Party, provided that any such Affiliate or Third Party will have entered into a written agreement with such Party that includes terms and conditions protecting and limiting use and disclosure of Confidential Information and Materials and Know-How at least to the same extent as under this Agreement, and requiring such Affiliate or Third Party and its personnel to assign to such Party all right, title and interest in and to any Patents, Know-How and Materials created, conceived or developed in connection with the performance of subcontracted activities to the extent required to research, Develop, Manufacture and commercialize Product Candidates, provided that with respect to Third Parties that are academic or other non-commercial Persons, a Party will be required only to use commercially reasonable efforts to obtain such assignment, and in the absence of such assignment, the Parties will mutually agree on the rights (e.g., a license or option to license) to be obtained from such academic or non-commercial Persons. Any such subcontracting activities will be described in the reports for the Collaboration Program required by Section 2.3(b).

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**3. Governance.**

3.1 Management. Management of the Collaboration Program activities will be under the responsibility of one person to be designated by Celgene (the “Celgene Program Director”) and one person to be designated by Bluebird (the “Bluebird Program Director,” and together with the Celgene Program Director, the “Program Directors”).

3.2 Joint Steering Committee.

(a) *Steering Committee.* As soon as practicable (but not later than sixty (60) days) following the Effective Date, the Parties will establish a Joint Steering Committee, comprised of three (3) representatives of Bluebird and three (3) representatives of Celgene (the “JSC”). Each Party may replace its representatives on the JSC or its Program Director at any time upon written notice to the other Party. With the consent of the other Party (such consent not to be unreasonably withheld, delayed or conditioned), each Party may invite non-voting employees and consultants to attend meetings of the JSC, subject to their agreement to be bound to the same extent as a permitted subcontractor under Section 2.4.

(b) *Meetings.* While in existence, the JSC will meet each calendar quarter and, at a minimum, two (2) of such meetings each calendar year starting in 2013 will be in person (which in-person meeting will be held at locations mutually agreed by the Parties). Meetings of the JSC will be effective only if at least one (1) representative of each Party is present or participating. Each Party will be responsible for all of its own expenses of participating in the meetings. The Parties will endeavor to schedule meetings of the JSC at least six (6) months in advance. Bluebird will prepare and circulate a meeting agenda prior to each such meeting. The Parties will alternate in preparing written minutes of such meeting, and the preparing Party will circulate such minutes within fifteen (15) days after such meeting. The Parties will agree on the minutes of each meeting promptly, but in no event later than the next meeting of the JSC.

(c) *Responsibilities.* The JSC will oversee and supervise the overall performance of the Collaboration Plan and within such scope will:

- (i) Periodically review the Parties’ efforts and progress under the Collaboration Plan;
- (ii) Review the Collaboration Program;
- (iii) Review any proposed modifications or amendments to the Collaboration Plan and the Collaboration Program;
- (iv) Prioritize and oversee execution of specific activities to be performed under the Collaboration Plan and the Collaboration Program;
- (v) Review Patent Committee advice with regard to scientific activities to be performed under the Collaboration Plan and the Collaboration Program;
- (vi) Review Collaboration Program Advisory Committee advice with regard to scientific activities to be performed under the Collaboration Plan and the Collaboration Program;
- (vii) Review and select Product Candidates for additional work as part of the Collaboration Program;

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(viii) Review and evaluate Product Candidates for which Development work should be performed as part of the Collaboration Program;

(ix) Review and approve of the regulatory plans and strategies for Product Candidates;

(x) Review all Regulatory Filings with respect to Product Candidates;

(xi) Form such other committees (“Sub-Committees”) as the JSC may deem appropriate. As soon as practicable (but not later than sixty (60) days) after the Effective Date, the Parties will establish a Sub-Committee comprised of three (3) representatives of Celgene, three (3) representatives of Bluebird, and Dr. Malcolm K. Brenner (the “Collaboration Program Advisory Committee”). The Collaboration Program Advisory Committee will monitor and advise the Parties on the conduct and progress of the Collaboration Program. Each Party may replace its representatives on the Collaboration Program Advisory Committee at any time upon written notice to the other Party. Any such Sub-Committee (including the Collaboration Program Advisory Committee) may make recommendations to the JSC but may not be delegated JSC decision-making authority;

(xii) Address such other matters relating to the activities of the Parties under this Agreement as either Party may bring before the JSC, including any matters that are expressly for the JSC to decide as provided in this Agreement; and

(xiii) Attempt to resolve any disputes on an informal basis.

(d) *Decision-making*. The three (3) JSC representatives of each Party will collectively have one (1) vote, and the JSC will make decisions only by unanimous consent in the sole discretion of each Party with respect to its vote. [\*\*\*]

(e) *Limits on JSC Authority*. Each Party will retain the rights, powers and discretion granted to it under this Agreement and no such rights, powers, or discretion will be delegated to or vested in the JSC unless such delegation or vesting of rights is expressly provided for in this Agreement or the Parties expressly so agree in writing. The JSC will not have the power to, nor will the Party having the tie-breaking vote in the JSC have the power to (i) amend, modify or waive compliance with this Agreement (other than as expressly permitted hereunder), (ii) alter, increase or expand the Parties’ rights or obligations under this Agreement, (iii) determine that a Party has fulfilled any obligations under this Agreement or that a Party has breached any obligation under this Agreement, (iv) make a decision that is expressly stated to require the mutual agreement of the Parties, (v) amend or modify the Collaboration Plan, (vi) change the Collaboration Program in any manner that would alter the fundamental objectives of the Collaboration Program as generally described in Section 2.1(a), or (vi) determine that milestone events required for the payment of milestone payments have or have not occurred.

(f) *Term*. The JSC and any subcommittees thereof will cease to exist three (3) months after the end of the Collaboration Program Term.

### 3.3 Patent Committee.

(a) Promptly (but no later than sixty (60) days) after the Effective Date, the Parties will (i) each designate representative(s) to consult with the other Party’s representative(s) with

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respect to Patent ownership, Prosecution and Maintenance, enforcement and defense matters (the “Patent Liaisons”), and (ii) establish a patent committee (the “Patent Committee”). The purpose of the Patent Committee is to determine ownership of intellectual property, and facilitate the discussion and coordination of Prosecution and Maintenance, enforcement and defense matters, in accordance with and subject to the terms of this Agreement. The Patent Liaisons will be the primary point of contact for the Parties regarding the foregoing activities and will facilitate all such activities hereunder, including preparing and finalizing minutes of the Patent Committee and will be responsible for assisting the Patent Committee in performing its oversight responsibilities.

(b) Decisions. All decisions of the Patent Committee will be made by consensus, with each Party having one vote. If the Patent Committee cannot agree on a matter within the Patent Committee’s authority within five (5) days after it has met and attempted to reach such decision, then, either Party may, by written notice to the other, have such issue referred to the Program Directors for resolution. The Parties’ respective Program Directors will meet within five (5) days after such matter is referred to them, and will negotiate in good faith to resolve the matter. If the Program Directors are unable to resolve the matter within five (5) days after the matter is referred to them, then the decision will be resolved as set forth below:

(i) IP Ownership. The Patent Committee will determine ownership of Collaboration IP in accordance with and subject to the terms of Section 2.1(f); provided that the Patent Committee may allocate ownership of a particular item of intellectual property to improve the prospects of obtaining patent protection with respect to such item of intellectual property, even if such allocation is not in accordance with the terms of Section 2.1(f), so long as the Parties mutually agree to such allocation. In the event the Patent Committee cannot agree on a matter regarding ownership of an item of intellectual property, and the Program Directors are unable to resolve such matter, then such dispute will be resolved by a Third Party patent counsel selected by the Patent Committee who (and whose firm) is not, and was not at any time during the five (5) years prior to such dispute, an employee, consultant, legal advisor, officer, director or stockholder of, and does not have any conflict of interest with respect to, either Party. Such patent counsel will determine ownership of such intellectual property in accordance with U.S. patent law and Section 2.1(f). Expenses of the patent counsel will be shared equally by the Parties.

(ii) Patent Prosecution. The Patent Committee will discuss material issues and provide input to each other regarding the Prosecution and Maintenance, enforcement and defense of Bluebird IP, Celgene IP and jointly owned Collaboration IP. The Patent Liaisons will be responsible for coordinating the implementation of each Party’s strategies for the protection of the foregoing intellectual property rights related to Product Candidates. All final decisions related to the Prosecution and Maintenance, enforcement or defense of any Bluebird IP, Celgene IP and jointly-owned Collaboration IP will be made by the Party with the right to control such Prosecution and Maintenance, enforcement or defense, as applicable, as set forth in Section 9.

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**4. Third Party Licenses.**

**4.1 New Licenses.**

(a) *Identification.* [\*\*\*]

(b) *Bluebird Contribution to the Collaboration.* [\*\*\*]

(c) *Celgene Applicable/New In-Licenses.* With respect to each Applicable Celgene In-License that is a Celgene New In-License:

(i) Celgene will be solely responsible for any upfront payment payable to the licensor under such Applicable Celgene In-License.

(ii) Except as provided in Sections 2.1(h)(ii), 5.7 and 12.4, Celgene and Bluebird will each be responsible for [\*\*\*] any other payments required to be paid to the licensor under such Applicable Celgene In-License in respect of Collaboration Program activities or the research, Development, Manufacture or commercialization of Product Candidates, but excluding any payments that are (a) triggered by the grant of a sublicense under the Applicable Celgene In-License (other than sublicenses granted by Bluebird or its sublicensees), (b) annual fees paid to maintain the Applicable Celgene In-License in effect, (c) Patent Costs, (d) any payments that are royalty payments (including sales-based milestone payments), and (e) payments resulting from Celgene’s breach of the Applicable Celgene In-License not attributable to Bluebird or its contract Third Parties or sublicensees, which excluded payments will be the sole responsibility of Celgene; provided that Bluebird’s [\*\*\*] share of such payments will become due and payable upon the execution of the first Development & Commercialization Agreement, and will be paid as follows: [\*\*\*]

(iii) Any payments that are royalties payable by Celgene or its Affiliates under the Applicable Celgene In-License will be subject to Section 4.3(d) of such License Agreement or Section 11.3(d) of any Co-Development, Co-Promote and Profit Share Agreement, as applicable.

(f) *Celgene Pre-Existing/Applicable In-Licenses.* With respect to any Applicable Celgene In-License that is a Celgene Pre-Existing In-License, except as provided in Sections 2.1(h)(ii), 5.7 and 12.4, Celgene will be solely responsible for all payments required to be paid to the licensor under such Applicable Celgene In-License, and any payments that are royalties payable by Celgene or its Affiliates under the Applicable Celgene In-License will be subject to Section 4.3(d) of such License Agreement or Section 11.3(d) of any Co-Development, Co-Promote and Profit Share Agreement, as applicable.

**4.2 Product Candidate In-Licenses.** Other than with respect to Baylor as contemplated by the Baylor Agreements, which are governed by Sections 4.5 and 5.5 hereof, in the event that the Parties desire to enter into an agreement with any Third Party to obtain rights to Patents, Know-How or Materials that would constitute solely a new Product Candidate (if developed pursuant to this Agreement) in the Field, as opposed to only being necessary or useful for supporting research, Development or Commercialization of existing Product Candidates (a “Product Candidate In-License”), the Parties will jointly determine a strategy for endeavoring to procure rights under such Patents, Know-How or Materials, including with respect to allocation of the Parties’ responsibilities for any payments that may become due during the Collaboration Program Term under such Product Candidate In-License. Any such Product Candidate

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In-License addressing any such new Product Candidate will require the prior written approval of both Parties, will be with both Parties and will be committed to the Collaboration Program (and not the Parties on an individual basis). Accordingly, any product candidate in-licensed pursuant to a Product Candidate In-License will be a “Product Candidate” hereunder, and will only be Developed or commercialized by either Party as a part of the Collaboration Program or under an executed Development and Commercialization Agreement, unless and until such Product Candidate becomes a Declined Product Candidate in accordance with Section 5.7. If the Parties agree that any Patents, Know-How or Materials in-licensed under a Product Candidate In-License will be used to Develop and commercialize a Product Candidate under a Development and Commercialization Agreement, the Parties will discuss in good faith and agree on the allocation of the Parties’ applicable rights and obligations thereto, including with respect to amounts payable under such Product Candidate In-License (other than a Baylor Product License), which terms will be set forth in such Development and Commercialization Agreement. If an in-license from a Third Party of rights to Patents, Materials or Know-How that would constitute a new Product Candidate also includes other rights that potentially have broader applicability (e.g., that may be useful for supporting research, Development or commercialization of Product Candidates that are against Target Antigens different than the Target Antigen in the Product Candidate in such Third Party in-license), such in-license will be treated as a “Product Candidate In-License” hereunder and the Parties will discuss in good faith the allocation of such other rights and obligations, along with costs, in accordance with the principles set forth in Section 4.1 and this Section 4.2. The Parties acknowledge that the terms of this Section 4.2 may need to be discussed and modified with respect to any particular Product Candidate In-License (other than a Baylor Product License) depending on the then existing facts and circumstances relating to such Product Candidate In-License.

4.3 Maintenance of Bluebird In-Licenses. Bluebird (i) will duly perform and observe all of its obligations under the Bluebird In-Licenses in all material respects and maintain in full force and effect the Bluebird In-Licenses, and (ii) will not, without Celgene’s prior written consent (such consent not to be unreasonably withheld, conditioned or delayed), (a) amend, modify, restate, cancel, supplement or waive any provision of any Bluebird In-License, or grant any consent thereunder, or agree to do any of the foregoing, or (a) exercise any right to terminate any Bluebird In-License, in each case ((a) and (b)) that would reasonably be expected to adversely affect in any respect the rights of Celgene under this Agreement or any potential or executed Development & Commercialization Agreement. Bluebird will provide Celgene with written notice as promptly as practicable (and in any event within five (5) business days) after becoming aware of any of the following: (1) any material breach or default by Bluebird or any of its Affiliates of any covenant, agreement or other provision of any Bluebird In-License, (2) any notice or claim from the counterparty to any Bluebird In-License terminating or providing notice of termination of any Bluebird In-License, (3) any notice or claim alleging any breach of default under any Bluebird In-License, or (4) the existence of any facts, circumstances or events which alone or together with other facts, circumstances or events would reasonably be expected (with or without the giving of notice or passage of time or both) to give rise to a breach of or default under or right to terminate any Bluebird In-License. If Bluebird fails to pay any amounts due under any Bluebird In-License and if such nonpayment would permit the counterparty to such Bluebird In-License to terminate or suspend the same or any rights thereunder, Celgene will have

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the right, but not the obligation, in its sole discretion, to pay such amounts on Bluebird’s behalf, and any amounts so paid by Celgene may be taken by Celgene as a credit against any amounts payable to Bluebird under this Agreement or any Development & Commercialization Agreement.

4.4 [\*\*\*]

4.6 No Implied Rights. No license, sublicense or other right is or will be created or granted hereunder by implication, estoppel or otherwise. Any licenses, sublicenses or rights will be granted only as expressly provided in this Agreement or any executed Development & Commercialization Agreement.

## **5. Option for Licensed Candidates.**

5.1 Option Period. Bluebird will provide Celgene with all safety and efficacy data generated with respect to a Product Candidate in an initial Phase 1 Study and all correspondence to and from any Regulatory Authority regarding such Product Candidate (collectively, “Phase 1 Study Data”), as soon as reasonably practicable following Bluebird’s receipt of same. From the period commencing at the Effective Date and ending, on a Product Candidate-by-Product Candidate basis, [\*\*\*] following the completion of an initial Phase 1 Study with respect to such Product Candidate (the “Celgene Option Period”), Celgene will have the exclusive option to take a license to each Product Candidate, provided that if Celgene reasonably requests any additional data or information for such Product Candidate within the Celgene Option Period set forth above, the Celgene Option Period will be extended for an additional [\*\*\*] after Celgene’s receipt of such additional data or information. Bluebird shall deliver to Celgene no later than [\*\*\*] following the completion of an initial Phase 1 Study with respect to such Product Candidate, the Schedule referred to in Section 16.2 of the Co-Development, Co-Promote and Profit Share Agreement or Section 9.2 of the License Agreement. Celgene may exercise such option by providing to Bluebird, prior to the expiration of the Celgene Option Period, (i) written notice that a Product Candidate is selected by Celgene to be an Optioned Candidate hereunder, and (ii) the additional information set forth in Exhibit F (collectively, the “Celgene Option Notice”). A separate Celgene Option Notice and Initial Option Fee will be required for each Product Candidate optioned by Celgene pursuant to this Section 5.1, and Celgene will pay to Bluebird the Initial Option Fee for each such Optioned Candidate as set forth in Section 8.4. Subject to Section 5.7 and Section 12.4, if not exercised prior to the expiration of the Celgene Option Period, the option and other rights granted to Celgene under this Section 5 with respect to a Product Candidate will terminate in full and will no longer be exercisable.

5.2 Celgene’s Exercise of Option. Within [\*\*\*] of Celgene’s delivery of a Celgene Option Notice to Bluebird, Celgene (or an Affiliate designated by Celgene) and Bluebird will enter into a License Agreement in the form attached hereto as Exhibit A with respect to such Optioned Candidate (updating the appendices thereto), modified, if appropriate, as provided in Sections 4.2 or 5.5, and subject to Section 5.9. Upon execution of such License Agreement, such Optioned Candidate will be an “Elected Candidate” thereunder.

5.3 Co-Promotion/Co-Development Option Exercise. Within [\*\*\*] following Celgene’s delivery of a Celgene Option Notice to Bluebird, and subject to Section 5.9, Bluebird may exercise an option, by delivery of written notice to Celgene (the “Bluebird Option Notice”) to

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co-promote and co-Develop the Optioned Candidate in the U.S. as set forth in the Co-Development, Co-Promote and Profit Share Agreement attached hereto as Exhibit B, provided that with respect to a Baylor-Only Candidate for which Celgene has delivered a Celgene Option Notice, such option will end on the earlier of (i) [\*\*\*] following Celgene’s commencement of a Pivotal Study (as defined in the License Agreement) for such Baylor-Only Candidate, and (ii) the date that Bluebird delivers written notice to Celgene that Bluebird is declining to exercise such option. Prior to the expiration of such option for a Baylor-Only Candidate, upon Bluebird’s written request, Celgene will provide Bluebird with (a) a reasonably detailed accounting of any payments made or other actions taken by Celgene pursuant to the License Agreement executed pursuant to Section 5.2 that would be the responsibility of Bluebird under the Co-Development, Co-Promote and Profit Share Agreement, including, for avoidance of doubt, costs incurred by Celgene in Developing such Baylor-Only Candidate through and including the Pivotal Study for such Baylor-Only Candidate, and (b) all safety and efficacy data in Celgene’s possession as of the date of such request generated with respect to such Baylor-Only Candidate in all clinical studies conducted by Celgene for such Baylor-Only Candidate, all correspondence to and from any Regulatory Authority in Celgene’s possession as of the date of such request regarding such Baylor-Only Candidate, and any other information relating to such Baylor-Only Candidate reasonably requested by Bluebird and in Celgene’s possession as of the date of such request. In the event that Bluebird exercises such option, the Parties will promptly, but in any event within [\*\*\*], terminate the License Agreement executed pursuant to Section 5.2 with respect to such Optioned Candidate, and enter into a Co-Development, Co-Promote and Profit Share Agreement in the form attached hereto as Exhibit B with respect to such Optioned Candidate, with appropriate amendments to reflect and reimburse Celgene for any payments made or other actions taken by Celgene pursuant to the License Agreement executed pursuant to Section 5.2 that are the responsibility of Bluebird under the Co-Development, Co-Promote and Profit Share Agreement, including, for avoidance of doubt, costs incurred by Celgene in Developing a Baylor-Only Candidate through and including the Pivotal Study for the Baylor-Only Candidate. Upon execution of such Co-Development, Co-Promote and Profit Share Agreement, such Optioned Candidate will be an “Elected Candidate” thereunder. [\*\*\*]

**5.4 Non-Co-Promotion/Co-Development Option Exercise.** If during the [\*\*\*] following Celgene’s delivery of a Celgene Option Notice to Bluebird, Bluebird notifies Celgene in writing that Bluebird will not exercise the option set forth above in Section 5.3, or Bluebird does not deliver a Bluebird Option Notice to Celgene prior to the expiration of the [\*\*\*] period following Celgene’s delivery of a Celgene Option Notice to Bluebird, Celgene will pay to Bluebird the Additional Option Fee as set forth in Section 8.5, subject to Section 5.5.

**5.5 Baylor-Only Candidate Royalty & Milestone Payments.** In the event that any Optioned Candidate is also a Baylor-Only Candidate (as reasonably determined by the Parties), (i) the Initial Option Fee and the Additional Option Fee will each be reduced [\*\*\*], and (ii) any royalties or milestone payments payable under the applicable Development & Commercialization Agreement with respect to such Optioned Candidate will be reduced [\*\*\*]. All such payments will become due and payable only upon the commencement of a Pivotal Study (as defined in the applicable Development & Commercialization Agreement) for such Optioned Candidate. At such time that the Optioned Candidate no longer satisfies all of the requirements of the definition of Baylor-Only Candidate as set forth below in this Section 5.5, all



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future milestone and royalty payments thereunder will be payable in the original amounts thereunder [\*\*\*]. For clarity, such [\*\*\*] reduction will only apply to royalties and milestone payments and no other payments under the applicable Development & Commercialization Agreement (and for clarity, in the Co-Development, Co-Promote and Profit Share Agreement attached hereto as Exhibit B, the profit share/loss will be unaffected). [\*\*\*]

5.6 Follow-On Product Candidates. The Parties acknowledge and agree that the Collaboration Program may include the Development and evaluation of multiple Product Candidates targeting the same Target Antigen. If Celgene exercises its option pursuant to Section 5.1 to take a license to a Product Candidate that targets the same Target Antigen as an existing Optioned Candidate (a “Follow-On Product Candidate”), then the Parties will enter into a new License Agreement in the form attached hereto as Exhibit A with respect to such Follow-On Product Candidate, unless (i) Bluebird has exercised or does exercise its option to co-promote and co-Develop such existing Optioned Candidate in the U.S. as set forth in Section 5.3, and (ii) this Agreement has not been terminated by Celgene pursuant to Section 12.3(a), in which case the Parties will enter into a Co-Development, Co-Promote and Profit Share Agreement with respect to such Follow-On Product Candidate, provided that no [\*\*\*] will be payable under any such Development & Commercialization Agreement. If the Parties enter into Co-Development, Co-Promote and Profit Share Agreement with respect to such Follow-On Product Candidate, Bluebird will have the option of paying [\*\*\*] Bluebird’s allowable expenses thereunder by [\*\*\*]. If the Parties enter into a License Agreement pursuant to this Section 5.6 with respect to a Follow-On Product Candidate, Celgene will pay to Bluebird (a) the Initial Option Fee in accordance with Section 8.4, and (b) the Additional Option Fee in accordance with Section 8.5 (but excluding subsections (i) and (ii) of such Section 8.5). If the Parties enter into a Co-Development, Co-Promote and Profit Share Agreement pursuant to this Section 5.6 with respect to a Follow-On Product Candidate, Celgene will pay to Bluebird the Initial Option Fee in accordance with Section 8.4 but no Additional Option Fee.

5.7 Declined Product Candidates.

(a) *Bluebird Development*. If Celgene does not exercise its option with respect to a Product Candidate as set forth in Section 5.1, such Product Candidate or IND Product Candidate, as applicable, will become a “Declined Product Candidate” hereunder. Bluebird will have the option, exercisable upon written notice to Celgene (a “Bluebird Development Notice”), to Develop such Declined Product Candidate outside of the scope of the Collaboration Program, and Celgene hereby grants to Bluebird an exclusive, worldwide, royalty-free right and license, with the right to grant sublicenses, under the Celgene IP and Celgene’s interest in jointly owned Collaboration IP, solely to Develop such Declined Product Candidate. [\*\*\*] In connection with any such Development activities, Bluebird will (i) maintain, or cause to be maintained, records of its activities with respect to the Development of such Declined Product Candidate in sufficient detail and in good scientific manner appropriate for scientific, Patent and regulatory purposes, for a period of at least ten (10) years after the creation of such records, but in no event less than required by applicable Laws, and Celgene will have the right to request and receive a copy of any such records, and (ii) furnish Celgene with a copy of any safety and efficacy data generated by Bluebird or its Affiliates in connection with a clinical trial performed with respect to such Declined Product Candidate, and all correspondence to and from any Regulatory Authority regarding such Declined Product Candidate, at least thirty (30) days prior to initiating a Declined Product Candidate Study for such Declined Product Candidate.

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(b) *Celgene Buy-In Rights*.

(i) At least thirty (30) days prior to initiating a Declined Product Candidate Study for a Declined Product Candidate, and no later than thirty (30) days after completing a Declined Product Candidate Study for a Declined Product Candidate, Bluebird will provide Celgene with written notice of same. Celgene will have the option, exercisable on a Declined Product Candidate-by-Declined Product Candidate basis at any time prior to [\*\*\*] completion of a Declined Product Candidate Study for such Declined Product Candidate, to designate such Declined Product Candidate as an Optioned Candidate. [\*\*\*] prior to the initiation of a Declined Product Candidate Study for a Declined Product Candidate, Bluebird will provide to Celgene (1) all safety and efficacy data in Bluebird’s possession as of the date of such disclosure generated with respect to such Declined Product Candidate in all clinical studies conducted prior to the Declined Product Candidate Study for such Declined Product Candidate, and (2) all correspondence to and from any Regulatory Authority regarding such Declined Product Candidate as of the date of such disclosure. Following such disclosure, Bluebird will provide Celgene with (x) any additional data and correspondence described in subsections (1) and (2) that comes into Bluebird’s possession during such [\*\*\*] period, and (y) any other information relating to such Declined Product Candidate reasonably requested by Celgene. Within [\*\*\*] after the completion of a Declined Product Candidate Study for a Declined Product Candidate, Bluebird will provide Celgene with all safety and efficacy data generated with respect to such Declined Product Candidate in such Declined Product Candidate Study, all correspondence to and from any Regulatory Authority regarding such Declined Product Candidate, and any other information relating to such Declined Product Candidate reasonably requested by Celgene.

(ii) Such option is exercisable by Celgene by providing Bluebird with a Celgene Option Notice, and if Celgene so elects, (1) such Declined Product Candidate will be an Optioned Candidate for all purposes hereunder (other than Section 5.1), and (2) Celgene will pay to Bluebird (in lieu of any Option Fees) an amount equal to:

(A) if the option is exercised by Celgene prior to the initiation of the Declined Product Candidate Study, the greater of [\*\*\*]; or

(B) if the option is exercised by Celgene after the initiation of the Declined Product Candidate Study, the greater of [\*\*\*].

(iii) If Celgene does not exercise its option with respect to a Declined Product Candidate as set forth above, (A) the Development license granted by Celgene to Bluebird under Section 5.7(a) will also include the rights to Manufacture and commercialize such Declined Product Candidate, provided that such license shall be limited to the Celgene IP and jointly owned Collaboration IP as it exists at the time Celgene’s option to the Declined Product Candidate has expired or been terminated (including in each case any additions, divisions, continuations, continuations-in-part, invention certificates, substitutions, reissues, reexaminations, extensions, registrations, supplementary protection certificates and renewals of such Celgene IP and Joint Collaboration IP), (B) such Declined Product Candidate will continue to be excluded from the scope of the Collaboration Program, (C) Bluebird will reimburse

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Celgene within ten (10) days of the expiration of Celgene’s option for the Declined Product Candidate for any royalty, milestone or other payments made by Celgene under the Applicable Celgene In-License (other than any upfront payment) in respect of such Declined Product Candidate; (D) if any royalty, milestone or other payment becomes due under any Applicable Celgene In-License that is attributable to Bluebird as a sublicensee (together with its licensees and their respective Affiliates) thereunder with respect to such Development, Manufacture or commercialization of the Declined Product Candidate, Celgene will pay same, provided that Bluebird will reimburse Celgene for any such payment within thirty (30) days of Bluebird’s receipt of Celgene’s written invoice therefor, and Bluebird’s failure to pay such reimbursement within such time period will entitle Celgene to terminate Bluebird’s sublicense under the Applicable Celgene In-License upon thirty (30) days written notice; and (E) subject to the exclusivity restrictions set forth in Section 2.1(e), Section 3.5 of the License Agreement (if applicable) or Section 10.4 of the Co-Development, Co-Promote and Profit Share Agreement (if applicable), Bluebird will be free to research, Develop, Manufacture and commercialize such Declined Product Candidate alone or with others with no further obligation to Celgene other than with respect to any payment that may become due under any Applicable Celgene In-License that is attributable to Bluebird as a sublicensee (together with its licensees and their respective Affiliates) thereunder with respect to such Development, Manufacture and commercialization.

5.8 Bluebird In-Licenses. Any Pre-Existing In-Licenses that are necessary or useful for a Product Candidate under a Development & Commercialization Agreement will automatically be included within the definition of Applicable Pre-Existing In-Licenses in such Development & Commercialization Agreement, and any Bluebird Collaboration In-Licenses that Celgene elects to include within the definition of Applicable New In-Licenses in such Development & Commercialization Agreement will be so included. Any Bluebird Collaboration In-Licenses that Celgene does not elect to include in such Development & Commercialization Agreement will be an Other In-License with respect to such Development & Commercialization Agreement unless and until Celgene elects to convert such Other In-License to an Applicable New In-License in accordance with the terms of the applicable Development & Commercialization Agreement. Promptly following Celgene’s delivery of a Celgene Option Notice with respect to a Product Candidate, the Parties will mutually update the applicable Appendices to the Development & Commercialization Agreement. If the Parties cannot agree on such update, Celgene will have the right to make the final decision with respect to such update. For clarity, if, (1) during the Collaboration Program Term or (2) at the time Celgene takes rights to an IND Candidate or Pre-IND Candidate under Section 12.4, Celgene elects to convert a Bluebird New In-License into a Bluebird Collaboration In-License pursuant to Section 4.1(d), such Collaboration In-License will be an “Other In-License” with respect to any Development & Commercialization Agreement in effect or to be entered into under Section 12.4 at the time of such election, and Celgene may elect to convert such Other In-License to an Applicable New In-License in accordance with the terms of such applicable Development & Commercialization Agreement.

5.9 Government Approvals.

(a) Each of Celgene and Bluebird shall use its commercially reasonable good faith efforts to eliminate any concern on the part of any court or government authority regarding the

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legality of any proposed Development & Commercialization Agreement (including with respect to IND Candidates and Pre-IND Candidates under Section 12.4), including, if required by federal or state antitrust authorities, promptly taking all steps to secure government antitrust clearance, including cooperating in good faith with any government investigation including the prompt production of documents and information demanded by a second request for documents and of witnesses if requested. Notwithstanding anything to the contrary in this Agreement, this Section 5.9 and the term “commercially reasonable good faith efforts” do not require that either Party (i) offer, negotiate, commit to or effect, by consent decree, hold separate order, trust or otherwise, the sale, divestiture, license or other disposition of any capital stock, assets, rights, products or businesses of Celgene, Bluebird or their respective Affiliates, (ii) agree to any restrictions on the businesses of Celgene, Bluebird or their respective Affiliates, or (iii) pay any material amount or take any other action to prevent, effect the dissolution of, vacate, or lift any decree, order, judgment, injunction, temporary restraining order, or other order in any suit or proceeding that would otherwise have the effect of preventing or delaying the transactions contemplated by any proposed Development and Commercialization Agreement.

(b) Each of Celgene and Bluebird shall, within ten (10) business days after the execution of a Development & Commercialization Agreement (or such later time as may be agreed to in writing by the Parties) file with the United States of America Federal Trade Commission (“FTC”) and the Antitrust Division of the United States of America Department of Justice (“DOJ”) any HSR Filing required of it under the HSR Act in the reasonable opinion of either Party with respect to the transactions contemplated by such Development and Commercialization Agreement. The Parties shall cooperate with one another to the extent necessary in the preparation of any such HSR Filing. [\*\*\*] In the event that the Parties make an HSR Filing under this Section 5.9, the relevant Development & Commercialization Agreement shall terminate (i) at the election of either Party, immediately upon notice to the other Party, in the event that the United States of America Federal Trade Commission or the United States of America Department of Justice obtains a preliminary injunction under the HSR Act against the Parties to enjoin the transactions contemplated by such Development & Commercialization Agreement or (ii) at the election of either Party, immediately upon notice to the other Party, in the event that the HSR Clearance Date shall not have occurred on or prior to one hundred eighty (180) days after the effective date of the HSR Filing. Notwithstanding anything to the contrary contained herein, except for the terms and conditions of this Section 5.9, none of the terms and conditions contained in a Development and Commercialization Agreement shall be effective until the “Implementation Date,” which is agreed and understood to mean the later of (1) the execution date of the Development & Commercialization Agreement, (2) if a determination is made pursuant to this Section 5.9 that a notification of this Agreement is not required to be made under the HSR Act, the date of such determination, or (3) if notification of this Agreement is required to be made under the HSR Act, the HSR Clearance Date. As used herein: (x) “HSR Act” means the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, and the rules and regulations promulgated thereunder; (y) “HSR Clearance Date” means the earliest date on which the Parties have actual knowledge that all applicable waiting periods under the HSR Act with respect to the transactions contemplated by a Development & Commercialization Agreement have expired or have been terminated; and (z) “HSR Filing” means a filing by Celgene and Bluebird with the United States of America Federal Trade Commission and the

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Antitrust Division of the United States of America Department of Justice of a Notification and Report Form for Certain Mergers and Acquisitions (as that term is defined in the HSR Act) with respect to the matters set forth in this Agreement, together with all required documentary attachments thereto.

(c) Each of Celgene and Bluebird shall, in connection with any HSR Filing, (i) reasonably cooperate with each other in connection with any communication, filing or submission and in connection with any investigation or other inquiry, including any proceeding initiated by a private party; (ii) keep the other Party and/or its counsel informed of any communication received by such Party from, or given by such Party to, the FTC, the DOJ or any other U.S. or other governmental authority and of any communication received or given in connection with any proceeding by a private party, in each case regarding the transactions contemplated by any proposed Development & Commercialization Agreement; (iii) consult with each other in advance of any meeting or conference with the FTC, the DOJ or any other governmental authority or, in connection with any proceeding by a private party, with any other person, and to the extent permitted by the FTC, the DOJ or such other governmental authority or other person, give the other Parties and/or their counsel the opportunity to attend and participate in such meetings and conferences; and (iv) permit the other Parties and/or their counsel to review in advance any submission, filing or communication (and documents submitted therewith) intended to be given by it to the FTC, the DOJ or any other governmental authority; provided, that materials may be redacted to remove references concerning the valuation of the business of Bluebird. Bluebird and Celgene, as each deems advisable and necessary, may reasonably designate any competitively sensitive material to be provided to the other under this Section 5.9(c) as “Antitrust Counsel Only Material.” Such materials and the information contained therein shall be given only to the outside antitrust counsel of the recipient and will not be disclosed by such outside counsel to employees, officers or directors of the recipient unless express permission is obtained in advance from the source of the materials (Celgene or Bluebird, as the case may be) or its legal counsel.

(d) Celgene and Bluebird shall cooperate and use respectively all reasonable efforts to make all other registrations, filings and applications, to give all notices and to obtain as soon as practicable all governmental or other consents, transfers, approvals, orders, qualifications authorizations, permits and waivers, if any, and to do all other things necessary or desirable for the consummation of the transactions as contemplated hereby. Neither Party shall be required, however, to divest or out-license products or assets or materially change its business if doing so is a condition of obtaining approval of the transactions contemplated by this Agreement.

(e) If a Development & Commercialization Agreement is terminated pursuant to this Section 5.9, then, notwithstanding any provision in this Agreement to the contrary, neither Party shall have any further obligation to the other Party with respect to the subject matter of such Development & Commercialization Agreement.

5.10 Section 365(n) of the Bankruptcy Code. All rights and licenses granted pursuant to any section of this Agreement are, and will be deemed to be, rights and licenses to “intellectual property” (as defined in Section 101(35A) of title 11 of the United States Code and of any similar provisions of applicable Laws under any other jurisdiction (the “Bankruptcy Code”). Each Party agrees that the other Party, as a licensee of rights and licenses under this Agreement,

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will retain and may fully exercise all of its rights and elections under the Bankruptcy Code. The Parties further agree that, in the event of the commencement of a bankruptcy proceeding by or against a Party under the Bankruptcy Code or analogous provisions of applicable Law outside the United States, the other Party will be entitled to a complete duplicate of (or complete access to, as appropriate) any intellectual property licensed to such Party and all embodiments of such intellectual property, which, if not already in such Party’s possession, will be promptly delivered to it (a) upon any such commencement of a bankruptcy proceeding upon such Party’s written request therefor, unless the Party in the bankruptcy proceeding elects to continue to perform all of its obligations under this Agreement or (b) if not delivered under clause (a), following the rejection of this Agreement by the Party in the bankruptcy proceeding upon written request therefor by the other Party.

**6. [\*\*\*]**

6.8 Definitions. For purposes of this Section 6,

(a) A “Corporate Event” means a Business Combination involving Bluebird; it being understood and agreed that, solely for purposes of this Section 6, references to “Third Party” and “Third Parties” in the definition of “Business Combination” shall be deemed to include Celgene and, therefore, a “Corporate Event” may involve Bluebird and Celgene.

(b) [\*\*\*]

6.9 Call Option. Bluebird grants to Celgene the rights set forth in Exhibit L (the “Call Option”).

**7. [\*\*\*]**

**8. Payments**

8.1 Up-Front Payment. Celgene will pay to Bluebird, within ten (10) days after the Effective Date, a one-time payment of [\*\*\*] in consideration for the research and Development work to be performed by or on behalf of Bluebird as a part of the Collaboration Program, which will be non-refundable and non-creditable and not subject to set-off.

8.2 First Collaboration Extension Term Fee. In consideration for the research and Development work to be performed by or on behalf of Bluebird as a part of the Collaboration Program, Celgene will pay to Bluebird a one-time payment of [\*\*\*] within [\*\*\*] after the date of the delivery to Bluebird of written notice electing to extend the Collaboration Program for the First Collaboration Program Extension Term in accordance with Section 2.1(d), which will, except as otherwise set forth in Sections 4.3 and 12.6 hereof, Section 10.6 of the License Agreement (if applicable) or Section 17.6 of the Co-Development, Co-Promote and Profit Share Agreement (if applicable), be non-refundable and non-creditable and not subject to set-off.

8.3 Second Collaboration Extension Term Fee. In consideration for the research and Development work to be performed by or on behalf of Bluebird as a part of the Collaboration Program, Celgene will pay to Bluebird a one-time payment of [\*\*\*] within [\*\*\*] after the date of the delivery to Bluebird of written notice electing to extend the Collaboration Program for the Second Collaboration Program Extension Term in accordance with Section 2.1(d), which will, except as otherwise set forth in Sections 4.3 and 12.6 hereof, Section 10.6 of the License Agreement (if applicable) or Section 17.6 of the Co-Development, Co-Promote and Profit Share Agreement (if applicable), be non-refundable and non-creditable and not subject to set-off.

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8.4 Initial Option Fee. Subject to Sections 5.5 and 5.6, Celgene will pay to Bluebird [\*\*\*] (an “Initial Option Fee”) within [\*\*\*] after the Implementation Date for each Development & Commercialization Agreement, which Initial Option Fee will, except as otherwise set forth in Sections 4.1(e), 4.3 and 12.6 hereof, Section 10.6 of the License Agreement (if applicable) or Section 17.6 of the Co-Development, Co-Promote and Profit Share Agreement (if applicable), be non-refundable and non-creditable and not subject to set-off.

8.5 Additional Option Fee. Subject to Sections 5.5 and 5.6, Celgene will pay to Bluebird [\*\*\*] (the “Additional Option Fee”) within [\*\*\*] after the later to occur of (i) Bluebird’s written notice to Celgene that Bluebird will not exercise the option set forth above in Section 5.2, (ii) Bluebird does not deliver a Bluebird Option Notice to Celgene prior to the expiration of the [\*\*\*] period following Celgene’s delivery of a Celgene Option Notice to Bluebird, and (iii) the Implementation Date, which Additional Option Fee will, except as otherwise set forth in Sections 4.1(e), 4.3 and 12.6 hereof, Section 10.6 of the License Agreement (if applicable) or Section 17.6 of the Co-Development, Co-Promote and Profit Share Agreement (if applicable), be non-refundable and non-creditable and not subject to set-off.

8.6 In-Licenses; New Celgene In-Licenses.

(a) *Pre-Existing In-Licenses*. If any payments become due during the Term under any Pre-Existing In-License, Bluebird will be solely responsible for such payments, other than as expressly provided in Section 9.2 and, provided such payment obligation is not specifically attributable to any executed Development & Commercialization Agreement, which will be addressed thereunder. [\*\*\*] Bluebird will not use any Patents, Know-How or Materials in-licensed pursuant to a Pre-Existing In-License in the Collaboration Program if Bluebird does not have the right under such Pre-Existing In-License to use such Patents, Know-How or Materials in the Field.

(b) *Bluebird Collaboration In-Licenses*. If any payments become due during the Term under any Bluebird Collaboration In-License, Bluebird will be solely responsible for such payments, other than as expressly provided in Section 9.2, provided that [\*\*\*]

(c) *Celgene In-Licenses*. Except as otherwise provided in Sections 2.1(h)(ii), 5.7 and 12.4, Payments that become due under any Applicable Celgene In-License will be paid as set forth in Section 4.1(e), and any royalties payable under such Applicable Product In-License will be paid by Celgene and will be subject to Section 4.3(d) of any License Agreement or Section 11.3(d) of any Co-Development, Co-Promote and Profit Share Agreement, as applicable.

8.7 Taxes. [\*\*\*]

9. Patent Prosecution and Maintenance.

9.1 Generally. Subject to Sections 9.2 and 9.3, Bluebird will have the sole right to Prosecute and Maintain Patents within the Bluebird IP, Celgene will have the sole right to Prosecute and Maintain Patents with the Celgene IP, and the Parties will jointly control the Prosecution and Maintenance of any Patents within jointly-owned Collaboration IP.

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9.2 Celgene Input: Expenses. Bluebird will regularly provide Celgene with copies of all applications for Patents within the Bluebird IP, and all other material submissions and correspondence with any patent authorities regarding such Patents, in sufficient time to allow for review and comment by Celgene. In addition, Bluebird will provide Celgene and its counsel with an opportunity to consult with Bluebird and its counsel regarding Prosecution and Maintenance of any such Patents in the Field, and Bluebird will consider in good faith all comments timely made by Celgene and its counsel. In the event of any disagreement between any of Bluebird or Celgene, Bluebird will have the final decision-making authority with respect to the matter involved as long as Bluebird acts in good faith, provided that if Celgene requests that Bluebird Prosecute and Maintain Patents in a particular jurisdiction, Bluebird will comply with such request, and provided further that Bluebird will not abandon Prosecution and Maintenance of any Patents within the Bluebird IP without Celgene’s prior written consent (such consent not to be unreasonably withheld, delayed or conditioned). In addition, for each Product Candidate, the Parties shall cooperate to develop a mutually acceptable patent strategy designed to obtain Patents that include only claims Covering the Product Candidate, pharmaceutical compositions comprising the Product Candidate, or their manufacture or use, and no other product (or its manufacture or use), and Bluebird shall, to the extent permitted under applicable Law, use its reasonable best efforts to implement such strategy. [\*\*\*]

9.3 Bluebird Input: Expenses. Celgene will regularly provide Bluebird with copies of all applications for Patents (i) within Collaboration IP solely owned by Celgene pursuant to Section 2.1(f) and (ii) within the Celgene IP that are in-licensed by Celgene pursuant to an Applicable Celgene New In-License (other than those sublicensed to Bluebird on a non-exclusive basis), and all other material submissions and correspondence with any patent authorities regarding such Patents, in sufficient time to allow for review and comment by Bluebird. In addition, Celgene will provide Bluebird and its counsel with an opportunity to consult with Celgene and its counsel regarding Prosecution and Maintenance of any such Patents in the Field, and Celgene will consider in good faith all comments timely made by Bluebird and its counsel. In the event of any disagreement between any of Bluebird or Celgene, Celgene will have the final decision-making authority with respect to the matter involved as long as Celgene acts in good faith. During the Term, Celgene will be solely responsible for all Patent Costs incurred in connection with the Prosecution and Maintenance of Patents within the Celgene IP.

9.4 Jointly Owned Collaboration IP. The Prosecution and Maintenance and the enforcement and defense of any Patents within jointly-owned Collaboration IP will be jointly managed by the Parties on mutually agreeable terms to be entered into by the Parties at the time any such Patents are first filed, provided that (i) absent further agreement, the enforcement and defense of any Patents within jointly-owned Collaboration IP will be governed by, and all recoveries and Patent Costs arising from the enforcement or defense of any Patents within jointly-owned Collaboration IP will be retained or borne, as applicable, in accordance with the principles set forth in Section 2.1(f)(iii) (i.e., U.S. patent law for joint ownership of Patents will apply), and (ii) Patent Costs incurred in connection with the Prosecution and Maintenance of Patents within jointly-owned Collaboration IP will be apportioned as set forth in Section 9.2, for the purposes of which, such Patents will be treated as Patents within the Bluebird IP, provided that in each case ((i) and (ii)), if either Party elects not to pay any such Patent Costs for any such Patent, the Parties will meet and agree upon an equitable way to treat such Patent.



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9.5 Third Party Rights.

(a) To the extent that a Third Party licensor of Bluebird has retained any right to Prosecute or Maintain any Patent within the Bluebird IP licensed to Bluebird pursuant to a Bluebird In-License, or otherwise be involved in such activities, Bluebird will use commercially reasonable efforts to cause such Third Party licensor to take the actions specified by Section 9. in a manner consistent with the Bluebird In-Licenses applicable thereto, but Bluebird will not be deemed to be in breach of its obligations under Section 9 if, after using such commercially reasonable efforts, it is unable to comply with such obligations because of actions taken or not taken by such Third Party licensor.

(b) To the extent that a Third Party licensor of Celgene has retained any right to Prosecute or Maintain any Patent within the Celgene In-Licensed IP licensed to Celgene pursuant to an Applicable Celgene In-License, or otherwise be involved in such activities, Celgene will use commercially reasonable efforts to cause such Third Party licensor to take the actions specified by Section 9 in a manner consistent with the Applicable Celgene In-Licenses applicable thereto, but Celgene will not be deemed to be in breach of its obligations under Section 9 if, after using such commercially reasonable efforts, it is unable to comply with such obligations because of actions taken or not taken by such Third Party licensor.

9.6 Common Interest Disclosures. With regard to any information or opinions disclosed pursuant to this Agreement by one Party to the other Party regarding Prosecution and Maintenance of Patent within the Bluebird IP, Celgene IP or Collaboration IP or enforcement or defense of intellectual property and/or technology by or against Third Parties, Bluebird and Celgene agree that they have a common legal interest in determining the ownership, scope, validity and/or enforcement of the Bluebird IP, Celgene IP or Collaboration IP, and whether, and to what extent, Third Party intellectual property rights may affect the conduct of the Development and commercialization of any Product Candidate, and have a further common legal interest in defending against any actual or prospective Third Party claims based on allegations of misuse or infringement of intellectual property rights relating to the Development or commercialization of any Product Candidate. Accordingly, the Parties agree that all such information and materials obtained by the Parties from each other will be used solely for purposes of the Parties’ common legal interests with respect to the conduct of the Agreement and otherwise for each Party to exercise its rights and perform its obligations hereunder. All such information and materials will be treated as protected by the attorney-client privilege, the work product privilege, and any other privilege or immunity that may otherwise be applicable. By sharing any such information and materials, neither Party intends to waive or limit any privilege or immunity that may apply to the shared information and materials. Neither Party will have the authority to waive any privilege or immunity on behalf of the other Party without such other Party’s prior written consent, nor will the waiver of privilege or immunity resulting from the conduct of one Party be deemed to apply against any other Party. This Section 9.6 will be subject to any right granted by Bluebird to any Third Party or by Celgene to any Third Party, provided that the grant of such right to such Third Party does not conflict with the other Party’s rights or a Party’s obligations under this Agreement.

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**10. Confidentiality.**

10.1 Confidential Information.

(a) *Confidential Information.* Each Party (“Disclosing Party”) may have disclosed or will disclose to the other Party (“Receiving Party”), and Receiving Party may acquire during the course and conduct of activities under this Agreement or any executed Development & Commercialization Agreement, certain proprietary or confidential information of Disclosing Party. The term “Confidential Information” means (i) all Materials and (ii) all ideas and information of any kind, whether in written, oral, graphical, machine-readable or other form, whether or not marked as confidential or proprietary, which are transferred, disclosed or made available by Disclosing Party or at the request of Receiving Party, including any of the foregoing of Third Parties. Without limiting the foregoing, Collaboration IP solely owned by Bluebird will be considered Confidential Information of Bluebird, Collaboration IP solely owned by Celgene will be considered Confidential Information of Celgene, and Collaboration IP jointly owned by the Parties will be considered Confidential Information of both Parties.

(b) *Restrictions.* During the Term and for ten (10) years thereafter, Receiving Party will keep all Disclosing Party’s Confidential Information in confidence with the same degree of care with which Receiving Party holds its own confidential information, provided that the foregoing obligation will apply to any Confidential Information that constitutes a trade secret for so long as such Confidential Information is afforded trade secret protection under applicable Law. Receiving Party will not use Disclosing Party’s Confidential Information except for in connection with the performance of its obligations and exercise of its rights under this Agreement or any executed Development & Commercialization Agreement. Receiving Party has the right to disclose Disclosing Party’s Confidential Information without Disclosing Party’s prior written consent (such consent not to be unreasonably withheld, delayed or conditioned), to the extent and only to the extent reasonably necessary, to Receiving Party’s Affiliates and their employees, subcontractors, sublicensees, consultants or agents who have a need to know such Confidential Information in order to perform its obligations and exercise its rights under this Agreement or any executed Development & Commercialization Agreement and who are required to comply with restrictions on use and disclosure similarly restrictive as those in this Section 10.1(b). Receiving Party will use diligent efforts to cause those entities and persons to comply with such restrictions on use and disclosure. Receiving Party assumes responsibility for those entities and persons maintaining Disclosing Party’s Confidential Information in confidence and using same only for the purposes described herein.

(c) *Exceptions.* Receiving Party’s obligation of nondisclosure and the limitations upon the right to use the Disclosing Party’s Confidential Information will not apply to the extent that Receiving Party can demonstrate that the Disclosing Party’s Confidential Information: (i) was known to Receiving Party or any of its Affiliates prior to the time of disclosure; (ii) is or becomes public knowledge through no fault or omission of Receiving Party or any of its Affiliates; (iii) is obtained by Receiving Party or any of its Affiliates from a Third Party under no obligation of confidentiality to Disclosing Party; or (iv) has been independently developed by employees, subcontractors, consultants or agents of Receiving Party or any of its Affiliates without the aid, application or use of Disclosing Party’s Confidential Information, as evidenced by contemporaneous written records.

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(d) *Permitted Disclosures*. Receiving Party may disclose Disclosing Party’s Confidential Information to the extent (and only to the extent) such disclosure is reasonably necessary in the following instances:

(i) in order to comply with applicable Law (including any securities law or regulation or the rules of a securities exchange) or with a legal or administrative proceeding;

(ii) in connection with prosecuting or defending litigation, Regulatory Approvals and other regulatory filings and communications, and filing, prosecuting and enforcing Patents in connection with Receiving Party’s rights and obligations pursuant to this Agreement or any executed Development & Commercialization Agreement; and

(iii) in connection with performing its obligations or exercising its rights hereunder or any executed Development & Commercialization Agreement, to its Affiliates; and subject to Section 10.3(a), to potential and future collaborators, licensees, sublicensees and permitted acquirers or assignees, and investment bankers, investors and lenders;

provided that (1) with respect to Sections 10.1(d)(i) or 10.1(d)(ii), where reasonably possible, Receiving Party will notify Disclosing Party of Receiving Party’s intent to make any disclosure pursuant thereto sufficiently prior to making such disclosure so as to allow Disclosing Party adequate time to take whatever action it may deem appropriate to protect the confidentiality of the information to be disclosed, and (2) with respect to Section 10.1(d)(iii), each of those named people and entities are required to comply with restrictions on use and disclosure at least as restrictive as those in Section 10.1(b) (other than investment bankers, investors and lenders, which must be bound prior to disclosure by commercially reasonable obligations of confidentiality).

**10.2 Publications.** The Parties may desire to publish in scientific journals and present at scientific conferences the results of the Collaboration Program, subject to the following process. Notwithstanding anything to the contrary herein, either Party may propose publication of the results of the Collaboration Program following scientific review by the JSC (if in force) and subsequent written approval by Bluebird’s and Celgene’s management, which approval will not be unreasonably withheld, delayed or conditioned. After receipt of the proposed publication by both Celgene’s and Bluebird’s managements, such written approval or disapproval will be provided within thirty (30) days. Both Parties understand that a reasonable commercial strategy may require delay of publication of information or filing of Patent applications, therefore the Parties agree to review and consider delay of publication and filing of Patent applications under certain circumstances for a reasonably limited period of time. Once publications have been reviewed by each Party and have been approved for publication, the same publications do not have to be provided again to the other Party for review for a later submission for publication. Expedited reviews for abstracts or poster presentations may be arranged if mutually agreeable to the Parties. Each Party will acknowledge the other Party’s technical, non-financial contributions in any such publication. For the avoidance of doubt, the foregoing requirements and restrictions will not apply with respect to either Party’s proposed publication of results of any work performed (i) following the expiration or termination of the Collaboration Program, or (ii) with respect to any Declined Product Candidate, in each case except as such results specifically relate to any Optioned Candidate or to any Product Candidate for which Celgene has an option

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hereunder (unless such option expires without Celgene having exercised such option), in which case Bluebird may not publish or present such results without Celgene’s prior written approval, which will not be unreasonably withheld, delayed or conditioned.

10.3 Terms of this Agreement: Publicity.

(a) *Restrictions.* The Parties agree that the terms of this Agreement (including, for clarity, for this Section 10.3(a), the Exhibits hereto) and any executed Development & Commercialization Agreement will be treated as Confidential Information of both Parties, and thus may be disclosed only as permitted by Section 10.1(d). Each Party shall also be permitted to disclose the terms of this Agreement, in each case under appropriate confidentiality provisions at least as protective as those contained in this Agreement, on a need to know basis, to a bona fide potential or future permitted acquirer or assignee, investment banker, investor, licensee, sublicensee, collaborator or lender with whom a Party has entered into good faith negotiations regarding a proposed transaction, provided that (i) such disclosure is solely in the form of the redacted version of (A) this Agreement attached hereto as Exhibit H or (B) the redacted version of such executed Development & Commercialization Agreement attached as an Appendix thereto and (ii) a corresponding summary of financial terms for each such agreement also attached as an Exhibit or Appendix (as applicable) thereto. Only after negotiations with any such Third Party have progressed so that such Party reasonably and in good faith believes it will execute a definitive agreement with such Third Party with respect to the proposed transaction within the following fifteen (15) business days may such Party provide an unredacted version of this Agreement and any executed Development & Commercialization Agreement to such Third Party. In addition to the foregoing, (1) Bluebird may provide an unredacted version of this Agreement and any executed Development & Commercialization Agreement to its investment bankers and other advisors, and (2) if Bluebird desires to enter into any such proposed transaction through an auction process, Bluebird may disclose the redacted form of this Agreement and any executed Development & Commercialization Agreement as part of that process, along with the financial summary, and may provide an unredacted version of this Agreement and any executed Development & Commercialization Agreement to those Third Parties that make a bona fide bid as part of such process, provided that Bluebird may not rely on this clause (2) until after the end of the Call Option Period. Except as required by Law, each Party agrees not to issue any press release or public statement disclosing information relating to this Agreement, any executed Development & Commercialization Agreement, the transactions contemplated hereby or thereby or any of the terms hereof or thereof without the prior written consent of the other Party (such consent not to be unreasonably withheld, delayed or conditioned), or as such consent may be obtained in accordance with Section 10.3(b), or as permitted by Section 10.3(d).

(b) *Review.* In the event either Party (the “Issuing Party”) desires to issue a press release or other public statement disclosing information relating to this Agreement, any executed Development & Commercialization Agreement, the transactions contemplated hereby or thereby or the terms hereof or thereof, the Issuing Party will provide the other Party (the “Reviewing Party”) with a copy of the proposed press release or public statement (the “Release”). The Issuing Party will specify with each such Release, taking into account the urgency of the matter being disclosed, a reasonable period of time within which the Reviewing Party may provide any

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comments on such Release and if the Reviewing Party fails to provide any comments during the response period called for by the Issuing Party, the Reviewing Party will be deemed to have consented to the issuance of such Release. If the Reviewing Party provides any comments, the Parties will consult on such Release and work in good faith to prepare a mutually acceptable Release. If the Reviewing Party does not provide its consent, not to be unreasonably withheld, conditioned or delayed, to the issuance of the Release, the Issuing Party will not issue the Release except as required by Law. Either Party may subsequently publicly disclose any information previously contained in any Release so consented to. Bluebird may issue a Release upon the payment by Celgene of a fee under Section 8.2 or 8.3 to extend the Collaboration Program Term, subject to Celgene’s prior review and approval (such approval not to be unreasonably withheld, delayed or conditioned).

(c) *Joint Press Release*. The Parties agree to issue the joint press release on Exhibit I.

(d) *Securities Filings*. Each Party acknowledges and agrees that the other Party may submit this Agreement (including, for clarity, the Exhibits hereto) and any executed Development & Commercialization Agreement to the United States Securities and Exchange Commission (the “SEC”) and if a Party does submit this Agreement or any executed Development & Commercialization Agreement to the SEC, such Party agrees to consult with the other Party with respect to the preparation and submission of, a confidential treatment request for this Agreement or such executed Development & Commercialization Agreement. If a Party is required by Law to make a disclosure of the terms of this Agreement or any executed Development & Commercialization Agreement in a filing with or other submission to the SEC, and (i) such Party has provided copies of the disclosure to the other Party as far in advance of such filing or other disclosure as is reasonably practicable under the circumstances, (ii) such Party has promptly notified the other Party in writing of such requirement and any respective timing constraints, and (iii) such Party has given the other Party a reasonable time under the circumstances from the date of notice by such Party of the required disclosure to comment upon, request confidential treatment or approve such disclosure, then such Party will have the right to make such public disclosure at the time and in the manner reasonably determined by its counsel to be required by Law. Notwithstanding anything to the contrary herein, it is hereby understood and agreed that if a Party seeking to make a disclosure to the SEC as set forth in this Section 10.3(d), and the other Party provides comments within the respective time periods or constraints specified herein or within the respective notice, the Party seeking to make such disclosure or its counsel, as the case may be, will in good faith (A) consider incorporating such comments and (B) use reasonable efforts to incorporate such comments, limit disclosure or obtain confidential treatment to the extent reasonably requested by the other Party.

10.4 Relationship to the Confidentiality Agreement. This Agreement supersedes that certain “Mutual Confidentiality Agreement” between the Parties dated May 21, 2012; provided that all “Confidential Information” disclosed or received by the Parties thereunder will be deemed “Confidential Information” hereunder and will be subject to the terms and conditions of this Agreement.

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**11. Warranties; Limitations of Liability; Indemnification.**

11.1 Representations and Warranties. Each Party represents and warrants to the other as of the Effective Date that it has the legal right and power to enter into this Agreement, to extend the rights granted or to be granted to the other in this Agreement, and to fully perform its obligations hereunder.

11.2 Additional Representations and Warranties of Bluebird. Bluebird represents and warrants to Celgene as of the Effective Date that:

(a) Except for the Pre-Existing In-Licenses, neither Bluebird nor any of its Affiliates is a party to any license, sublicense or other agreement pursuant to which Bluebird or such Affiliate has received a license or other rights relating to the Collaboration Program or the Field.

(b) The Pre-Existing In-Licenses in effect as of the Effective Date are valid and binding obligations of Bluebird and, to the Knowledge of Bluebird, the applicable licensor, enforceable against Bluebird and, to the Knowledge of Bluebird, the applicable licensor, in accordance with their terms, except as may be limited by general principles of equity (regardless of whether considered in a proceeding at law or in equity) and by applicable bankruptcy, insolvency, moratorium and other similar Laws of general application relating to or affecting creditors' rights generally. Neither Bluebird nor any of its Affiliates has received any notice of any counterparty's intention to terminate any Pre-Existing In-Licenses in whole or in part or any notice requesting any amendment, alteration or modification of such Pre-Existing In-License or any sublicense or assignment thereunder. There is no breach or default, or event which upon notice or the passage of time, or both, would give rise to any breach or default, in the performance of any Pre-Existing In-License by Bluebird or any of its Affiliates or, to the Knowledge of Bluebird, the counterparty thereto, and Bluebird has not received any notice of any such breach, default or event. All Patents and Know-How licensed to Bluebird under the Pre-Existing In-Licenses are Controlled by Bluebird for purposes of the licenses granted to Celgene under this Agreement and under any Development & Commercialization Agreement.

(c) Neither Bluebird nor any of its Affiliates has entered into any agreement or otherwise licensed, granted, assigned, transferred, conveyed or otherwise encumbered or disposed of any right, title or interest in or to any of its assets, including any intellectual property rights, that would in any way conflict with or impair the scope of any rights or licenses granted to Celgene hereunder (including the rights granted under Section 6 and under [\*\*\*]) or that would be granted to Celgene under any Development & Commercialization Agreement, including under any of the agreements which Bluebird has identified to Celgene prior to the Effective Date.

(d) Exhibit J sets forth a complete and accurate list of all Patents included in the Bluebird IP, indicating the owner, licensor and/or co-owner(s), if applicable. Bluebird Controls the Patents listed on Exhibit J and the Know-How within the Bluebird IP, and is entitled to grant the licenses specified herein. To Bluebird's Knowledge, the Patents listed on Exhibit J have been procured or are being procured from the respective patent offices in accordance with applicable Law. None of the Patents included in the Bluebird IP is or has been involved in any opposition, cancellation, interference, reissue or reexamination proceeding, and no Bluebird IP is the subject of any judicial, administrative or arbitral order, award, decree, injunction, lawsuit,

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proceeding or stipulation. Neither Bluebird nor any of its Affiliates has received any notice alleging that the Patents in the Bluebird IP are invalid or unenforceable, or challenging Bluebird’s ownership of or right to use any such rights.

(e) Exhibit K sets forth a complete and accurate list of all agreements relating to the licensing, sublicensing or other granting of rights by Bluebird to any Person with respect to the Bluebird IP and the Field, and Bluebird has provided complete and accurate copies of all such agreements to Celgene. Except for the Pre-Existing In-Licenses, Bluebird and its Affiliates are not subject to any payment obligations to Third Parties as a result of the execution or performance of this Agreement. Neither Bluebird nor any of its Affiliates has granted any liens or security interests on the Bluebird IP and the Bluebird IP is free and clear of any mortgage, pledge, claim, security interest, covenant, easement, encumbrance, lien or charge of any kind.

(f) The execution, delivery and performance by Bluebird of this Agreement and the consummation of the transactions contemplated hereby will not result in any violation of, conflict with, result in a breach of or constitute a default under any understanding, contract or agreement to which Bluebird is a party or by which it is bound, including each of the agreements which Bluebird has identified to Celgene prior to the Effective Date.

(g) There is no action, suit, proceeding or investigation pending or, to the Knowledge of Bluebird, currently threatened in writing against or affecting Bluebird that questions the validity of this Agreement or the right of Bluebird to enter into this Agreement or consummate the transactions contemplated hereby.

(h) Other than with respect to any Patents, Know-How or Materials licensed to Celgene pursuant to any of the Baylor Agreements, (i) neither Bluebird nor any of its Affiliates has received any notice of any claim that any Patent, Know-How or other intellectual property owned or controlled by a Third Party would be infringed or misappropriated by the production, use, research, Development, Manufacture or Commercialization of any Product Candidate pursuant to this Agreement and any Development & Commercialization Agreement, and (ii) to the Knowledge of Bluebird, there are no Patents, Know-How or other intellectual property owned by a Third Party and not included in the Bluebird IP that are necessary for the production, use, research, Development, Manufacture or commercialization of any Product Candidate.

11.3 Disclaimers. Without limiting the respective rights and obligations of the Parties expressly set forth herein, each Party specifically disclaims any guarantee that the Collaboration Program will be successful, in whole or in part. EXCEPT AS OTHERWISE EXPRESSLY PROVIDED IN THIS AGREEMENT, THE PARTIES MAKE NO REPRESENTATIONS AND EXTEND NO WARRANTY OF ANY KIND, EITHER EXPRESS OR IMPLIED, WITH RESPECT TO ANY BLUEBIRD IP, CELGENE IP, PRODUCT CANDIDATES, MATERIALS, INCLUDING WARRANTIES OF VALIDITY OR ENFORCEABILITY OF ANY PATENT RIGHTS, TITLE, QUALITY, MERCHANTABILITY, FITNESS FOR A PARTICULAR USE OR PURPOSE, PERFORMANCE, AND NONINFRINGEMENT OF ANY THIRD PARTY PATENTS OR OTHER INTELLECTUAL PROPERTY RIGHTS.

11.4 [\*\*\*]

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11.5 Performance by Others. The Parties recognize that each Party may perform some or all of its obligations under this Agreement through Affiliates and permitted subcontractors provided, however, that each Party will remain responsible and liable for the performance by its Affiliates and permitted subcontractors and will cause its Affiliates and permitted subcontractors to comply with the provisions of this Agreement in connection therewith.

11.6 Indemnification.

(a) *Indemnification by Celgene*. Celgene will indemnify Bluebird, its Affiliates and their respective directors, officers, employees, Third Party licensors and agents, and their respective successors, heirs and assigns (collectively, “Bluebird Indemnitees”), and defend and save each of them harmless, from and against any and all losses, damages, liabilities, costs and expenses (including reasonable attorneys’ fees and expenses) (collectively, “Losses”) in connection with any and all suits, investigations, claims or demands of Third Parties (collectively, “Third Party Claims”) against the Bluebird Indemnitees arising from or occurring as a result of: (i) the material breach by Celgene of any term of this Agreement; (ii) Celgene’s performance of the Collaboration Program (other than with respect to claims of actual or alleged infringement, misappropriation or other violation of a Third Party’s Patents, trade secrets, or other intellectual property or proprietary rights); or (iii) any gross negligence or willful misconduct on the part of Celgene in performing its obligations under this Agreement, except in each case for those Losses for which Bluebird has an obligation to indemnify Celgene pursuant to Section 11.6(b), as to which Losses each Party will indemnify the other to the extent of their respective liability; provided, however, that Celgene will not be obligated to indemnify Bluebird Indemnitees for any Losses to the extent that such Losses arise as a result of gross negligence or willful misconduct on the part of a Bluebird Indemnitee.

(b) *Indemnification by Bluebird*. Bluebird will indemnify Celgene, its Affiliates and their respective directors, officers, employees and agents, and their respective successors, heirs and assigns (collectively, “Celgene Indemnitees”), and defend and save each of them harmless, from and against any and all Losses in connection with any and all Third Party Claims against Celgene Indemnitees arising from or occurring as a result of: (i) the material breach by Bluebird of any term of this Agreement; (ii) Bluebird’s performance of the Collaboration Program (other than with respect to claims of actual or alleged infringement, misappropriation or other violation of a Third Party’s Patents, trade secrets, or other intellectual property or proprietary rights); (iii) [\*\*\*]; or (iv) any gross negligence or willful misconduct on the part of Bluebird in performing its obligations under this Agreement, except in each case for those Losses for which Celgene has an obligation to indemnify Bluebird pursuant to Section 11.6(a), as to which Losses each Party will indemnify the other to the extent of their respective liability for the Losses; provided, however, that Bluebird will not be obligated to indemnify Celgene Indemnitees for any Losses to the extent that such Losses arise as a result of gross negligence or willful misconduct on the part of a Celgene Indemnitee.

(c) *Notice of Claim*. All indemnification claims provided for in Section 11.6(a) and 11.6(b) will be made solely by such Party to this Agreement (the “Indemnified Party”). The Indemnified Party will promptly notify the indemnifying Party (an “Indemnification Claim Notice”) of any Losses or the discovery of any fact upon which the Indemnified Party intends to base a request for indemnification under Section 11.6(a) or 11.6(b), but in no event will the



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indemnifying Party be liable for any Losses that result from any delay in providing such notice. Each Indemnification Claim Notice must contain a description of the claim and the nature and estimated amount of such Loss (to the extent that the nature and amount of such Loss is known at such time). The Indemnified Party will furnish promptly to the indemnifying Party copies of all papers and official documents received in respect of any Losses and Third Party Claims.

(d) *Defense, Settlement, Cooperation and Expenses* .

(i) *Control of Defense*. At its option, the indemnifying Party may assume the defense of any Third Party Claim by giving written notice to the Indemnified Party within thirty (30) days after the indemnifying Party’s receipt of an Indemnification Claim Notice, provided however that (i) the Third Party Claim solely seeks monetary damages and (ii) the indemnifying Party expressly agrees in writing that as between the indemnifying Party and the Indemnified Party, the indemnifying Party will be solely obligated to satisfy and discharge the Third Party Claim in full and is able to reasonably demonstrate that it has sufficient financial resources (the matters described in (i) and (ii), the “Litigation Conditions”). The assumption of the defense of a Third Party Claim by the indemnifying Party will not be construed as an acknowledgment that the indemnifying Party is liable to indemnify the Indemnified Party in respect of the Third Party Claim (except as provided in the immediately prior sentence), nor will it constitute a waiver by the indemnifying Party of any defenses it may assert against the Indemnified Party’s claim for indemnification. Upon assuming the defense of a Third Party Claim, the indemnifying Party may appoint as lead counsel in the defense of the Third Party Claim any legal counsel selected by the indemnifying Party (the indemnifying Party will consult with the Indemnified Party with respect to a possible conflict of interest of such counsel retained by the indemnifying Party). In the event the indemnifying Party assumes the defense of a Third Party Claim, the Indemnified Party will immediately deliver to the indemnifying Party all original notices and documents (including court papers) received by the Indemnified Party in connection with the Third Party Claim. Should the indemnifying Party assume the defense of a Third Party Claim, except as provided in Section 11.6(d)(ii), the indemnifying Party will not be liable to the Indemnified Party for any legal costs or expenses subsequently incurred by such Indemnified Party in connection with the analysis, defense or settlement of the Third Party Claim. The Indemnified Party may, at any time, assume the defense of a Third Party Claim if at any time the Litigation Conditions are not satisfied with respect to such Claim. In the event that it is ultimately determined that the indemnifying Party is not obligated to indemnify, defend or hold harmless the Indemnified Party from and against the Third Party Claim, the Indemnified Party will reimburse the indemnifying Party for any and all costs and expenses (including attorneys’ fees and costs of suit) and any Third Party Claims incurred by the indemnifying Party in its defense of the Third Party Claim.

(ii) *Right to Participate in Defense*. Without limiting Section 11.6(d)(i), any Indemnified Party will be entitled to participate in, but not control, the defense of such Third Party Claim and to employ counsel of its choice for such purpose; provided, however, that such employment will be at the Indemnified Party’s own cost and expense unless (i) the employment thereof has been specifically authorized by the indemnifying Party in writing, (ii) the indemnifying Party has failed to assume the defense and employ counsel in accordance with Section 11.6(d)(i) (in which case the Indemnified Party will control the defense), (iii) the

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interests of the Indemnified Party and the indemnifying Party with respect to such Third Party Claim are sufficiently adverse to prohibit the representation by the same counsel of both Parties under applicable Law, ethical rules or equitable principles, or (iv) the indemnifying Party no longer satisfies the Litigation Conditions, in which case the indemnifying Party will assume one hundred percent (100%) of any such costs and expenses of counsel for the Indemnified Party.

(iii) *Settlement*. With respect to any Third Party Claims that relate solely to the payment of money damages in connection with a Third Party Claim and that will not result in the Indemnified Party’s becoming subject to injunctive or other relief or otherwise adversely affecting the business of the Indemnified Party in any manner, and as to which the indemnifying Party will have acknowledged in writing the obligation to indemnify the Indemnified Party hereunder, and subject to the Litigation Conditions being satisfied, the indemnifying Party will have the sole right to agree to the entry of any judgment, enter into any settlement or otherwise dispose of such Loss, on such terms as the indemnifying Party, in its sole discretion, will deem appropriate. With respect to all other Losses in connection with Third Party Claims, where the indemnifying Party has assumed the defense of the Third Party Claim in accordance with Section 11.6(d)(i), the indemnifying Party will have authority to agree to the entry of any judgment, enter into any settlement or otherwise dispose of such Loss provided it obtains the prior written consent of the Indemnified Party (such consent not to be unreasonably withheld, delayed or conditioned). The indemnifying Party will not be liable for any settlement or other disposition of a Loss by an Indemnified Party that is reached without the prior written consent of the indemnifying Party. Regardless of whether the indemnifying Party chooses to defend or prosecute any Third Party Claim, no Indemnified Party will admit any liability with respect to or settle, compromise or discharge, any Third Party Claim without the prior written consent of the indemnifying Party, such consent not to be unreasonably withheld, delayed or conditioned.

(iv) *Cooperation*. If the indemnifying Party chooses to defend or prosecute any Third Party Claim, the Indemnified Party will, and will cause each other Indemnified Party to, cooperate in the defense or prosecution thereof and will furnish such records, information and testimony, provide such witnesses and attend such conferences, discovery proceedings, hearings, trials and appeals as may be reasonably requested in connection therewith. Such cooperation will include access during normal business hours afforded to indemnifying Party to, and reasonable retention by the Indemnified Party of, records and information that are reasonably relevant to such Third Party Claim, and making Indemnified Parties and other employees and agents available on a mutually convenient basis to provide additional information and explanation of any material provided hereunder, and the indemnifying Party will reimburse the Indemnified Party for all its reasonable out-of-pocket costs and expenses in connection therewith.

(v) *Costs and Expenses*. Except as provided above in this Section 11.6(d), the costs and expenses, including attorneys’ fees and expenses, incurred by the Indemnified Party in connection with any claim will be reimbursed on a calendar quarter basis by the indemnifying Party, without prejudice to the indemnifying Party’s right to contest the Indemnified Party’s right to indemnification and subject to refund in the event the indemnifying Party is ultimately held not to be obligated to indemnify the Indemnified Party.

11.7 [\*\*\*]

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## **12. Term and Termination.**

12.1 Term. This Agreement will commence as of the Effective Date and, unless sooner terminated in accordance with the terms hereof or by mutual written consent, will continue until the later of the expiration of the Collaboration Program Term and expiration of the last-to-expire Celgene Option Period (the “Term”).

12.2 Termination by Bluebird. Bluebird will have the right to terminate this Agreement in full upon delivery of written notice to Celgene in the event of any material breach by Celgene of any terms and conditions of this Agreement in a manner that fundamentally frustrates the transactions contemplated by this Agreement, provided that such termination will not be effective if such breach has been cured within [\*\*\*] after written notice thereof is given by Bluebird to Celgene specifying the nature of the alleged breach (or, if such default cannot be cured within such [\*\*\*] after such notice if Celgene commences actions to cure such default within such [\*\*\*] and thereafter diligently continues such actions, but fails to cure the default by the end of such [\*\*\*]); provided, however, that to the extent such material breach involves the failure to make a payment when due, such breach must be cured within [\*\*\*] after written notice thereof is given by Bluebird to Celgene.

### **12.3 Termination by Celgene.**

(a) *Breach.* Celgene will have the right to terminate this Agreement in full upon delivery of written notice to Bluebird in the event of any material breach by Bluebird of any terms and conditions of this Agreement in a manner that fundamentally frustrates the transactions contemplated by this Agreement, provided that such termination will not be effective if such breach has been cured within [\*\*\*] after written notice thereof is given by Celgene to Bluebird specifying the nature of the alleged breach (or, if such default cannot be cured within such [\*\*\*] after such notice if Bluebird commences actions to cure such default within such [\*\*\*] and thereafter diligently continues such actions, but fails to cure the default by the end of such [\*\*\*]).

(b) *Discretionary Termination.* Celgene will have the right to terminate this Agreement in full at its discretion for any reason [\*\*\*] days after delivery of written notice to Bluebird.

12.4 Effects of Termination or Expiration. Upon termination or expiration of this Agreement for any reason, all rights granted by Bluebird to Celgene hereunder will terminate, provided that:

(a) *IND Product Candidates.* Bluebird will, at Celgene’s election, complete initial Phase 1 Studies for one (1) or more IND Product Candidates selected by Celgene, and, if Celgene elects not to have Bluebird complete any such Phase 1 Study, Bluebird may do so at its own expense. In either case, (i) the provisions of Sections 5.1 through 5.6 will apply with respect to any such IND Product Candidates (but excluding Section 5.3 if Celgene has terminated this Agreement pursuant to Section 12.3(a)), (ii) Celgene will grant to Bluebird an exclusive, worldwide, royalty-free right and license in the Field, without the right to grant sublicenses (other than to permitted subcontractors under Section 2.4), under the Celgene IP and Celgene’s interest in jointly owned Collaboration IP solely to complete any such Phase 1 Study,

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and (iii) if Celgene has elected to have Bluebird complete such Phase 1 Study, or if Celgene has not elected to have Bluebird complete a Phase 1 Study but Bluebird has completed such Phase 1 Study and Celgene exercises its option with respect to such IND Product Candidate as set forth in Section 5.1, Celgene will reimburse Bluebird for [\*\*\*] Notwithstanding the foregoing, if Bluebird has terminated this Agreement pursuant to Section 12.2, upon Celgene’s exercise of its option with respect to an IND Product Candidate as set forth in Section 5.1, in lieu of paying the Option Fees and reimbursing Bluebird for its costs and expenses incurred in connection with completing a Phase 1 Study for such IND Product Candidate, Celgene will pay to Bluebird an amount equal to the greater of [\*\*\*] If Celgene does not exercise its option with respect to such IND Product Candidate as set forth in Section 5.1, Bluebird (or an Affiliate designated by Bluebird) and Celgene will, at Bluebird’s option, enter into a License Agreement in the form attached hereto as Exhibit A with respect to the IND Product Candidate, but reversing the roles of the Parties thereunder, *mutatis mutandis*, and updating the Appendices thereto and making such other changes as are appropriate from the context, provided that (A) such license shall be limited to the Celgene IP and jointly owned Collaboration IP as it exists at the time this Agreement has expired or been terminated (including in each case any additions, divisions, continuations, continuations-in-part, invention certificates, substitutions, reissues, reexaminations, extensions, registrations, supplementary protection certificates and renewals of such Celgene IP and Joint Collaboration IP), (B) no Option Fee will be payable by Bluebird in connection with such IND Product Candidates, (C) any royalties or Milestone Payments payable under such License Agreement will be reduced [\*\*\*], (D) such IND Product Candidate will not be subject to the provisions of Section 5.7, (E) if any royalty, milestone or other payment, excluding [\*\*\*], becomes due under any Applicable Celgene In-License that is attributable to Bluebird as a sublicensee (together with its licensees and their respective Affiliates) thereunder with respect to such Development, Manufacture or commercialization, Celgene will pay same, provided that Bluebird will reimburse Celgene for any such payment within thirty (30) days of Bluebird’s receipt of Celgene’s written invoice therefor, and Bluebird’s failure to pay such reimbursement within such time period will entitle Celgene to terminate Bluebird’s sublicense under the applicable Celgene In-License upon thirty (30) days written notice, (F) such License Agreement will be subject to any Target Antigen-related exclusivity agreed to between the Parties under any Development & Commercialization Agreement (whether executed before or after the date of such License Agreement), and (G) Bluebird will be responsible for [\*\*\*] of any amounts owed to Third Parties (including under any Bluebird In-License) in connection with the acquisition of rights in order to Develop or commercialize such IND Product Candidates, provided that any such payments that are royalties will be subject to Section 4.3(d) of such License Agreement.

(b) *Outstanding Options*. Any options exercised by Celgene pursuant to Section 5.1 that have not at the time of termination or expiration resulted in an executed Development & Commercialization Agreement will, at Celgene’s option, be consummated pursuant to Sections 5.2 through 5.4, and Bluebird’s option under Section 5.3 will not terminate unless Celgene has terminated this Agreement pursuant to Section 12.3(a).

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(c) *Pre-IND Product Candidates*. For all Product Candidates (other than Optioned Candidates and IND Product Candidates) for which *in vivo* efficacy or safety studies have been initiated or authorized by the JSC (each, a “Pre-IND Product Candidate”), the following will apply:

(i) The Parties will choose from the pool of Pre-IND Product Candidates as follows:

(A) if such termination is by Celgene pursuant to Section 12.3(a), Celgene will select the first and second Pre-IND Product Candidates, Bluebird will select the third and fourth Pre-IND Product Candidates, and thereafter the selection of each Pre-IND Product Candidate will alternate between Celgene and Bluebird until all Pre-IND Product Candidates have been selected;

(B) if such termination is by Bluebird pursuant to Section 12.2, Bluebird will select the first and second Pre-IND Product Candidates, Celgene will select the third and fourth Pre-IND Product Candidates, and thereafter the selection of each Pre-IND Product Candidate will alternate between the Bluebird and Celgene until all Pre-IND Product Candidates have been selected;

(C) if such termination or expiration occurs other than pursuant to Sections 12.2 or 12.3(a), the first Party to select from the pool of Pre-IND Product Candidates will be chosen by a coin toss. The winner of the coin toss will have the right to select the first Pre-IND Product Candidate, or may instead allow the other Party to select the first Pre-IND Product Candidate. The Party that does not make the first selection from the pool of Pre-IND Product Candidates will have the second and third selections, and thereafter the selection of each Pre-IND Product Candidate will alternate between the Parties until all Pre-IND Product Candidates have been selected; and

(D) In selecting from the pool of Pre-IND Product Candidates, Bluebird will not select any Pre-IND Product Candidate that targets the same Target Antigen that is targeted by any Optioned Candidates or the same Target Antigen that is targeted by the first Pre-IND Product Candidate selected by Celgene pursuant to Section 12.4(c)(i).

(E) In selecting from the pool of Pre-IND Product Candidates, Celgene will not select any Pre-IND Product Candidate that targets the same Target Antigen that is targeted by the first Pre-IND Product Candidate selected by Bluebird pursuant to Section 12.4(c)(i).

(ii) For the first Pre-IND Product Candidate selected by Celgene pursuant to Section 12.4(c)(i), Bluebird will grant to Celgene an exclusive, worldwide, royalty-free right and license in the Field, without the right to grant sublicenses (other than to permitted subcontractors under Section 2.4), under the Bluebird IP and Bluebird’s interest in jointly owned Collaboration IP, solely to conduct research and Development activities with respect to such Pre-IND Product Candidate during the applicable Celgene Option Period. Celgene will have the exclusive option to take an exclusive license from Bluebird under the Bluebird IP and Bluebird’s interest in jointly owned Collaboration IP to commercialize such Pre-IND Product Candidate, exercisable by providing to Bluebird a Celgene Option Notice for same within the time period set forth in Section 5.1. Upon such election by Celgene, the provisions of Sections 5.2 through 5.7 (but excluding Section 5.3 if Celgene has terminated this Agreement pursuant to Section 12.3(a)), Section 5.9 and Sections 8.4 and 8.5 will apply with respect to such Pre-IND Product Candidate,

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provided that (A) the Option Fees payable with respect to such Pre-IND Candidate will be reduced by [\*\*\*], (B) any royalties or Milestone Payments payable under the applicable Development & Commercialization Agreement with respect to such Pre-IND Product Candidate will be reduced by [\*\*\*], (C) Celgene will be responsible for [\*\*\*] of any In-License Payments under the applicable Development & Commercialization Agreement, and other amounts owed to Third Parties (including under any Celgene In-License) in connection with the acquisition of rights in order to Develop or commercialize such Pre-IND Product Candidate, provided that any such In-License Payments and such other payments that are royalties will be subject to Section 4.3(d) of such License Agreement or Section 11.3(d) of any Co-Development, Co-Promote and Profit Share Agreement, as applicable, and (D) Celgene will not have any diligence obligations with respect to such Pre-IND Product Candidate under such Development & Commercialization Agreement.

(iii) For the first Pre-IND Product Candidate selected by Bluebird pursuant to Section 12.4(c)(i), the provisions of Sections 5.1 through 5.6 and Section 5.9 will apply and Celgene will grant to Bluebird an exclusive, worldwide, royalty-free right and license in the Field, without the right to grant sublicenses (other than to permitted subcontractors under Section 2.4), under the Celgene IP and Celgene’s interest in jointly owned Collaboration IP solely to conduct research and Development activities with respect to such Pre-IND Product Candidate. If any royalty, milestone or other payment becomes due under any Applicable Celgene In-License that is attributable to Bluebird as a sublicensee (together with its licensees and their respective Affiliates) thereunder with respect to such Development work, Celgene will pay same, provided that Bluebird will reimburse Celgene for any such payment within thirty (30) days of Bluebird’s receipt of Celgene’s written invoice therefor, and Bluebird’s failure to pay such reimbursement within such time period will entitle Celgene to terminate Bluebird’s sublicense under the applicable Celgene In-License upon [\*\*\*] written notice. If Celgene exercises its option with respect to such Pre-IND Product Candidate as set forth in Section 5.1, Celgene will reimburse Bluebird for [\*\*\*] If Celgene does not exercise its option with respect to such Pre-IND Product Candidate as set forth in Section 5.1, at Bluebird’s option, Bluebird (or an Affiliate designated by Bluebird) and Celgene will enter into a License Agreement in the form attached hereto as Exhibit A with respect to such Pre-IND Product Candidate, but reversing the roles of the Parties thereunder, *mutatis mutandis*, and updating the Appendices thereto and making such other changes as are appropriate from the context, provided that (A) such license shall be limited to the Celgene IP and jointly owned Collaboration IP as it exists at the time this Agreement has expired or been terminated (including in each case any additions, divisions, continuations, continuations-in-part, invention certificates, substitutions, reissues, reexaminations, extensions, registrations, supplementary protection certificates and renewals of such Celgene IP and Joint Collaboration IP), (B) no Option Fee will be payable by Bluebird in connection with such Pre-IND Product Candidate, (C) any royalties or Milestone Payments payable under such License Agreement will be reduced by [\*\*\*], (D) such License Agreement will include Target Antigen-related exclusivity, (E) Milestone Payments under such License Agreement are payable with respect to each Pre-IND Product Candidate to achieve an applicable Milestone Event, (F) Bluebird will be responsible for [\*\*\*] of any amounts owed to Third Parties (including under any Bluebird In-License) in connection with the acquisition of rights in order to Develop or commercialize such Pre-IND Product Candidates, provided that any such payments

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that are royalties will be subject to Section 4.3(d) of such License Agreement, (G) such Pre-IND Product Candidate will not be subject to the provisions of Section 5.7, (H) if any royalty, milestone or other payment becomes due under any Celgene In-License that is attributable to Bluebird as a sublicensee (together with its licensees and their respective Affiliates) thereunder with respect to such Development, Manufacture or commercialization, Celgene will pay same, provided that Bluebird will reimburse Celgene for any such payment excluding [\*\*\*], and Bluebird’s failure to pay such reimbursement within such time period will entitle Celgene to terminate Bluebird’s sublicense under the applicable Celgene In-License upon thirty (30) days written notice, and (I) bluebird will not have any diligence obligations with respect to such Pre-IND Product Candidate under such Development & Commercialization Agreement.

(iv) Except with respect to the first Pre-IND Product Candidate selected by Celgene pursuant to Section 12.4(c)(i), Celgene (or an Affiliate designated by Celgene) and Bluebird, subject to Section 5.9, will enter into a License Agreement in the form attached hereto as Exhibit A with respect to the Pre-IND Product Candidates selected by Celgene in accordance with Section 12.4(c)(i), updating the Appendices thereto and making such other changes as are appropriate from the context, provided that (A) such license shall be limited to the Bluebird IP and jointly owned Collaboration IP as it exists at the time this Agreement has expired or been terminated (including in each case any additions, divisions, continuations, continuations-in-part, invention certificates, substitutions, reissues, reexaminations, extensions, registrations, supplementary protection certificates and renewals of such Celgene IP and Joint Collaboration IP), (B) no Option Fee will be payable by Celgene in connection with such Pre-IND Product Candidates, (C) any royalties or Milestone Payments payable under such License Agreement will be reduced by [\*\*\*], (D) Milestone Payments under such License Agreement are payable with respect to each Pre-IND Product Candidate to achieve an applicable Milestone Event, (E) such License Agreement will not include Target Antigen-related exclusivity, (F) Celgene will be responsible for [\*\*\*] of any In-License Payments under such License Agreement, and other amounts owed to Third Parties (including under any Celgene In-License) in connection with the acquisition of rights in order to Develop or commercialize such Pre-IND Product Candidates, provided that any such In-License Payments and such other payments that are royalties will be subject to Section 4.3(d) of such License Agreement, and (G) Celgene will not have any diligence obligations with respect to such Pre-IND Product Candidate under such Development & Commercialization Agreement.

(v) Except with respect to the first Pre-IND Product Candidate selected by Bluebird pursuant to Section 12.4(c)(i), Bluebird (or an Affiliate designated by Bluebird) and Celgene, subject Section 5.9, will enter into a License Agreement in the form attached hereto as Exhibit A with respect to the Pre-IND Product Candidates selected by Bluebird in accordance with Section 12.4(c)(i), but reversing the roles of the Parties thereunder, *mutatis mutandis*, and updating the Appendices thereto and making such other changes as are appropriate from the context, provided that (A) such license shall be limited to the Celgene IP and jointly owned Collaboration IP as it exists at the time this Agreement has expired or been terminated (including in each case any additions, divisions, continuations, continuations-in-part, invention certificates, substitutions, reissues, reexaminations, extensions, registrations, supplementary protection certificates and renewals of such Celgene IP and Joint Collaboration IP), (B) no Option Fee will be payable by Bluebird in connection with such Pre-IND Product Candidates, (C) any royalties

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or Milestone Payments payable under such License Agreement will be reduced by [\*\*\*], (D) such License Agreement will not include Target Antigen-related exclusivity, (E) Milestone Payments under such License Agreement are payable with respect to each Pre-IND Product Candidate to achieve an applicable Milestone Event, (F) Bluebird will be responsible for [\*\*\*] of any amounts owed to Third Parties (including under any Bluebird In-License) in connection with the acquisition of rights in order to Develop or commercialize such Pre-IND Product Candidates, provided that any such payments that are royalties will be subject to Section 4.3(d) of such License Agreement, (G) if any royalty, milestone or other payment becomes due under any Celgene In-License that is attributable to Bluebird as a sublicensee (together with its licensees and their respective Affiliates) thereunder with respect to such Development, Manufacture or commercialization, Celgene will pay same, provided that Bluebird will reimburse Celgene for any such payment, excluding [\*\*\*], within thirty (30) days of Bluebird’s receipt of Celgene’s written invoice therefor, and Bluebird’s failure to pay such reimbursement within such time period will entitle Celgene to terminate Bluebird’s sublicense under the applicable Celgene In-License upon thirty (30) days written notice, and (H) Bluebird will not have any diligence obligations with respect to such Pre-IND Product Candidate under such Development & Commercialization Agreement.

(d) Neither Party will (i) Develop an IND Product Candidate or Pre-IND Product Candidate following the expiration of the applicable option period set forth in this Section 12.4 with respect to such IND Product Candidate or Pre-IND Product Candidate, or (ii) Commercialize any IND Product Candidate or Pre-IND Product Candidate in each case ((i) and (ii)) without first entering into a License Agreement with the other Party in the form attached hereto as Exhibit A with respect to such IND Product Candidate or Pre-IND Product Candidate as set forth in this Section 12.4. For clarity, any agreement in the form of License Agreement attached hereto as Exhibit A entered into between the Parties pursuant to this Section 12.4 with respect to an IND Product Candidate or Pre-IND Product Candidate, and any resulting Co-Development, Co-Promote and Profit Share Agreement, shall be “Development & Commercialization Agreements” hereunder.

(e) Other than with respect to the rights and licenses granted to Bluebird hereunder pursuant to Section 2.1(h)(ii), all rights granted by Celgene to Bluebird hereunder will terminate.

(f) All executed Development & Commercialization Agreements will continue in full force and effect, provided that if Celgene has terminated this Agreement pursuant to Section 12.3(a), then (i) Bluebird’s rights to co-develop, co-promote and share in profits under any Co-Development, Co-Promote and Profit Share Agreements will terminate, and the Parties promptly will execute a License Agreement to replace each such Co-Development, Co-Promote and Profit Share Agreement, and (ii) all up-front payments, milestone payments and royalty payments under any License Agreement will be reduced by [\*\*\*], provided that such reduction will not apply to the extent any such up-front payments, milestone payments and royalty payments have already been reduced pursuant to Section 10.3(c) of such License Agreement.

12.5 Survival. In addition to the termination consequences set forth in Section 12.4, the following provisions will survive termination or expiration of this Agreement: Sections 1, 2.1(f), 2.1(h)(ii), 2.2, 2.3(a), 2.3(c), 4.3(through the expiration of any options granted to Celgene hereunder), 4.5, 4.6, 5.5, 5.7, 5.8, 5.9, 5.10, 8.7, 9.4, 10, 11, 12.4,



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12.5 and 13, and any other provisions of this Agreement that are required to survive to give effect to any Development & Commercialization Agreement. Termination or expiration of this Agreement will not relieve the Parties of any liability or obligation which accrued hereunder prior to the effective date of such termination or expiration nor preclude either Party from pursuing all rights and remedies it may have hereunder or at law or in equity with respect to any breach of this Agreement nor prejudice either Party’s right to obtain performance of any obligation. All other rights and obligations will terminate upon expiration of this Agreement.

12.6 Right to Set-off. Notwithstanding anything to the contrary in this Agreement, each Party has the right at all times to retain and set off against all amounts due and owing to the other Party as determined in a final judgment any damages recovered by such Party for any Losses incurred by such Party.

### **13. General Provisions.**

#### **13.1 Dispute Resolution for this Agreement and Executed Development & Commercialization Agreements .**

(a) *Disputes*. Disputes arising under or in connection with this Agreement or any executed Development and Commercialization Agreement will be resolved pursuant to this Section 13.1.

(b) *Dispute Escalation*. In the event of a dispute between the Parties, the Parties will first attempt in good faith to resolve such dispute by negotiation and consultation between themselves or the Program Directors. In the event that such dispute is not resolved on an informal basis within twenty (20) days, any Party may, by written notice to the other, have such dispute referred to the Bluebird CEO and the Celgene CEO or in either case his or her designee (who will be a senior executive), who will attempt in good faith to resolve such dispute by negotiation and consultation for a thirty (30) day period following receipt of such written notice.

(c) *Dispute Resolution*. In the event the Parties are not able to resolve such dispute in accordance with Section 13.1(b), either Party may at any time after such 20-day period submit such dispute to be finally settled in the federal courts located in the Southern District of New York. Each Party hereby irrevocably and unconditionally consents to submit to the exclusive jurisdiction of the federal courts located in the Southern District of New York, for any actions, suits or proceedings arising out of or relating to this Agreement and the transactions contemplated hereby. Each Party hereby irrevocably and unconditionally waives any objection to the laying of venue of any action, suit or proceeding arising out of or relating to this Agreement and the transactions contemplated hereby in the federal courts located in the Southern District of New York, and waives and agrees not to plead or claim in any such court that any such action, suit or proceeding brought in such court has been brought in an inconvenient forum. Notwithstanding the foregoing, a Party will be entitled to seek enforcement of a judgment entered pursuant to this Section in any court having competent jurisdiction thereof where enforcement is deemed necessary.

(d) *Injunctive Relief*. Notwithstanding the dispute resolution procedures set forth in this Section 13.1, in the event of an actual or threatened breach hereunder (or any executed Development & Commercialization Agreement, if applicable), the aggrieved Party may seek

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equitable relief (including restraining orders, specific performance or other injunctive relief) in any court or other forum, without first submitting to any dispute resolution procedures hereunder.

(e) *Tolling*. The Parties agree that all applicable statutes of limitation and time-based defenses (such as estoppel and laches) will be tolled while the dispute resolution procedures set forth in this Section 13.1 are pending, and the Parties will cooperate in taking all actions reasonably necessary to achieve such a result. In addition, during the pendency of any dispute under this Agreement initiated before the end of any applicable cure period under Section 12.2 or 12.3 (or the cure periods under any executed Development & Commercialization Agreement, if applicable), (i) this Agreement (or any executed Development & Commercialization Agreement, if applicable) will remain in full force and effect, (ii) the provisions of this Agreement (or any executed Development & Commercialization Agreement, if applicable) relating to termination for material breach will not be effective, (iii) the time periods for cure under Section 12 (and the time periods from any executed Development & Commercialization Agreement, if applicable) as to any termination notice given prior to the initiation of the court proceeding will be tolled, and (iv) neither Party will issue a notice of termination pursuant to this Agreement (or any executed Development & Commercialization Agreement, if applicable) based on the subject matter of the court proceeding (and no effect will be given to previously issued termination notices), until the court has confirmed the existence of the facts claimed by a Party to be the basis for the asserted material breach.

13.2 Cumulative Remedies and Irreparable Harm. All rights and remedies of the Parties hereunder will be cumulative and in addition to all other rights and remedies provided hereunder or available by agreement, at law or otherwise. Each Party acknowledges and agrees that breach of any of the terms or conditions of this Agreement would cause irreparable harm and damage to the other and that such damage may not be ascertainable in money damages and that as a result thereof the non-breaching Party would be entitled to seek from a court equitable or injunctive relief restraining any breach or future violation of the terms contained herein by the breaching Party without the necessity of proving actual damages or posting bond. Such right to equitable relief is in addition to whatever remedies either Party may be entitled to as a matter of law or equity, including money damages.

### 13.3 Business Combination and IP.

(a) *Bluebird Business Combination*. Notwithstanding anything to the contrary herein, for purposes of this Agreement and any Development & Commercialization Agreement, no Know-How, Materials, Patents, Regulatory Data, Regulatory Filings or Regulatory Approvals not Controlled by Bluebird or any of its Affiliates prior to a Business Combination of Bluebird will be Controlled for purposes of this Agreement or any Development & Commercialization Agreement after such Business Combination of Bluebird, other than (i) Collaboration IP, (ii) Bluebird In-Licenses to the extent in effect immediately prior to such Business Combination of Bluebird and later Bluebird Collaboration In-Licenses (provided that after any such Business Combination, Bluebird may, but will not be obligated to, make any Bluebird New In-License available to Celgene or the JSC for review, election or conversion into a Bluebird Collaboration In-License pursuant to Section 4.1), and (iii) any Patent that claims priority, directly or indirectly, to any other Patent first Controlled before such Business Combination of Bluebird will be Controlled thereafter no matter when such Patent is filed or issued.

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(b) *Celgene Business Combination*. Notwithstanding anything to the contrary herein, for purposes of this Agreement and any Development & Commercialization Agreement, no Know-How, Materials, Patents, Regulatory Data, Regulatory Filings or Regulatory Approvals not Controlled by Celgene or any of its Affiliates prior to a Business Combination of Celgene will be Controlled for purposes of this Agreement or any Development & Commercialization Agreement after such Business Combination of Celgene, other than (i) Collaboration IP, (ii) Applicable Celgene In-Licenses, and (iii) any Patent that claims priority, directly or indirectly, to any other Patent first Controlled before such Business Combination of Celgene will be Controlled thereafter no matter when such Patent is filed or issued.

13.4 Relationship of Parties. Nothing in this Agreement is intended or will be deemed to constitute a partnership, agency, employer-employee or joint venture relationship between the Parties. No Party will incur any debts or make any commitments for the other, except to the extent, if at all, specifically provided therein. There are no express or implied third party beneficiaries hereunder (except for Bluebird Indemnitees and Celgene Indemnitees, and any Third Party indemnitees under any executed Development & Commercialization Agreement, if applicable, for purposes of Section 11.6).

13.5 Compliance with Law. Each Party will perform or cause to be performed any and all of its obligations or the exercise of any and all of its rights hereunder in good scientific manner and in compliance with all applicable Law. Without limiting the foregoing, Bluebird will comply with all applicable Laws and regulations (including U.S. Foreign Corrupt Practices Act and any other applicable anti-bribery or anti-kickback laws or regulations).

13.6 Force Majeure. Neither Party will be liable to the other for failure of or delay in performing obligations set forth in this Agreement (other than any obligation to pay monies when due), and neither will be deemed in breach of such obligations, if such failure or delay is due to natural disasters or any causes reasonably beyond the control of such Party and without the fault or negligence of the Party so failing or delaying; provided that the Party affected will promptly notify the other of the force majeure condition and will exert reasonable efforts to eliminate, cure or overcome any such causes and to resume performance of its obligations as soon as possible.

13.7 Governing Law. This Agreement will be governed by and construed in accordance with the Laws of the state of New York, without respect to its conflict of laws rules; provided, however, that any dispute relating to the scope, validity, enforceability or infringement of any Patents or Know-How will be governed by, and construed and enforced in accordance with, the substantive Laws of the jurisdiction in which such Patents or Know-How apply.

13.8 Counterparts; Facsimiles. This Agreement may be executed in one or more counterparts, each of which will be deemed an original, and all of which together will be deemed to be one and the same instrument. Facsimile or PDF execution and delivery of this Agreement by either Party will constitute a legal, valid and binding execution and delivery of this Agreement by such Party

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13.9 Headings. All headings in this Agreement are for convenience only and will not affect the meaning of any provision hereof.

13.10 Waiver of Rule of Construction. Each Party has had the opportunity to consult with counsel in connection with the review, drafting and negotiation of this Agreement. Accordingly, the rule of construction that any ambiguity in this Agreement will be construed against the drafting party will not apply.

13.11 Interpretation. Whenever any provision of this Agreement uses the term “including” (or “includes”), such term will be deemed to mean “including without limitation” (or “includes without limitations”). “Herein,” “hereby,” “hereunder,” “hereof” and other equivalent words refer to this Agreement as an entirety and not solely to the particular portion of this Agreement in which any such word is used. All definitions set forth herein will be deemed applicable whether the words defined are used herein in the singular or the plural. Unless otherwise provided, all references to Sections and Exhibits in this Agreement are to Sections and Exhibits of this Agreement. References to any Sections include Sections and subsections that are part of the related Section ( e.g., a section numbered “Section 2.1” would be part of “Section 2”, and references to “Section 2.1” would also refer to material contained in the subsection described as “Section 2.1(a)”).

13.12 Binding Effect. This Agreement will inure to the benefit of and be binding upon the Parties, their Affiliates, and their respective lawful successors and assigns.

13.13 Assignment. This Agreement may not be assigned by either Party, nor may either Party delegate its obligations or otherwise transfer licenses or other rights created by this Agreement, except as expressly permitted hereunder or otherwise without the prior written consent of the other Party, which consent will not be unreasonably withheld, delayed or conditioned; provided that without consent (i) Celgene may assign this Agreement to (x) an Affiliate or (y) its successor in connection with the merger, consolidation, or sale of all or substantially all of its assets, and (ii) Bluebird may assign this Agreement to (x) an Affiliate or (y) its successor in connection with the merger, consolidation, or sale of all or substantially all of its assets or that portion of its business pertaining to the subject matter of this Agreement; provided however that, except in the case where a Party is involved in a merger or consolidation where it is the surviving entity and no assets of such Party have been transferred as a result of such merger or consolidation, that (A) such assigning Party provides the other Party to this Agreement with at least thirty (30) business days advance written notice of such assignment(s) and the assigning Party agrees in a written agreement delivered prior to such assignment(s) to the non-assigning Party (and upon which such non-assigning Party may rely) to remain fully liable for the performance of its obligations under this Agreement by its assignee(s), (B) the assignee(s) agree in a written agreement delivered prior to such assignment(s) to the non-assigning Party (and upon which such non-assigning Party may rely) to assume performance of all such assigned obligations, (C) in the case of any assignment(s) by Bluebird, all Bluebird IP licensed to Celgene or subject to Celgene’s option rights under this Agreement, along with all Product Candidates will be transferred to such assignee(s) effective as of such assignment(s), (D) all of the matters referred to in clauses (A), (B) and (C), as applicable, will be set forth in documentation reasonably acceptable to the non-assigning Party prior to any such assignment(s) (and with such reasonable acceptance not to be unreasonably withheld, conditioned or delayed) and in all cases

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will provide the non-assigning Party with the full benefits of its rights under this Agreement (after taking into account all risks involving applicable counterparty performance and bankruptcy and insolvency risks, including those involving contractual rejection under 11 USC §365) as if no such assignment(s) had occurred, and (E) in the case of any assignment(s), the assigning Party will reimburse the non-assigning Party for all of the legal fees and expenses incurred by such non-assigning Party in connection with the matters set forth in clause (D) of this sentence in an aggregate amount not to exceed \$50,000; and provided, further, that if Bluebird wishes to assign any Bluebird IP to its Affiliates, it will be permitted to do so conditioned on such Affiliate becoming a party to this Agreement, in the form of an amendment to this Agreement executed by Celgene, Bluebird and such Affiliate, pursuant to which such Affiliate would agree to assume all obligations hereunder, and grant to Celgene all rights hereunder, with respect to the Bluebird IP so assigned. The terms of this Agreement will be binding upon and will inure to the benefit of the successors, heirs, administrators and permitted assigns of the Parties. Any purported assignment in violation of this Section 13.13 will be null and void *ab initio*.

13.14 Notices. All notices, requests, demands and other communications required or permitted to be given pursuant to this Agreement will be in writing and will be deemed to have been duly given upon the date of receipt if delivered by hand, recognized international overnight courier, confirmed facsimile transmission, or registered or certified mail, return receipt requested, postage prepaid to the following addresses or facsimile numbers:

If to Bluebird:           bluebird bio, Inc.  
840 Memorial Drive  
Cambridge, MA 02139  
Attention: President & CEO  
Facsimile:

With a copy to:           Goodwin|Procter LLP  
53 State Street  
Boston, MA 02109  
Attention: Michael Bison, Esq. & Kingsley Taft, Esq.  
Facsimile: 617-523-1231

If to Celgene:            Celgene Corporation  
86 Morris Avenue  
Summit, NJ 07901  
Attention: George Golumbeski, Ph. D.  
Facsimile: 908-673-2791

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with a copy to: Celgene Legal  
86 Morris Avenue  
Summit, NJ 07901  
Attention: General Counsel  
Telephone: (908) 673-9000  
Facsimile: (908) 673-2771

Dechert LLP  
902 Carnegie Center  
Suite 500  
Princeton, NJ 08540  
Attention: James J. Marino, Esq.  
David E. Schulman, Esq.  
Telephone: (609) 955-3230  
Facsimile: (609) 873-9138

Either Party may change its designated address and facsimile number by notice to the other Party in the manner provided in this Section 13.14.

13.15 Amendment and Waiver. This Agreement may be amended, supplemented, or otherwise modified only by means of a written instrument signed by both Parties; provided that any unilateral undertaking or waiver made by one Party in favor of the other will be enforceable if undertaken in a writing signed by the Party to be charged with the undertaking or waiver. Any waiver of any rights or failure to act in a specific instance will relate only to such instance and will not be construed as an agreement to waive any rights or fail to act in any other instance, whether or not similar.

13.16 Severability. In the event that any provision of this Agreement will, for any reason, be held to be invalid or unenforceable in any respect, such invalidity or unenforceability will not affect any other provision hereof, and the Parties will negotiate in good faith to modify this Agreement to preserve (to the extent possible) their original intent.

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13.17 Payment Floor. Except as permitted by Section 12.6, Section 10.6 of any License Agreement or Section 17.6 of any Co-Development, Co-Promote and Profit Share Agreement, in no event will any credits permitted to be taken by Celgene under this Agreement or any Development & Commercialization Agreement against any particular Milestone Payment, royalty payment or Profit & Loss Share payment owed to Bluebird under any Development & Commercialization Agreement act to reduce such payment by more than [\*\*\*] than would otherwise be payable to Bluebird thereunder or thereunder (and for clarity “otherwise payable” above means that (i) any reductions pursuant to Section 10.3(c) of any License Agreement or Section 17.3 of any Co-Development, Co-Promote and Profit Share Agreement will be made before determining the [\*\*\*] floor specified above, but (ii) any royalty reductions pursuant to Section 4.3(d) of any License Agreement or Section 11.3(d) of any Co-Development, Co-Promote and Profit Share Agreement will be included in calculating the up to [\*\*\*] reduction permitted above).

13.18 Entire Agreement. This Agreement is the sole agreement with respect to the subject matter and supersedes all other agreements and understandings between the Parties with respect to same (including the Confidential Agreement).

*[Remainder of this Page Intentionally Left Blank]*

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IN WITNESS WHEREOF, the Parties have caused this Master Collaboration Agreement to be executed by their respective duly authorized officers as of the Effective Date.

BLUEBIRD BIO, INC.

By: /s/ Nick Leschly  
(Signature)

Name: Nick Leschly

Title: CEO

Date: March 19, 2013

CELGENE CORPORATION

By: /s/ Perry Karsen  
(Signature)

Name: Perry Karsen

Title: COO

Date: March 19, 2013



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**Exhibit A**

**License Agreement**

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**License Agreement**

**by and between**

**bluebird bio, Inc.**

**and**

**Celgene Corporation**

[            ]

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**List of Appendices**

- Appendix A Additional Definitions
- Appendix B Applicable New In-Licenses
- Appendix C Applicable Pre-Existing In-Licenses
- Appendix D Certain Manufacturing Definitions
- Appendix E Press Release
- Appendix F Certain Patents Within the Licensed IP  
as of the License Agreement Effective Date
- Appendix G Bluebird Agreements
- Schedule 9.2 Exceptions to Bluebird’s Representations and Warranties

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LICENSE AGREEMENT

### License Agreement

This License Agreement (this “License Agreement”), dated as of [ ] (the “License Agreement Effective Date”), is made by and between bluebird bio, Inc., a Delaware corporation (“Bluebird”), and Celgene Corporation, a Delaware Corporation (“Celgene”). Each of Bluebird and Celgene may be referred to herein as a “Party” or together as the “Parties.”

WHEREAS, Bluebird has developed and owns or has rights to certain Patents and technology relating to developing innovative gene therapies for genetic disorders;

WHEREAS, Celgene is a biopharmaceutical company focused on acquiring, Developing and Commercializing innovative anti-cancer agents; and

WHEREAS, Bluebird and Celgene are parties to that certain Master Collaboration Agreement (dated [ ], 2013) (the “Master Collaboration Agreement”) pursuant to which Celgene has an option to take a license to Product Candidates;

WHEREAS, pursuant to the terms of the Master Collaboration Agreement, Celgene has exercised its option to select a Product Candidate to be an Optioned Candidate by delivering to Bluebird a Celgene Option Notice and payment of the applicable Initial Option Fee and Additional Option Fee (such Optioned Candidate, as defined more fully in Appendix A, the “Elected Candidate”); and

WHEREAS, the Parties now wish to enter into an exclusive licensing arrangement whereby Celgene will have exclusive rights to Develop and Commercialize Licensed Product, all on the terms and conditions set forth here.

NOW, THEREFORE, in consideration of the mutual covenants contained herein, and for other good and valuable consideration, the amount and sufficiency of which are hereby acknowledged, the Parties hereby agree as follows:

#### 1. Definitions.

The following terms and their correlatives will have the meanings set forth below. Capitalized terms used, but not defined, herein will have the meanings ascribed to such terms in the Master Collaboration Agreement.

1.1 “Applicable Bluebird In-Licenses” means the Applicable Pre-Existing In-Licenses and the Applicable New In-Licenses.

1.2 “Applicable New In-Licenses” means all New In-Licenses of Bluebird or its Affiliates necessary or useful for the research, Development and/or Commercialization of Elected Candidate and Licensed Product that Celgene has elected to list on Appendix B as of the License Agreement Effective Date, plus any other New In-License of Bluebird or its Affiliates that Celgene has elected to include as an Applicable New In-License pursuant to Section 3.2(b).

1.3 “Applicable Pre-Existing In-Licenses” means all Pre-Existing In-Licenses necessary or useful for the research, Development and/or Commercialization of Elected Candidate and Licensed Product, and any extensions or expansions of the scope of such Pre-Existing In-Licenses, including those listed on Appendix C.

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1.4 “Biosimilar Product” means, with respect to a Licensed Product in any country, any biosimilar product sold by a Third Party not authorized by or on behalf of Celgene, its Affiliates or Sublicensees, (i) that is a biosimilar biological product, as defined in 21 USC 379j-51 (or any successor or replacement thereof), a similar biological medicinal product, as defined in Annex I to Directive 2001/83/EC (or any successor or replacement thereof), or any similar biosimilar or generic product under the Laws of any country or jurisdiction, or (ii) regarding which Regulatory Approval is obtained by referencing Regulatory Data of such Licensed Product.

1.5 “Bluebird In-Licensed IP” means all Patents, Materials and Know-How in-licensed by Bluebird pursuant to Applicable Bluebird In-Licenses, including any extensions or expansions of the scope thereof.

1.6 “Bluebird Technology” means all Bluebird Solely Owned IP and all of Bluebird’s right, title and interest in and to Joint IP.

1.7 “Celgene Development & Commercialization Program” means a Development and Commercialization program for Licensed Product in the Field worldwide.

1.8 “Celgene Licensed Product In-License” means [\*\*\*]

1.9 “Celgene Licensed Product In-Licensed IP” means [\*\*\*]

1.10 “Celgene Licensed Product IP” means [\*\*\*]

1.11 “Celgene Other In-License” means [\*\*\*]

1.12 “Celgene Regulatory Rights” means all Regulatory Data, Regulatory Filings and Regulatory Approvals for Elected Candidate and Licensed Product worldwide Controlled by Celgene or any of its Affiliates.

1.13 “Celgene Technology” means all Celgene Solely Owned IP and all of Celgene’s right, title and interest in and to Joint IP.

1.14 “Commercialization” means any and all activities directed to the Manufacturing, marketing, detailing, promotion and securing of reimbursement of a product after Regulatory Approval has been obtained (including making, having made, using, importing, selling and offering for sale such product), and will include post-approval clinical studies, post-launch marketing, promoting, detailing, marketing research, distributing, customer service, administering and commercially selling such product, importing, exporting or transporting such product for commercial sale, and all regulatory compliance with respect to the foregoing.

1.15 “Commercially Reasonable Efforts” means, with respect to the Development or Commercialization of Licensed Product by a Party, that level of efforts and resources that such Party would normally devote to the Development or Commercialization, as the case may be, of a product owned by it or to which it has rights of the type it has hereunder, which is of a similar commercial potential at a similar stage in its lifecycle, in each case taking into account issues of safety and efficacy, product profile, the proprietary position, the then current competitive environment for such product and the likely timing of such product’s entry into the market, the pricing and launching strategy for the respective product, the regulatory environment and status of such product, and other relevant scientific, technical and commercial factors.

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1.16 “Control” or “Controlled” means, with respect to any Know-How, Material, Patent, Regulatory Data, Regulatory Filings and Regulatory Approvals, the possession (whether by ownership or license, other than by a license or sublicense granted pursuant to this License Agreement) by a Party or its Affiliates of the ability to grant to the other Party a license or access as provided herein to such item, without violating the terms of any agreement or other arrangement with any Third Party or, other than under Applicable Bluebird In-Licenses, being obligated to pay any royalties or other consideration therefor ( “Additional Payments”). For clarity, Other In-Licenses are not “Controlled” for purposes of this License Agreement, unless and only after such Other In-License is converted into an Applicable New In-License pursuant to Section 3.2(b). Notwithstanding the foregoing, as provided in Section 3.2(a), if on or after the License Agreement Effective Date and for such time as the other Party agrees to pay and does in fact pay all Additional Payments with respect to such Party’s access or license to any Know-How, Material, Patent, Regulatory Data, Regulatory Filings and Regulatory Approvals (other than that in-licensed by Bluebird pursuant to an Other In-License), such Know-How, Material, Patent, Regulatory Data, Regulatory Filings and Regulatory Approvals will be deemed to be included in the definition of “Control”.

1.17 “Covers”, with reference to (i) a Patent, means that the making, using, selling, offering for sale or importing of a product or practice of a method would infringe a Valid Claim of such Patent in the country in which such activity occurs, and (ii) Materials or Know-How, means that the Manufacture, Development or Commercialization of a product incorporates, embodies or otherwise makes use of such Materials or Know-How.

1.18 “EU” means the organization of member states of the European Union as it may be constituted from time to time.

1.19 “EU Regulatory Event” means, with respect to a Licensed Product, the earlier to occur of [\*\*\*]

1.20 “First Commercial Sale” means [\*\*\*]

1.21 “First Indication” means the [\*\*\*]

1.22 “GAAP” means U.S. generally accepted accounting principles or International Financial Reporting Standards, consistently applied, as designated and used by the applicable Party.

1.23 “In-License Payments” means any amounts paid or payable under any Applicable Bluebird In-License that are incurred by Bluebird solely and directly as a result of the grant of a sublicense thereunder under this License Agreement to Celgene, any of Celgene’s contract Third Parties under Section 3.5, or any further Sublicensees of Celgene (including of Celgene’s Affiliates that are granted sublicenses) under this License Agreement. Any such payments will include [\*\*\*] but excluding [\*\*\*]

1.24 “Licensed IP” means all (i) Patents, Materials and Know-How Controlled at any time during the term of this License Agreement by Bluebird or any of its Affiliates (including any applicable Collaboration IP and Bluebird Technology), other than pursuant to an Applicable Bluebird In-License, and (ii) Bluebird In-Licensed IP, in each case to the extent necessary or useful to Develop Elected Candidate and Develop and Commercialize Licensed Product.

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1.25 “Licensed Product” means any product that constitutes or incorporates an Elected Candidate (including all modified and improved versions thereof), in all forms, presentations, and formulations (including manner of delivery and dosage). A modified or improved version of an Elected Candidate constituted or incorporated in a product will be deemed a “Modified Licensed Product” for purposes of Section 4.2 if it is Covered by patentable technology Controlled by Bluebird that (i) is first discovered, created, conceived, developed or reduced to practice after the later of (a) the License Agreement Effective Date and (b) the end of the Collaboration Program Term, (ii) requires the submission of a new BLA with respect to such modified or improved Elected Candidate, and (iii) materially contributes to the Elected Candidate being approved for a new indication or new patient population. For clarity, “Modified Licensed Products” are Licensed Products hereunder for all purposes other than Section 4.2.

1.26 “Manufacturing” means the production, manufacture, processing, filling, finishing, packaging, labeling, shipping and holding of product or any intermediate thereof, including process development, process qualification and validation, scale-up, commercial manufacture and analytic development, product characterization, stability testing, quality assurance and quality control. With reference to Elected Candidate and Licensed Product, Manufacturing includes Vector and associated Payload supply.

1.27 “Net Sales” means [\*\*\*]

1.28 “Pivotal Study” means (i) a Phase 3 Study that is intended by Celgene to be submitted (together with any other registration trials that are prospectively planned when such Phase 3 Study is initiated) for Regulatory Approval in the U.S. or the EU, or (ii) any other clinical study that is designed to establish that a pharmaceutical product is safe and efficacious for its intended use, and to determine warnings, precautions, and adverse reactions that are associated with such pharmaceutical product in the dosage range to be prescribed, which clinical study is a registration trial intended to be sufficient for filing an application for a Regulatory Approval for the Licensed Product in the U.S. or another country or some or all of an extra-national territory, solely as evidenced by the acceptance for filing for a Regulatory Approval for such product after completion of such study.

1.29 “Regulatory Exclusivity Period” means with respect to a Licensed Product in a country, the period of time during which (a) Celgene or any of its Affiliates or Sublicensees has been granted the exclusive legal right by a Regulatory Authority (or is otherwise entitled to the exclusive legal right by operation of Law) in such country to market and sell the Licensed Product, or (b) the data and information submitted by Celgene or any of its Affiliates or Sublicensees to the relevant Regulatory Authority in such country for purposes of obtaining Regulatory Approval may not be disclosed, referenced or relied upon in any way by such Regulatory Authority (including by relying upon the Regulatory Authority’s previous findings regarding the safety or effectiveness of the Licensed Product) to support the Regulatory Approval or marketing of any product by a Third Party in such country.

1.30 “Second Indication” means a [\*\*\*]

1.31 “Selling Party” means Celgene and its Sublicensees (including Celgene’s Affiliates that are granted sublicenses pursuant to Section 3.3).



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1.32 “Sublicensee” means any person or entity (including Affiliates of Celgene) that is granted a sublicense as permitted by Section 3.3 (or an option to take such a sublicense), either directly by Celgene or indirectly by any other Sublicensee hereunder.

1.33 “Valid Claim” means, with respect to a particular country, (i) any claim of an issued and unexpired Patent in such country that (a) has not been held revoked, unenforceable or invalid by a decision of a court or governmental agency of competent jurisdiction, which decision is unappealable or unappealed within the time allowed for appeal and (b) has not been abandoned, disclaimed, denied or admitted to be invalid or unenforceable through reissue or disclaimer or otherwise in such country, or (ii) a claim of a pending Patent application that has not been finally abandoned or finally rejected or expired and which has been pending [\*\*\*] from the date of filing of the earliest priority Patent application to which such pending Patent application is entitled to claim benefit.

1.34 “Vector Supplies” means supplies of Vectors and associated Payloads Manufactured for incorporation into Elected Candidate and Licensed Product for Development or Commercialization thereof.

Definitions for each of the following terms are found in the body of this License Agreement or the Appendices hereto as indicated below:

<u>Defined Terms</u>	<u>Location</u>
Additional IP	Section 3.2(a)
Allocable Manufacturing Overhead [***]	Appendix D
Biosimilar Product Competition	Section 4.3(e)
Bluebird Indemnitees	Section 9.6(a)
Business Acquisition	Section 3.4
Business Party	Section 3.4
Business Program	Section 3.4
Celgene Indemnitees	Section 9.6(b)
Commercial Supplies	Appendix D
Competitive Infringement	Section 7.1
Elected Candidate	Appendix A
Fully Burdened Manufacturing Cost	Appendix D
Indemnification Claim Notice	Section 9.6(c)
Indemnified Party	Section 9.6(c)
Joint IP	Section 5.2
License Agreement Term	Section 10.1
Losses	Section 9.6(a)
Major EU Countries	Section 1.18
Manufacturing and Supply Agreement	Section 2.4(c)(ii)
Milestone Event	Section 4.2
Milestone Payment	Section 4.2

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<u>Defined Terms</u>	<u>Location</u>
Modified Licensed Product [***]	Section 1.21
Solely Owned IP	Section 5.1
Third Party Claims	Section 9.6(a)

## **2. Development and Commercialization.**

2.1 Development. As of and after the License Agreement Effective Date, Celgene will assume sole responsibility for, and control of, Developing Elected Candidate and Licensed Product in the Field worldwide, and will establish a Celgene Development & Commercialization Program for that purpose. As of and after the License Agreement Effective Date, Celgene will have sole responsibility for all costs and expenses arising from the Development and Commercialization of Elected Candidate and Licensed Product in the Field worldwide. Notwithstanding the foregoing, if the initial Phase 1 Study with respect to Optioned Candidate has not been completed as of the License Agreement Effective Date, at Celgene’s election, Bluebird will continue to be responsible for the performance of such initial Phase 1 Study under the oversight of the JSC under the Master Collaboration Agreement until completion of such initial Phase 1 Study. In the event Bluebird continues, at Celgene’s election, to continue to be responsible for the performance of such initial Phase 1 Study, Bluebird will be responsible for the costs of performing such initial Phase 1 Study until the earlier to occur of (i) completion of such initial Phase 1 Study and (ii) expiration or termination of the Collaboration Program Term; following the end of the Collaboration Program Term, Celgene will reimburse Bluebird for the out-of-pocket costs of performing such initial Phase 1 Study incurred after the end of the Collaboration Program Term within thirty (30) days of Celgene’s receipt of Bluebird’s written invoice therefor.

2.2 Regulatory. Subject to the last sentence of Section 2.1, (i) as of and after the License Agreement Effective Date, Celgene will lead and have sole control of all efforts with Regulatory Authorities regarding the Development and Commercialization of Elected Candidate and Licensed Product in the Field worldwide, including taking full responsibility for preparing and filing the relevant Regulatory Filings and seeking Regulatory Approval and (ii) promptly following the License Agreement Effective Date, Bluebird will, at Celgene’s expense, assign to Celgene all Regulatory Filings with respect to Elected Candidate and Licensed Product. For clarity, in the event Bluebird continues to be responsible for the performance of an initial Phase 1 Study following the License Agreement Effective Date in accordance with Section 2.1, Bluebird will retain ownership of any Regulatory Filings (including the IND) for Optioned Candidate until completion of such initial Phase 1 Study. In the event of failure to assign such Regulatory Filings to Celgene, Bluebird hereby consents and grants to Celgene the right to access and reference (without any further action required on the part of Bluebird, whose authorization to file this consent with any Regulatory Authority is hereby granted) any such Regulatory Filing.

2.3 Technical Assistance. During the Collaboration Program Term, Bluebird will reasonably cooperate with Celgene to provide all technical assistance, and to transfer to Celgene any additional Know-How licensed to Celgene under Section 3.1, requested by Celgene to facilitate the transfer of Development efforts related to Elected Candidate and Licensed Product.

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Such cooperation will include providing Celgene with reasonable access by teleconference or in-person at Bluebird’s facilities to Bluebird personnel involved in the research and Development of Elected Candidate to provide Celgene with a reasonable level of technical assistance and consultation in connection with the transfer of such Know-How. Following the Collaboration Program Term, Bluebird will reasonably cooperate with Celgene to provide reasonable amounts of technical assistance, including to transfer to Celgene any additional Know-How licensed to Celgene under Section 3.1, with respect to Elected Candidate or Licensed Product as reasonably requested by Celgene with reasonable advance notice to Bluebird. Any dispute with respect to the amount and completeness of the technical assistance and cooperation to be provided by Bluebird under this Section 2.3 will be referred to and finally resolved by binding arbitration by a mutually agreeable, disinterested, conflict-of-interest-free individual not affiliated or consulting with either Party. Any such arbitration will be conducted under the then-current rules of the American Arbitration Association.

#### 2.4 Manufacture and Supply.

(a) *Manufacturing.* Subject to Section 2.4(b), Celgene will be solely responsible for, and will bear all the costs and expenses of, Manufacturing and supplying all Elected Candidate and Licensed Product for Development and Commercialization in the Field worldwide and, subject to Section 2.4(c), Celgene will purchase Vector Supply from Bluebird or its designee for such purpose.

(b) *Vector Supply.* Bluebird will have the sole right to Manufacture or have Manufactured Vector Supply, and Celgene will have no rights with respect thereto except as provided in Section 2.4(c)(iv). Except as provided in Section 2.4(c)(iv) or in the Manufacturing Supply Agreement, neither Celgene nor any Affiliate of Celgene (nor any others on behalf of or under license or sublicense from Celgene or any of its Affiliates) will Manufacture (i) any Vector and associated Payload for Licensed Product or (ii) Licensed Product, except for the Manufacture of Licensed Product using Vector Supply supplied by or on behalf of Bluebird. Except as provided in Section 2.4(c)(iv) or in the Manufacturing Supply Agreement, Celgene and its Affiliates and Sublicensees will purchase all Vector Supply exclusively from Bluebird or its designee.

#### (c) *Vector Supply Terms.*

(i) Except as provided otherwise in this Section 2.4(c) or in the Manufacturing Supply Agreement, Bluebird and its Affiliates will Manufacture, or cause a Third Party to Manufacture, all Vector Supply for all Elected Candidate and Licensed Product required for clinical Development and Commercialization in the Field worldwide, and will have the right to make all necessary decisions regarding arrangements with Third Party manufacturers, provided that Bluebird will reasonably consult with Celgene with respect to all such arrangements and obtain Celgene’s prior written consent, which will not be unreasonably withheld, conditioned or delayed. [\*\*\*]

(ii) The Parties will enter into a “Manufacturing and Supply Agreement,” between each other or among the Parties and an Affiliate or a Third Party, covering Vector Supply as soon as reasonably practicable after the License Agreement Effective Date, which agreement will be consistent with and supersede the terms of this Section 2.4(c) and will otherwise be subject in all respects to the terms and conditions of this License Agreement.

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(iii) The cost to Celgene of Vector Supply will equal [\*\*\*] of Bluebird’s Fully Burdened Manufacturing Cost for such Manufacture, plus [\*\*\*], unless otherwise agreed by the Parties in writing.

(iv) The Manufacturing and Supply Agreement will include the terms set forth in Appendix H, including terms permitting Celgene to establish “back-up” and/or “second source” rights for Vector Supply and license grants from Celgene to Bluebird under the Celgene Licensed Product IP and Celgene Licensed Product In-Licensed IP to the extent necessary or useful for Bluebird to Manufacture Vector Supply. [\*\*\*]

(v) At Celgene’s request, Bluebird will cooperate with Celgene’s reasonable requests, at Celgene’s cost and expense, to engage in a technology transfer to allow Celgene, in accordance with Section 2.4(c)(iv), to Manufacture Vector Supply (through the first commercial batch of Vector Supply) itself or by through its designated Third Party manufacturer, by transferring all Know-How, Materials, technology and trade secrets Controlled by Bluebird or its Affiliates that are necessary to Manufacture Vector Supply, thereby enabling Celgene (or such Third Party) to Manufacture the Vector Supply.

(vi) Any purchase of Vector Supply from Bluebird or its designee will expressly not include any license rights to any Know-How or Patents, but instead all licenses (implied, by exhaustion or otherwise) will arise under Section 3.1, if and as applicable.

(vii) For the purpose of this License Agreement, certain words and phrases (and their correlatives) relating to Manufacturing will have the meanings set forth on Appendix D.

**2.5 Celgene Diligence.** Celgene, directly or through one or more of its Sublicensees, will use Commercially Reasonable Efforts: (i) to Develop Licensed Product in the Field and to obtain Regulatory Approvals therefor; and (ii) to Commercialize Licensed Product in the Field after obtaining such Regulatory Approval, in each country worldwide where Commercializing Licensed Product would be warranted by using Commercially Reasonable Efforts.

**2.6 Annual Update Meetings.** At least once during each consecutive twelve (12)-month period from the License Agreement Effective Date until the earlier of first approval of a BLA for Licensed Product by the FDA or first approval of an MAA for Licensed Product by the EMA, within thirty (30) days of Bluebird’s written request, the Parties will meet in person at a U.S. site of Celgene for Celgene to provide Bluebird with an update on the Development of Licensed Product by Celgene and its Sublicensees. During such meeting, Celgene will disclose to Bluebird all material information regarding such Development.

**2.7 Reports by Celgene.** Celgene will prepare and maintain, and will cause its Sublicensees to prepare and maintain, reasonably complete and accurate records regarding the Development of Elected Candidate and Licensed Product, and Commercialization of Licensed Product worldwide after Regulatory Approval therefor. Celgene will provide to Bluebird a reasonably detailed report regarding such efforts at least once every twelve (12)-month period from the License Agreement Effective Date. Such report will contain sufficient detail to enable Bluebird to assess Celgene’s compliance with its Development and Commercialization obligations in Section 2.5, including information with respect to the following: (i) the design,

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status and results of any animal studies and clinical trials for Licensed Product; (ii) any regulatory milestones, and any Regulatory Approvals achieved, for Licensed Product; and (iii) activities with respect to selling, promoting, supporting, detailing and marketing of Licensed Product. In addition to the foregoing, Celgene will provide Bluebird with such additional information regarding any such activities as Bluebird may reasonably request from time to time.

## 2.8 Applicable Bluebird In-Licenses and Other IP.

(a) *Maintenance of Applicable Bluebird In-Licenses.* Bluebird (i) will duly perform and observe all of its obligations under the Applicable Bluebird In-Licenses in all material respects and maintain in full force and effect the Applicable Bluebird In-Licenses, and (ii) will not, without Celgene’s prior written consent (such consent not to be unreasonably withheld, conditioned or delayed), (1) amend, modify, restate, cancel, supplement or waive any provision of any Applicable Bluebird In-License, or grant any consent thereunder, or agree to do any of the foregoing, or (2) exercise any right to terminate any Applicable Bluebird In-License in each case ((1) and (2)) that would reasonably be expected to adversely affect in any respect the rights of Celgene under this License Agreement, provided that Bluebird will provide prior written notice to Celgene of all of the foregoing notwithstanding whether or not any of the foregoing would reasonably be expected to adversely affect in any respect the rights of Celgene under this License Agreement. Bluebird will provide Celgene with written notice as promptly as practicable (and in any event within five (5) business days) after becoming aware of any of the following: (A) any material breach or default by Bluebird or any of its Affiliates of any covenant, agreement or other provision of any Applicable Bluebird In-License, (B) any notice or claim from the counterparty to any Applicable Bluebird In-License terminating or providing notice of termination of any Applicable Bluebird In-License, (C) any notice or claim alleging any breach of default under any Applicable Bluebird In-License, or (D) the existence of any facts, circumstances or events which alone or together with other facts, circumstances or events could reasonably be expected (with or without the giving of notice or passage of time or both) to give rise to a breach of or default under or right to terminate any Applicable Bluebird In-License. If Bluebird fails to pay any amounts due under any Applicable Bluebird In-License and if such nonpayment would permit the counterparty to such Applicable Bluebird In-License to terminate or suspend the same or any rights thereunder, Celgene will have the right, but not the obligation, in its sole discretion, to pay such amounts on Bluebird’s behalf, and any amounts so paid by Celgene may be taken by Celgene as a credit against any amounts payable to Bluebird under this License Agreement.

(b) [\*\*\*]

(c) *Applicable Bluebird In-License Requirements.* Celgene will abide, and will cause all its Affiliates and applicable Sublicensees to abide, by all requirements of each Applicable Bluebird In-License in all material respects (and in any case in all respects in the case that failure to so abide would result in a breach under the Applicable Bluebird In-License), to the extent applicable to sublicensees thereunder and to the extent disclosed by Bluebird to Celgene, with the understanding that disclosure by Bluebird of any Applicable Bluebird In-License to Celgene will be deemed disclosure of such requirements of such Applicable Bluebird In-License to Celgene. In the event of a termination of any Applicable Bluebird In-License, Bluebird agrees, to the extent requested by Celgene, to reasonably assist Celgene in securing a direct license from

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the applicable licensor under any Patents, Materials and Know-How that was licensed to Bluebird and sublicensed to Celgene hereunder prior to such termination. In addition, Bluebird agrees, if requested by Celgene, to reasonably assist Celgene in securing a standby license from the applicable licensor under any Patents, Materials and Know-How that are licensed to Bluebird and sublicensed to Celgene.

### **3. License Grants.**

3.1 License by Bluebird. Subject to the terms and conditions of this License Agreement, Bluebird hereby grants to Celgene a worldwide, exclusive (even as to Bluebird) license, with the right to sublicense only as permitted by Section 3.4, under Licensed IP, to Develop Elected Candidate and to Develop and Commercialize Licensed Product. Further, (i) the license to Commercialize granted in this Section 3.1 will cover only the sale and offer for sale of Licensed Product in finished form and not the sale or offer for sale of Vectors (other than as and to the extent incorporated in the Licensed Product), and (ii) rights to Manufacture Vectors and associated Payloads are included within the scope of the license granted to Celgene under this Section 3.1, which rights are subject to the terms and conditions of Section 2.4(c).

#### 3.2 Additional IP; Other In-Licenses.

(a) *Additional IP.* Except as set forth in Section 3.2(b), Celgene may, on or after the License Agreement Effective Date, elect to include within the scope of the Licensed IP any Know-How, Material, Patent, Regulatory Data, Regulatory Filings or Regulatory Approvals (“Additional IP”), that would be Controlled by Bluebird but for required payments of Additional Payments to a Third Party, by (i) providing notice to Bluebird of same and (ii) agreeing to pay and in fact paying all Additional Payments with respect to Celgene’s access or license to such Additional IP. Following Bluebird’s receipt of such notice and subject to Celgene’s performance of its obligations to pay any Additional Payments with respect to Celgene’s access or license to such Additional IP, such Additional IP will be deemed Licensed IP hereunder. For avoidance of doubt, this Section 3.2(a) does not apply to Know-How, Materials, Patents, Regulatory Data, Regulatory Filings or Regulatory Approvals licensed to Bluebird under the Applicable Bluebird In-Licenses, all of which are deemed Controlled by Bluebird notwithstanding this Section 3.2(a).

(b) *Other In-Licenses.* Celgene may, on or after the License Agreement Effective Date, elect to convert any Other In-License to an Applicable New In-License by providing notice to Bluebird of same. Upon Bluebird’s receipt of such notice, such Other In-License will be an Applicable New In-License hereunder, Appendix B will automatically be updated to include such New In-License and the provisions of this License Agreement applicable to New In-Licenses, including Section 4.1(b), will apply with respect to such New In-License.

#### 3.3 Sublicensing Rights.

(a) *Transfer.* The licenses granted in Sections 3.1 are transferable only upon a permitted assignment of this License Agreement in accordance with Section 11.12.

(b) *Celgene Sublicenses.* The license granted in Section 3.1 may be sublicensed, in full or in part, by Celgene by a written agreement to its Affiliates and Third Parties (with the right to sublicense through multiple tiers), provided, that as a condition precedent to and requirement of any such sublicense:

(i) Celgene will provide Bluebird with a copy of any sublicense agreement with a non-Affiliated Sublicensee within thirty (30) days of execution thereof, and to the extent permitted under any Applicable Bluebird In-License, such sublicense agreement may be redacted as necessary to protect commercially sensitive information;

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(ii) Celgene will be responsible for any and all obligations of such Sublicensee as if such Sublicensee were “Celgene” hereunder; and

(iii) Any such Sublicensee will agree in writing to be bound by substantially identical obligations as Celgene hereunder with respect to the activities of such Sublicensee hereunder (and not with respect to the activities of any other), including Know-How disclosure obligations Celgene has to Bluebird hereunder with respect to the activities of such Sublicensee hereunder (but excluding payment obligations).

3.4 Exclusivity. During the License Agreement Term, neither Party nor its Affiliates (nor any others on behalf of or with, or under license or sublicense from, such Party or any its Affiliates) will research, Develop, Manufacture or Commercialize any products (including Vectors and associated Payloads) to be used in the Field (which, for the purposes of this Section 3.4, will include all indications and will not be limited to cancer) that specifically target the same Target Antigen as Elected Candidate, other than pursuant to this License Agreement (which includes, for avoidance of doubt, research, development, Manufacture and Commercialization of improved and modified versions of the Licensed Product by Celgene) or any other Development & Commercialization Agreement (if against the same Target Antigen) (which includes, for avoidance of doubt, research, development, Manufacture and Commercialization of improved and modified versions of the Licensed Product by Celgene). Notwithstanding this Section 3.4, if (i) a Business Combination occurs with respect to either Party with a Third Party or (ii) a Party acquires a Third Party (including by a merger or consolidation) so that such Third Party becomes an Affiliate over which the acquiring Party has control (as defined in the definition of Affiliate), or (iii) a Party acquires all or substantially all of the assets of a Third Party (including any Subsidiaries or divisions thereof) (each of (i), (ii) and (iii), a “Business Acquisition”; such Party, the “Business Party”), and, in each case, the Third Party (or any of such Third Party’s Affiliates or any successors or assigns of such Third Party or such Third Party’s Affiliates, other than the Business Party and its Affiliates as of the Business Acquisition) (a) already has, or the acquired assets contain, as applicable, a program that existed prior to, or was planned prior to and is demonstrably to be implemented shortly after, the Business Acquisition or (b) initiates and pursues a new program following such Business Acquisition, in each case that would otherwise violate this Section 3.4 (a “Business Program”), then such Third Party (or any of such Third Party’s Affiliates or any successors or assigns of such Third Party or such Third Party’s Affiliates, other than the Business Party and its Affiliates as of the Business Acquisition), as applicable, will be permitted to initiate, pursue and continue such Business Program after such Business Acquisition and such initiation, pursuit and continuation will not constitute a violation of this Section 3.4; provided however that (1) none of the Licensed IP, or other Patents, Materials or Know-How Controlled by the other Party and, in each case, licensed to the Business Party will be used in the Business Program, and (2) the research or Development activities required under this License Agreement will be conducted separately from any research or Development activities directed to such Business Program, including the maintenance of separate

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lab notebooks and records (password-protected to the extent kept on a computer network) and separate personnel working on each of the activities under this License Agreement and the activities covered under such Business Program.

3.5 Contract Manufacturers. Subject to the terms and conditions of this License Agreement, Celgene will have the right to appoint by a written agreement “contract manufacturers”, meaning any Third Party or Affiliate of Celgene that manufactures Licensed Product (or components therefor) for re-sale, but who itself is not a “Sublicensee” hereunder and thereby exercises “have made” rights granted by Bluebird under Section 3.1, as well as “contract research organizations” and other providers performing services on Celgene’s behalf, none of which will be deemed a “Sublicensee” hereunder. Celgene will be responsible for any such contract manufacturer, contract research organization or service provider hereunder, and further will require any such contract manufacturer, contract research organization or service provider to agree in writing to comply with Sections 3.6 and 8.

3.6 No Implied Rights. No license, sublicense or other right is or will be created or granted hereunder by implication, estoppel or otherwise. Any licenses, sublicenses or rights will be granted only as expressly provided in this License Agreement. Celgene will not practice or otherwise use any Licensed IP other than in accordance with the licenses granted in Section 3.1.

3.7 Section 365(n) of the Bankruptcy Code. All rights and licenses granted pursuant to any section of this License Agreement are, and will be deemed to be, rights and licenses to “intellectual property” (as defined in Section 101(35A) of title 11 of the United States Code and of any similar provisions of applicable Laws under any other jurisdiction (the “Bankruptcy Code”). Bluebird agrees that Celgene, as a licensee of rights and licenses under this License Agreement, will retain and may fully exercise all of its rights and elections under the Bankruptcy Code. The Parties further agree that, in the event of the commencement of a bankruptcy proceeding by or against Bluebird under the Bankruptcy Code or analogous provisions of applicable Law outside the United States, Celgene will be entitled to a complete duplicate of (or complete access to, as appropriate) any intellectual property licensed to Celgene and all embodiments of such intellectual property, which, if not already in Celgene’s possession, will be promptly delivered to it (i) upon any such commencement of a bankruptcy proceeding upon Celgene’s written request therefor, unless Bluebird elects to continue to perform all of its obligations under this License Agreement or (ii) if not delivered under clause (i), following the rejection of this License Agreement by Bluebird in the bankruptcy proceeding upon written request therefor by Celgene.

#### **4. Payments and Royalties.**

##### **4.1 Applicable Bluebird In-Licenses and Celgene Licensed Product In-Licenses .**

(a) *Applicable Pre-Existing In-Licenses*. If any In-License Payment becomes due under any Applicable Pre-Existing In-License during the License Agreement Term, Bluebird will pay same, provided that Celgene will reimburse Bluebird for any such In-License Payment within thirty (30) days of Celgene’s receipt of Bluebird’s written invoice therefor, which In-License Payment (other than payments that are royalties) will not exceed [\*\*\*], and subject to Section 6.1. Any such reimbursement by Celgene to Bluebird (1) is in addition to and not in lieu of the other payments required by this Section 4 and (2) will not be subject to Section 4.3(d). [\*\*\*]



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(b) *Applicable New In-Licenses*. Celgene may elect to take a sublicense under any New In-License of Bluebird and its Affiliates and upon such election, such New In-License will be an Applicable New In-License hereunder for all purposes. For the purposes of determining the Parties’ respective payment obligations, all Applicable New In-Licenses as of and following the License Agreement Effective Date will be listed on Appendix B. If any In-License Payment becomes due under any Applicable New In-License during the License Agreement Term, Bluebird will pay same and, subject to Section 6.1, Celgene will reimburse Bluebird for (i) [\*\*\*] of such payment that are royalties, which royalties will be subject to Section 4.3(d), and (ii) [\*\*\*] of such payment that are not royalties, in each case (i) and (ii) within thirty (30) days of receipt of Bluebird’s written invoice therefor. If Celgene elects to convert an Other In-License to an Applicable In-License pursuant to Section 3.2(b), Celgene will reimburse Bluebird for [\*\*\*] of any In-License Payments that became due under such Applicable New In-License during the License Agreement Term to the same extent as if such Applicable New In-License was designated as such as of the License Agreement Effective Date, including with respect to applicable Patent Costs in accordance with Section 6.1, provided that Bluebird provides Celgene with a reasonable accounting of same. If any In-License Payments are royalties due under any Applicable New In-License during the License Agreement Term, such royalties will be subject to Section 4.3(d). To the extent that any grant of a sublicense by Celgene or any Sublicensees under an Applicable New In-License triggers a payment obligation under such Applicable New In-License, Bluebird will pay same and Celgene will reimburse Bluebird for [\*\*\*] of such payment within thirty (30) days of receipt of Bluebird’s written invoice therefor.

(c) *Celgene Licensed Product In-Licenses*. If any payments become due under any Celgene Licensed Product In-License with respect to the Licensed Product, Bluebird will be responsible for [\*\*\*] of such payments as provided in Section 4.1(e) of the Master Collaboration Agreement, provided that if any such payments are royalties, such royalties will be subject to Section 4.3(d).

4.2 Milestone Payments. Celgene will make milestone payments (each, a “Milestone Payment”) to Bluebird upon the occurrence of each of the milestones events (each, a “Milestone Event”) as set forth below in this Section 4.2. Each of the Milestone Payments will be payable to Bluebird by Celgene within forty-five (45) days of the achievement of the specified Milestone Event, and such payments when owed or paid will be non-refundable and non-creditable, and not subject to set-off, except as otherwise set forth in Sections 2.8(a), 10.3(c) and 10.6 hereof, and Sections 4.1(e), 4.3 and 12.6 of the Master Collaboration Agreement. Except with respect to Modified Licensed Products, each of the Milestone Payments are payable only once in total under this License Agreement, whether achieved by one or more Licensed Products. Notwithstanding the foregoing, Bluebird will be entitled to receive [\*\*\*] of the Milestone Payments below, other than the Milestone Payment for the first Milestone Event ( *i.e.*, [\*\*\*]).

	<u>Milestone Event</u>	<u>Milestone Payment</u>
[***]		
[***]		

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4.3 Royalties.

(a) *Rates.* Subject to the remainder of this Section 4.3, Celgene will pay to Bluebird running royalties, on a Licensed Product-by-Licensed Product basis, based on the total aggregate annual Net Sales worldwide by Selling Parties of such Licensed Product in a given calendar year at the following royalty rates:

	<u>Annual Worldwide Net Sales of each Licensed Product</u>	<u>Royalty Rate</u>
[***]		

By way of example, in a given calendar year, if the aggregate annual worldwide Net Sales for a Licensed Product is [\*\*\*], the following royalty payment would be payable for those Net Sales under this Section 4.3(a): [\*\*\*]

(b) *Royalty Term.* Royalties under Section 4.3(a) will be payable, on a Licensed Product-by-Licensed Product and country-by-country basis, on the Net Sales of any Licensed Product if at least one of the following two (2) conditions apply: [\*\*\*]

(c) *Royalty Reduction.* If Licensed Product is royalty-bearing only on account of Section 4.3(b)(ii), then the royalty rates set forth in Section 4.3(a) with respect to Net Sales attributable to Licensed Product will be reduced by [\*\*\*].

(d) *Third Party Royalty Payments.* If Celgene or its Sublicensee, in its reasonable judgment, is required to obtain a license from any Third Party under any Patent Covering Licensed Product in order to Develop or Commercialize such Licensed Product, and if Celgene (or its Sublicensee) is required to pay to such Third Party under such license any royalties, and the infringement of such Patent cannot reasonably be avoided by Celgene (or its Sublicensee), or if Celgene (or its Sublicensee) is required by a court of competent jurisdiction to pay royalties or lost profits to such a Third Party (and the infringement of such Patent cannot reasonably be avoided), then the amount of Celgene’s royalty obligations under this Section 4.3 will be reduced by [\*\*\*] of the amount of such royalties paid to such Third Party, provided however, that the royalties payable under Section 4.3(a) will not be reduced in any such event below [\*\*\*] of the amounts set forth in Section 4.3(a) (but as may be further reduced pursuant to Section 4.3(c) or Section 4.3(e)) for each royalty tier. Any royalties payable under any Applicable Pre-Existing In-Licenses may not be deducted under this Section 4.3(d) from royalties owed to Bluebird. Any royalties payable under any Applicable New In-Licenses and Celgene Licensed Product In-Licenses may be deducted under this Section 4.3(d) from royalties owed to Bluebird. Celgene (or its Sublicensee) will use its commercially reasonable efforts to minimize the amount of any of the foregoing payments owed to Third Parties. Prior to Celgene or its Sublicensee exercising its reasonable judgment under this Section 4.3(d), Celgene will provide Bluebird with written notice of a potential need to obtain any license from Third Parties. The Parties will discuss the best course of action to resolve such potential license requirement(s).

(e) [\*\*\*]

(f) *Additional Royalty Provisions.* The royalties payable under Section 4.3(a) will be subject to the following:

(i) only one royalty will be payable hereunder with respect to each Licensed Product unit;

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(ii) royalties when owed or paid hereunder will, except as provided in Section 4.3(d), be non-refundable and non-creditable and not subject to set-off (except as otherwise provided in Sections 2.8(a), 10.3(c) and 10.6 hereof, [\*\*\*] and 17.6 of any Co-Development, Co-Promote and Profit Share Agreement, and Sections 4.1(e), 4.3 and 12.6 of the Master Collaboration Agreement); and

(iii) except as expressly set forth in Sections 4.3(c), 4.3(d) and 4.3(e), no other royalty deductions are permitted hereunder.

#### 4.4 Payment Terms. [\*\*\*]

(h) *Mutual Convenience of the Parties*. The royalty and other payment obligations set forth hereunder have been agreed to by the Parties for the purpose of reflecting and advancing their mutual convenience, including the ease of calculating and paying royalties and other amounts to Bluebird.

### 5. Ownership and Inventorship of IP.

5.1 Solely-Owned IP. Subject to Section 5.2, as between the Parties, each Party will own and retain all right, title and interest in and to any and all Know-How and Patents arising therefrom that are discovered, created, conceived, developed or reduced to practice solely by or on behalf of such Party under or in connection with this License Agreement, including as part of the Celgene Development & Commercialization Program (“Solely Owned IP”). Subject to the licenses hereunder and the other terms and conditions of this License Agreement, each Party will be solely responsible for the Prosecution and Maintenance, and the enforcement and defense, of any Patents within its Solely Owned IP, and the other Party will have no rights with respect thereto.

5.2 Joint IP. The Parties will jointly own any and all Know-How and Patents arising therefrom that are discovered, created, conceived, developed or reduced to practice jointly by or on behalf of the Parties, under or in connection with this License Agreement, including as part of the Celgene Development & Commercialization Program (“Joint IP”). Each Party will have an undivided one-half interest in and to Joint IP. Each Party will exercise its ownership rights in and to such Joint IP, including the right to license and sublicense or otherwise to exploit, transfer or encumber its ownership interest, without an accounting or obligation to, or consent required from, the other Party, but subject to the licenses hereunder and the other terms and conditions of this License Agreement, including Section 3.4. At the reasonable written request of a Party, the other Party will in writing grant such consents and confirm that no such accounting is required to effect the foregoing regarding Joint IP. Each Party, for itself and on behalf of its Affiliates, licensees and sublicenses, and employees, subcontractors, consultants and agents of any of the foregoing, hereby assigns (and to the extent such assignment can only be made in the future hereby agrees to assign), to the other Party a joint and undivided interest in and to all Joint IP. The Prosecution and Maintenance, and the enforcement and defense, of any Patents within Joint IP will be jointly managed by the Parties on mutually agreeable terms to be entered into by the Parties at the time any such Patents are first filed, provided that (i) all recoveries and Patent

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Costs arising from the enforcement or defense of any Patents within Joint IP, absent further agreement, will be shared by the Parties in accordance with Section 7.2(e) (provided that sufficient advance written notice of any such Patent Costs is given to the Party not incurring same) and (ii) Patent Costs incurred in connection with the Prosecution and Maintenance of Patents within Joint IP will be apportioned as set forth in Sections 6.1 and 6.3, provided that in each case ((i) and (ii)), if either Party elects not to pay any such Patent Costs for any such Patent, the Parties will meet and agree upon an equitable way to treat such Patent.

5.3 Inventorship. Inventorship determination for all Patents worldwide arising from any Know-How created, conceived or developed by or on behalf of the Parties under or in connection with this License Agreement and thus the ownership thereof will be made in accordance with applicable United States patent Laws.

5.4 Allocation. Notwithstanding Sections 5.1 – 5.3, the Patent Committee may allocate ownership of a particular item of intellectual property to improve the prospects of obtaining patent protection with respect to such item of intellectual property, even if such allocation is not in accordance with the terms of Sections 5.1 – 5.3, so long as the Parties mutually agree to such allocation.

## **6. Patent Prosecution and Maintenance.**

6.1 Generally. Subject to Sections 6.2 and 6.3, Bluebird will have the sole right to Prosecute and Maintain Patents within the Licensed IP . Bluebird will use commercially reasonable efforts to, where applicable and upon Celgene’s reasonable request, separate parent Patent applications within the Licensed IP into one or more separate Patent applications for Specific Patents, to the extent permitted under applicable Law, where doing so would not reasonably be expected to materially harm any Patent within the Licensed IP or other Patents owned by Bluebird or its Affiliates, provided that the foregoing limitation will not apply to Licensed IP that is Collaboration IP. [\*\*\*]

6.2 Celgene Input. Bluebird will regularly provide Celgene with copies of all applications for Patents within the Licensed IP, and all other material submissions and correspondence with any patent authorities regarding such Patents, in sufficient time to allow for review and comment by Celgene. In addition, Bluebird will provide Celgene and its counsel with an opportunity to consult with Bluebird and its counsel regarding Prosecution and Maintenance of any such Patents in the Field, and Bluebird will consider in good faith all comments timely made by Celgene and its counsel. In the event of any disagreement between any of Bluebird or Celgene, Bluebird will have the final decision-making authority with respect to the matter involved as long as Bluebird acts in good faith.

6.3 Specific Patents. For any Patent within the Licensed IP [\*\*\*] (each “Specific Patent”), the following will apply: upon Celgene’s written request, and provided that Bluebird reasonably agrees with Celgene that the following Prosecution and Maintenance activities would not materially harm any other Patent within the Licensed IP or other Patents owned by Bluebird or its Affiliates (other than Collaboration IP), Celgene will control the Prosecution and Maintenance of the Specific Patents, and notwithstanding anything in Section 6.1 to the contrary, Celgene will be solely responsible for the payment of all related Patent Costs. In addition, Celgene will provide Bluebird and its counsel with an opportunity to consult with Celgene and

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its counsel regarding Prosecution and Maintenance of any such Specific Patents, and Celgene will include or reflect all reasonable comments timely made by Bluebird and its counsel. Celgene acknowledges and agrees that Bluebird may grant similar rights to other exclusive Third Party licensees under any Patent within the Licensed IP that has claims Covering only a product that is not a Licensed Product (or its manufacture or use) and no other product (or its manufacture or use), other than Specific Patents. If the Parties cannot agree whether or not any Patent within the Licensed IP is a Specific Patent, or if Bluebird claims that the foregoing Prosecution and Maintenance activities would materially harm any other Patent within the Licensed IP or other Patents owned by Bluebird or any of its Affiliates, either of the Parties may refer such dispute to a mutually agreeable, disinterested, conflict-of-interest-free individual not affiliated or consulting with either Party and who has at least fifteen (15) years of patent prosecution experience in the pharmaceutical field. Any such arbitration will be conducted under the then-current rules of the American Arbitration Association, and the decision of the arbitrator will be final.

6.4 Election Not to Prosecute or Maintain or Pay Patent Costs. If Bluebird elects not (i) to Prosecute or Maintain any Patents within the Licensed IP in any particular country before the applicable filing deadline or continue such activities once filed in a particular country, or (ii) to pay the Patent Costs associated with Prosecution or Maintenance of any Patents within the Licensed IP, then in each such case Bluebird will so notify Celgene, promptly in writing and in good time to enable Bluebird to meet any deadlines by which an action must be taken to preserve such Patent in such country, if Celgene so requests. Upon receipt of each such notice by Bluebird, Celgene will have the right, but not the obligation, to notify Bluebird in writing on a timely basis that Celgene will assume control of the Prosecution or Maintenance of such Patent, and bear the Patent Costs thereafter incurred by Celgene with respect thereto. In addition, Celgene will provide Bluebird and its counsel with an opportunity to consult with Celgene and its counsel regarding Prosecution and Maintenance of any such Patents, and Celgene will include or reflect all reasonable comments timely made by Bluebird and its counsel. If after making such election, Celgene elects not to pay the Patent Costs associated with Prosecution or Maintenance of any such Patent, then in each such case Celgene will so notify Bluebird and on the ninetieth (90<sup>th</sup>) day after Bluebird’s receipt of such notice such Patent will no longer be licensed to Celgene hereunder and will no longer be included within the “Licensed IP” hereunder.

6.5 Third Party Rights. To the extent that a Third Party licensor of Bluebird has retained any right to Prosecute or Maintain any Patent within the Licensed IP licensed to Celgene hereunder (including pursuant to an Applicable Bluebird In-License), or otherwise be involved in such activities, Bluebird will use commercially reasonable efforts to cause such Third Party licensor to take the actions specified by this Section 6 (including Sections 6.6 and 6.7) in a manner consistent with the in-license applicable thereto, but Bluebird will not be deemed to be in breach of its obligations under this Section 6 if, after using such commercially reasonable efforts, it is unable to comply with such obligations because of actions taken or not taken by such Third Party licensor.

6.6 Patent Extensions. Subject to the remainder of this Section 6.6, if any election for patent term restoration or extension, supplemental protection certificate or any of their

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equivalents may be made with respect to any Patent within the Licensed IP, after consultation with Celgene, the Parties will discuss and seek to reach mutual agreement whether or not to take such action. If the Parties are not able to reach mutual agreement, (i) Celgene will have the sole right to make the final decision whether or not to seek such patent term restoration or extension, supplemental protection certificate or any of their equivalents with respect to Specific Patents and Patents within the Collaboration IP licensed to Celgene hereunder and (ii) Bluebird will have the sole right to make the final decision whether or not to seek such patent term restoration or extension, supplemental protection certificate or any of their equivalents with respect to all other Patents within the Licensed IP.

6.7 Regulatory Exclusivity Periods. With respect to any Patent listings required for any Regulatory Exclusivity Periods for Product, the Parties will mutually agree on which Patents within the Licensed IP to list, provided that if the Parties are not able to agree, Celgene will have the right to make the final decision, and provided further that the exercise of such right by Celgene will not increase or otherwise change the rights or obligations of the Parties hereunder.

6.8 Cooperation. Each Party will reasonably cooperate with the other Party in the Prosecution and Maintenance of Patents within the Licensed IP. Such cooperation includes promptly executing all documents, or requiring inventors, subcontractors, employees and consultants and agents of Celgene and Bluebird and their respective Affiliates and Sublicensees to execute all documents, as reasonable and appropriate so as to enable the Prosecution and Maintenance of any such Patents in any country.

6.9 Patent Marking. Celgene will mark, and will cause all other Selling Parties to mark, Product with all Patents within the Licensed IP in accordance with applicable Law, which marking obligation will continue for as long as (and only for as long as) required under applicable Law.

6.10 Common Interest Disclosures. With regard to any information or opinions disclosed pursuant to this License Agreement by one Party to the other Party regarding Prosecution and Maintenance of Patent within the Licensed IP, or enforcement of intellectual property and/or technology by or against Third Parties, Bluebird and Celgene agree that they have a common legal interest in determining the ownership, scope, validity and/or enforcement of the Licensed IP, and whether, and to what extent, Third Party intellectual property rights may affect the conduct of the Development and Commercialization of any Licensed Product, and have a further common legal interest in defending against any actual or prospective Third Party claims based on allegations of misuse or infringement of intellectual property rights relating to the Development or Commercialization of any Licensed Product. Accordingly, the Parties agree that all such information and materials obtained by the Parties from each other will be used solely for purposes of the Parties' common legal interests with respect to the conduct of the Agreement. All such information and materials will be treated as protected by the attorney-client privilege, the work product privilege, and any other privilege or immunity that may otherwise be applicable. By sharing any such information and materials, neither Party intends to waive or limit any privilege or immunity that may apply to the shared information and materials. Neither Party will have the authority to waive any privilege or immunity on behalf of the other Party without such other Party's prior written consent, nor will the waiver of privilege or immunity resulting from the conduct of one Party be deemed to apply against any other Party. This

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Section 6.10 will be subject to any right granted by either Party to any Third Party, provided that the grant of such right to such Third Party does not conflict with the other Party’s rights or the first Party’s obligations under this License Agreement.

**7. Patent Enforcement and Defense.**

7.1 Notice. Each Party will promptly notify, in writing, the other Party upon learning of any actual or suspected Competitive Infringement of any Patents within the Licensed IP by a Third Party, or of any claim of invalidity, unenforceability, or non-infringement of any Patents within the Licensed IP, and will, along with such notice, supply the other Party with any evidence in its possession pertaining thereto. For purposes of this License Agreement, “Competitive Infringement” means any allegedly infringing activity in the Field (which, for the purposes of this definition, will include all indications and will not be limited to cancer) with respect to a Patent within the Licensed IP, which activity (i) falls within the scope then in effect of the licenses granted by Bluebird to Celgene as set forth in Sections 3.1, (ii) is subject to Section 7.2(f), or (iii) would be competitive with a Licensed Product and targets the same Target Antigen as such Licensed Product.

7.2 Enforcement and Defense. [\*\*\*]

7.3 Third Party Rights. To the extent that a Third Party licensor of Bluebird has retained any right to (i) defend against a declaratory judgment action or other action challenging any Patents within the Licensed IP, (ii) seek to abate any Competitive Infringement of the Patents within the Licensed IP by a Third Party, or (iii) take any other actions described in Section 7.2 for any Patent within the Licensed IP licensed to Celgene hereunder (including pursuant to an Applicable Bluebird In-License), or otherwise be involved in such activities, Bluebird will use commercially reasonable efforts to cause such Third Party licensor to take the actions specified by this Section 7.3 in a manner consistent with the in-license applicable thereto, but Bluebird will not be deemed to be in breach of its obligations under this Section 7.3 if, after using such commercially reasonable efforts, it is unable to comply with such obligations because of actions taken or not taken by such Third Party licensor.

**8. Confidentiality.**

The Parties acknowledge and agree that terms of this License Agreement and all Materials, ideas and information of any kind, whether in written, oral, graphical, machine-readable or other form, whether or not marked as confidential or proprietary, which are transferred, disclosed or made available by a Party or at the request of a Party, including any of the foregoing of Third Parties, will be subject to the provisions of Section 10 of the Master Collaboration Agreement. The Parties agree to issue the joint press release on Appendix E promptly following the License Agreement Effective Date. A redacted version of this License Agreement is attached hereto as Appendix I.

**9. Warranties; Limitations of Liability; Indemnification.**

9.1 Representations and Warranties. Each Party represents and warrants to the other as of the License Agreement Effective Date that it has the legal right and power to enter into this License Agreement, to extend the rights and licenses granted or to be granted to the other in this License Agreement, and to fully perform its obligations hereunder.

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9.2 Additional Representations and Warranties of Bluebird. Except as set forth in Schedule 9.2, Bluebird represents and warrants to Celgene that, as of the License Agreement Effective Date:

(a) *Licensed IP*. Appendix F sets forth a complete and accurate list of all Patents included in the Licensed IP, indicating the owner, licensor and/or co-owner(s), if applicable, and, for any Elected Candidate and Licensed Product-relevant subject matter or Materials, if no Patent is specifically licensed, a list of all subject matter or Materials that are included in the Licensed IP, including those licensed under a materials use license or equivalent. Bluebird Controls the Patents listed on Appendix F and the Know-How within the Licensed IP, and is entitled to grant the licenses specified herein. Bluebird has not granted to any Third Party any rights or licenses under such Patents or Know-How within the Licensed IP that would conflict with the licenses granted to Celgene hereunder.

(b) *Third Party Agreements*. The Applicable Bluebird In-Licenses are valid and binding obligations of Bluebird and, to the Knowledge of Bluebird, the applicable licensor, enforceable against Bluebird and, to the Knowledge of Bluebird, the applicable licensor, in accordance with their terms, except as may be limited by general principles of equity (regardless of whether considered in a proceeding at law or in equity) and by applicable bankruptcy, insolvency, moratorium and other similar Laws of general application relating to or affecting creditors' rights generally. Neither Bluebird nor any of its Affiliates has received any notice of any counterparty's intention to terminate any Applicable Bluebird In-License in whole or in part or any notice requesting any amendment, alteration or modification of such Applicable Bluebird In-License or any sublicense or assignment thereunder. There is no breach or default, or event which upon notice or the passage of time, or both, could give rise to any breach or default, in the performance of any Applicable Bluebird In-License by Bluebird or any of its Affiliates or, to the Knowledge of Bluebird, the counterparty thereto, and Bluebird has not received any notice of any such breach, default or event. Except for the Applicable Bluebird In-Licenses, neither Bluebird nor any of its Affiliates is a party to any license, sublicense or other agreement pursuant to which Bluebird or such Affiliate has received a license or other rights relating to the Elected Candidate or Licensed Product. All Patents and Know-How licensed to Bluebird under the Applicable Bluebird In-Licenses are Controlled by Bluebird for purposes of the licenses granted to Celgene under this License Agreement.

(c) *Patents*. To Bluebird's Knowledge, the Patents listed on Appendix F have been procured or are being procured from the respective patent offices in accordance with applicable Law. None of the Patents included in the Licensed IP is or has been involved in any opposition, cancellation, interference, reissue or reexamination proceeding, and no Licensed IP is the subject of any judicial, administrative or arbitral order, award, decree, injunction, lawsuit, proceeding or stipulation. Neither Bluebird nor any of its Affiliates has received any notice alleging that the Patents in the Licensed IP are invalid or unenforceable, or challenging Bluebird's ownership of or right to use any such rights.

(d) *No Conflicts*. The execution, delivery and performance by Bluebird of this License Agreement and the consummation of the transactions contemplated hereby will not result in any violation of, conflict with, result in a breach of or constitute a default under any understanding, contract or agreement to which Bluebird is a party or by which it is bound.



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Neither Bluebird nor any of its Affiliates has entered into any agreement or otherwise licensed, granted, assigned, transferred, conveyed or otherwise encumbered or disposed of any right, title or interest in or to any of its assets, including any intellectual property rights, that would in any way conflict with or impair the scope of any rights or licenses granted to Celgene hereunder.

(e) *Outlicenses*. Appendix G sets forth a complete and accurate list of all agreements relating to the licensing, sublicensing or other granting of rights by Bluebird to any Person with respect to the Licensed IP and the Field, and Bluebird has provided complete and accurate copies of all such agreements to Celgene. Except for the Applicable Bluebird In-Licenses, Bluebird and its Affiliates are not subject to any payment obligations to Third Parties as a result of the execution or performance of this License Agreement. Neither Bluebird nor any of its Affiliates has granted any liens or security interests on the Licensed IP and the Licensed IP is free and clear of any mortgage, pledge, claim, security interest, covenant, easement, encumbrance, lien or charge of any kind.

(f) *No Proceedings*. There is no action, suit, proceeding or investigation pending or, to the Knowledge of Bluebird, currently threatened in writing against or affecting Bluebird that questions the validity of this License Agreement or the right of Bluebird to enter into this License Agreement or consummate the transactions contemplated hereby.

(g) *No Infringement*. Neither Bluebird nor any of its Affiliates has received any notice of any claim that any Patent, Know-How or other intellectual property Controlled by a Third Party would be infringed or misappropriated by the production, use, research, Development, Manufacture or Commercialization of the Elected Candidate or Licensed Product pursuant to this License Agreement, and, to the Knowledge of Bluebird, there are no Patents, Know-How or other intellectual property owned by a Third Party and not included in the Licensed IP or In-Licensed IP that are necessary for the production, use, research, Development, Manufacture or Commercialization of Elected Candidate or Licensed Product.

9.3 Disclaimers. Without limiting the respective rights and obligations of the Parties expressly set forth herein, each Party specifically disclaims any guarantee that any Licensed Product will be successful, in whole or in part. EXCEPT AS OTHERWISE EXPRESSLY PROVIDED IN THIS LICENSE AGREEMENT, THE PARTIES MAKE NO REPRESENTATIONS AND EXTEND NO WARRANTY OF ANY KIND, EITHER EXPRESS OR IMPLIED, WITH RESPECT TO ANY PATENTS, KNOW-HOW, ELECTED CANDIDATE OR LICENSED PRODUCT, INCLUDING WARRANTIES OF VALIDITY OR ENFORCEABILITY OF ANY PATENT RIGHTS, TITLE, QUALITY, MERCHANTABILITY, FITNESS FOR A PARTICULAR USE OR PURPOSE, PERFORMANCE, AND NONINFRINGEMENT OF ANY THIRD PARTY PATENTS OR OTHER INTELLECTUAL PROPERTY RIGHTS.

9.4 [\*\*\*]

9.5 Performance by Others. The Parties recognize that each Party may perform some or all of its obligations under this License Agreement through Affiliates and permitted subcontractors provided, however, that each Party will remain responsible and liable for the performance by its Affiliates and permitted subcontractors and will cause its Affiliates and permitted subcontractors to comply with the provisions of this License Agreement in connection therewith.

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9.6 Indemnification.

(a) *Indemnification by Celgene*. Celgene will indemnify Bluebird, its Affiliates and their respective directors, officers, employees, Third Party licensors and agents, and their respective successors, heirs and assigns (collectively, “Bluebird Indemnitees”), and defend and save each of them harmless, from and against any and all losses, damages, liabilities, costs and expenses (including reasonable attorneys’ fees and expenses) (collectively, “Losses”) in connection with any and all suits, investigations, claims or demands of Third Parties (collectively, “Third Party Claims”) against the Bluebird Indemnitees arising from or occurring as a result of: (i) the material breach by Celgene of any term of this License Agreement; (ii) any gross negligence or willful misconduct on the part of Celgene in performing its obligations under this License Agreement; or (iii) the Development or Commercialization by or on behalf of Celgene or any of its Affiliates or Sublicensees of Elected Candidate or Licensed Product, except in each case for those Losses for which Bluebird has an obligation to indemnify Celgene pursuant to Section 9.6(b), as to which Losses each Party will indemnify the other to the extent of their respective liability; provided, however, that Celgene will not be obligated to indemnify Bluebird Indemnitees for any Losses to the extent that such Losses arise as a result of gross negligence or willful misconduct on the part of an Bluebird Indemnitee.

(b) *Indemnification by Bluebird*. Bluebird will indemnify Celgene, its Affiliates and their respective directors, officers, employees and agents, and their respective successors, heirs and assigns (collectively, “Celgene Indemnitees”), and defend and save each of them harmless, from and against any and all Losses in connection with any and all Third Party Claims against Celgene Indemnitees arising from or occurring as a result of: (i) the material breach by Bluebird of any term of this License Agreement; (ii) any gross negligence or willful misconduct on the part of Bluebird in performing its obligations under this License Agreement; or (iii) the Development by or on behalf of Bluebird or any of its Affiliates or Sublicensees of Elected Candidate or Licensed Product, except in each case for those Losses for which Celgene has an obligation to indemnify Bluebird pursuant to Section 9.6(a), as to which Losses each Party will indemnify the other to the extent of their respective liability for the Losses; provided, however, that Bluebird will not be obligated to indemnify Celgene Indemnitees for any Losses to the extent that such Losses arise as a result of gross negligence or willful misconduct on the part of a Celgene Indemnitee.

(c) *Notice of Claim*. All indemnification claims provided for in Sections 9.6(a) and 9.6(b) will be made solely by such Party to this License Agreement (the “Indemnified Party”). The Indemnified Party will promptly notify the indemnifying Party (an “Indemnification Claim Notice”) of any Losses or the discovery of any fact upon which the Indemnified Party intends to base a request for indemnification under Section 9.6(a) and 9.6(b), but in no event will the indemnifying Party be liable for any Losses that result from any delay in providing such notice. Each Indemnification Claim Notice must contain a description of the claim and the nature and estimated amount of such Loss (to the extent that the nature and amount of such Loss is known at such time). The Indemnified Party will furnish promptly to the indemnifying Party copies of all papers and official documents received in respect of any Losses and Third Party Claims.

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(d) *Defense, Settlement, Cooperation and Expenses* .

(i) *Control of Defense*. At its option, the indemnifying Party may assume the defense of any Third Party Claim by giving written notice to the Indemnified Party within thirty (30) days after the indemnifying Party’s receipt of an Indemnification Claim Notice, provided however that (i) the Third Party Claim solely seeks monetary damages and (ii) the indemnifying Party expressly agrees in writing that as between the indemnifying Party and the Indemnified Party, the indemnifying Party will be solely obligated to satisfy and discharge the Third Party Claim in full and is able to reasonably demonstrate that it has sufficient financial resources (the matters described in (i) and (ii), the “Litigation Conditions”). The assumption of the defense of a Third Party Claim by the indemnifying Party will not be construed as an acknowledgment that the indemnifying Party is liable to indemnify the Indemnified Party in respect of the Third Party Claim, nor will it constitute a waiver by the indemnifying Party of any defenses it may assert against the Indemnified Party’s claim for indemnification. Upon assuming the defense of a Third Party Claim, the indemnifying Party may appoint as lead counsel in the defense of the Third Party Claim any legal counsel selected by the indemnifying Party (the indemnifying Party will consult with the Indemnified Party with respect to a possible conflict of interest of such counsel retained by the indemnifying Party). In the event the indemnifying Party assumes the defense of a Third Party Claim, the Indemnified Party will immediately deliver to the indemnifying Party all original notices and documents (including court papers) received by the Indemnified Party in connection with the Third Party Claim. Should the indemnifying Party assume the defense of a Third Party Claim, except as provided in Section 9.6(d)(ii), the indemnifying Party will not be liable to the Indemnified Party for any legal costs or expenses subsequently incurred by such Indemnified Party in connection with the analysis, defense or settlement of the Third Party Claim. The Indemnified Party may, at any time, assume the defense of a Third Party Claim if at any time the Litigation Conditions are not satisfied with respect to such Claim. In the event that it is ultimately determined that the indemnifying Party is not obligated to indemnify, defend or hold harmless the Indemnified Party from and against the Third Party Claim, the Indemnified Party will reimburse the indemnifying Party for any and all costs and expenses (including attorneys’ fees and costs of suit) and any Third Party Claims incurred by the indemnifying Party in its defense of the Third Party Claim.

(ii) *Right to Participate in Defense*. Without limiting Section 9.6(d)(i), any Indemnified Party will be entitled to participate in, but not control, the defense of such Third Party Claim and to employ counsel of its choice for such purpose; provided, however, that such employment will be at the Indemnified Party’s own cost and expense unless (i) the employment thereof has been specifically authorized by the indemnifying Party in writing, (ii) the indemnifying Party has failed to assume the defense and employ counsel in accordance with Section 9.6(d)(i) (in which case the Indemnified Party will control the defense), (iii) the interests of the Indemnified Party and the indemnifying Party with respect to such Third Party Claim are sufficiently adverse to prohibit the representation by the same counsel of both Parties under applicable Law, ethical rules or equitable principles, or (iv) the indemnifying Party no longer satisfies the Litigation Conditions, in which case the indemnifying Party will assume [\*\*\*]percent ([\*\*\*]%) of any such costs and expenses of counsel for the Indemnified Party.

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(iii) *Settlement*. With respect to any Third Party Claims that relate solely to the payment of money damages in connection with a Third Party Claim and that will not result in the Indemnified Party’s becoming subject to injunctive or other relief or otherwise adversely affecting the business of the Indemnified Party in any manner, and as to which the indemnifying Party will have acknowledged in writing the obligation to indemnify the Indemnified Party hereunder, and subject to the Litigation Conditions being satisfied, the indemnifying Party will have the sole right to agree to the entry of any judgment, enter into any settlement or otherwise dispose of such Loss, on such terms as the indemnifying Party, in its sole discretion, will deem appropriate. With respect to all other Losses in connection with Third Party Claims, where the indemnifying Party has assumed the defense of the Third Party Claim in accordance with Section 9.6(d)(i), the indemnifying Party will have authority to agree to the entry of any judgment, enter into any settlement or otherwise dispose of such Loss provided it obtains the prior written consent of the Indemnified Party (such consent not to be unreasonably withheld, delayed or conditioned). The indemnifying Party will not be liable for any settlement or other disposition of a Loss by an Indemnified Party that is reached without the prior written consent of the indemnifying Party. Regardless of whether the indemnifying Party chooses to defend or prosecute any Third Party Claim, no Indemnified Party will admit any liability with respect to or settle, compromise or discharge, any Third Party Claim without the prior written consent of the indemnifying Party, such consent not to be unreasonably withheld, delayed or conditioned.

(iv) *Cooperation*. If the indemnifying Party chooses to defend or prosecute any Third Party Claim, the Indemnified Party will, and will cause each other Indemnified Party to, cooperate in the defense or prosecution thereof and will furnish such records, information and testimony, provide such witnesses and attend such conferences, discovery proceedings, hearings, trials and appeals as may be reasonably requested in connection therewith. Such cooperation will include access during normal business hours afforded to indemnifying Party to, and reasonable retention by the Indemnified Party of, records and information that are reasonably relevant to such Third Party Claim, and making Indemnified Parties and other employees and agents available on a mutually convenient basis to provide additional information and explanation of any material provided hereunder, and the indemnifying Party will reimburse the Indemnified Party for all its reasonable out-of-pocket costs and expenses in connection therewith.

(v) *Costs and Expenses*. Except as provided above in this Section 9.6(d), the costs and expenses, including attorneys’ fees and expenses, incurred by the Indemnified Party in connection with any claim will be reimbursed on a calendar quarter basis by the indemnifying Party, without prejudice to the indemnifying Party’s right to contest the Indemnified Party’s right to indemnification and subject to refund in the event the indemnifying Party is ultimately held not to be obligated to indemnify the Indemnified Party.

9.7 Insurance. Each Party will maintain at its sole cost and expense, an adequate liability insurance or self-insurance program (including product liability insurance) to protect against potential liabilities and risk arising out of activities to be performed under this License Agreement, and any agreement related hereto and upon such terms (including coverages, deductible limits and self-insured retentions) as are customary in the U.S. pharmaceutical industry for the activities to be conducted by such Party under this License Agreement. Subject to the preceding sentence, such liability insurance or self-insurance program will insure against

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all types of liability, including personal injury, physical injury or property damage arising out of the manufacture, sale, use, distribution or marketing of Licensed Product. The coverage limits set forth herein will not create any limitation on a Party’s liability to the other under this License Agreement.

**10. Term and Termination**

10.1 Term. This License Agreement will commence as of the License Agreement Effective Date and, unless sooner terminated in accordance with the terms hereof or by mutual written consent, will continue on a country-by-country basis, until there are no more payments owed Bluebird on Licensed Product in such country (the longest such period of time for any Licensed Product hereunder, the “License Agreement Term”). Upon there being no more such payments hereunder for any such Licensed Product in such country, the licenses contained in Section 3.1 for such Licensed Product will become fully paid up and will remain exclusive with respect to such Licensed Product in such country.

10.2 Termination by Bluebird.

(a) *Breach*. Bluebird will have the right to terminate this License Agreement in full upon delivery of written notice to Celgene in the event of any material breach by Celgene of any terms and conditions of this License Agreement in a manner that fundamentally frustrates the transactions contemplated by this License Agreement, provided that such termination will not be effective if such breach, has been cured within [\*\*\*] after written notice thereof is given by Bluebird to Celgene specifying the nature of the alleged breach (or, if such default cannot be cured within such [\*\*\*] after such notice if Celgene commences actions to cure such default within such [\*\*\*] and thereafter diligently continues such actions, but fails to cure the default by the end of such [\*\*\*]); provided, however, that to the extent such material breach involves the failure to make a payment when due, such breach must be cured within [\*\*\*] after written notice thereof is given by Bluebird to Celgene.

(b) [\*\*\*]

10.3 Termination by Celgene.

(a) *Breach*. Celgene will have the right to terminate this License Agreement in full upon delivery of written notice to Bluebird in the event of any material breach by Bluebird of any terms and conditions of this License Agreement in a manner that fundamentally frustrates the transactions contemplated by this License Agreement, provided that such termination will not be effective if such breach has been cured within [\*\*\*] after written notice thereof is given by Celgene to Bluebird specifying the nature of the alleged breach (or, if such default cannot be cured within such [\*\*\*] period, within [\*\*\*] days after such notice if Bluebird commences actions to cure such default within such [\*\*\*] period and thereafter diligently continues such actions, but fails to cure the default by the end of such [\*\*\*]).

(b) *Discretionary Termination*. Beginning with [\*\*\*], Celgene will have the right to terminate this License Agreement in full at its discretion for any reason by delivering written notice to Bluebird, such termination to be effective [\*\*\*] following the date of such notice.

(c) *Alternative to Termination Under Section 10.3(a)*. If Celgene has the right to terminate this License Agreement under Section 10.3(a) (including expiration of all applicable

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cure periods thereunder), in lieu of exercising such termination right, Celgene may elect once by written notice to Bluebird before the end of such applicable cure period to have this License Agreement continue in full force and effect and instead have, starting immediately after the end of such applicable cure period, any future Milestone Payments set forth in Section 4.2 and the royalty rates set forth in the table set forth in Section 4.3(a) be reduced by [\*\*\*], provided that such reduction will not apply if such future Milestone Payments and royalty rates have already been reduced pursuant to Section 11.4(c) of the Master Collaboration Agreement.

10.4 Effects of Termination. Upon termination (but not expiration pursuant to Section 10.1) of this License Agreement for any reason:

(a) *Wind Down*. Celgene will responsibly wind-down, in accordance with accepted pharmaceutical industry norms and ethical practices, any on-going clinical studies for which it has responsibility hereunder in which patient dosing has commenced or, if reasonably practicable and requested by Bluebird, allow Celgene, its Affiliates or its Sublicensees to complete such trials. Celgene will be responsible for any costs associated with such wind-down. Bluebird will pay all costs incurred by either Party to complete such studies should Bluebird request that such studies be completed.

(b) *Sublicenses*. A termination of this License Agreement will not automatically terminate any sublicense granted by Celgene pursuant to Section 3.3 for Commercialization rights with respect to a non-Affiliated Sublicensee, provided that (i) such Sublicensee is not then (a) in material breach of any provision of this License Agreement or (b) in material breach of the applicable sublicense agreement or otherwise in breach of such sublicense agreement in a manner that would give rise to a right of termination on the part of Celgene, (ii) if Bluebird terminates this License Agreement pursuant to Section 10.2(a)(iii) for Celgene’s failure to fulfill its payment obligations hereunder, such Sublicensee agrees to and does pay to Bluebird all outstanding amounts that accrued as a result of such Sublicensee’s activities under the sublicense, (iii) Bluebird will have the right to step into the role of Celgene as sublicensor under any such sublicense executed after the License Agreement Effective Date, with all the rights that Celgene had under such sublicense, solely with respect to the Licensed IP, prior to termination of this License Agreement (including the right to receive any payments to Celgene by such Sublicensee that accrue from and after the date of the termination of this License Agreement solely with respect to the Licensed IP), (iv) such Sublicensee will pay to Bluebird all amounts that Celgene would have been obligated to pay to Bluebird hereunder with respect to such Sublicensee’s activities had this License Agreement not terminated (less any amounts received by Bluebird in clause (iii) above) and (v) the survival of such sublicense will not result in an imposition of any additional obligations on the part of Bluebird that are not included within the scope of this License Agreement. Celgene will include in any sublicense agreement executed after the License Agreement Effective Date that relates solely to the Licensed IP a provision in which said Sublicensee acknowledges its obligations to Bluebird under this Section 10.4(b).

(c) *Cessation of Rights*. Except as otherwise expressly provided in Sections 10.4(b), all rights and licenses granted by Bluebird to Celgene in Section 3.1 will terminate, and Celgene and its Affiliates and Sublicensees will cease all use of Licensed IP and all Development, Manufacture and Commercialization of Elected Candidate and Licensed Product.

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(d) *Regulatory Approvals*. To the extent permitted by applicable Law, and subject to Bluebird paying commercially reasonable compensation to Celgene for the assets to be transferred pursuant to this Section 10.4(d) (such compensation to either be mutually agreed to or determined through arbitration as provided in Section 10.4(g) below, and such compensation to be reduced by [\*\*\*] from what would be commercially reasonable compensation if this License Agreement is terminated by Bluebird pursuant to Section 10.2(a)), all Regulatory Approvals and other regulatory filings and communications owned (in whole or in part) or otherwise Controlled by Celgene and its Affiliates and Sublicensees solely relating to the Elected Candidate and/or Licensed Product, and all other documents solely relating to and necessary to further Develop and Commercialize Elected Candidate and Licensed Product, as such items exist as of the effective date of such termination (including all solely related completed and ongoing clinical studies) will be assigned to Bluebird, and Celgene will provide to Bluebird one (1) copy of the foregoing and all documents contained in or referenced in any such items, together with the raw and summarized data for any clinical studies (and where reasonably available, electronic copies thereof). In the event of failure to obtain assignment, subject to the Parties agreeing on commercially reasonable compensation for the right to access and reference, Celgene hereby consents and grants to Bluebird the right to access and reference (without any further action required on the part of Celgene, whose authorization to file this consent with any Regulatory Authority is hereby granted) any such item.

(e) *Licenses*. Subject to Bluebird paying (i) commercially reasonable compensation to Celgene for the licenses to be granted pursuant to subsection (1) of this Section 10.4(e) (such compensation to either be mutually agreed to or determined through arbitration as provided in Section 10.4(g) below, and such compensation to be reduced by [\*\*\*] from what would be commercially reasonable compensation if this License Agreement is terminated by Bluebird pursuant to Section 10.2(a)), and (ii) amounts payable to Celgene’s applicable licensors as set forth below, Celgene will grant to Bluebird and its Affiliates (1) a worldwide, perpetual and irrevocable, nontransferable (except in connection with a permitted assignment of this License Agreement in accordance with Section 11.12), exclusive license, with the right to grant sublicenses through multiple tiers (subject to Section 3.3(b), *mutatis mutandis*), under the Celgene Licensed Product IP, and (2) an exclusive sublicense under the Celgene Licensed Product In-Licensed IP, in each case ((1) and (2)) to the extent such Celgene Licensed Product IP and Celgene Licensed Product In-Licensed IP are used in or Cover the Licensed Product as of the effective date of termination and to the extent such Celgene Licensed Product IP and Celgene Licensed Product In-Licensed IP exist as of the effective date of such termination (including in each case any additions, divisions, continuations, continuations-in-part, invention certificates, substitutions, reissues, reexaminations, extensions, registrations, supplementary protection certificates and renewals of such Celgene Licensed Product IP and Celgene Licensed Product In-Licensed IP) solely to the extent necessary to research, Develop, Manufacture and Commercialize the Elected Candidate and Licensed Product. With respect to grants of a sublicense under subsection (2) above, Bluebird will be responsible for all amounts payable to the applicable licensor, excluding maintenance fee payments, payments that are triggered by the grant of a sublicense (but including payments triggered by further grants of sublicenses by Bluebird or its sublicensees) and Patent Costs, that are attributable to Bluebird as a sublicensee thereunder under this License Agreement and Celgene will pay same and Bluebird will

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reimburse Celgene for [\*\*\*] of such payments within thirty (30) days of receipt of Celgene’s written invoice therefor. Celgene will provide Bluebird with copies of all applicable Celgene Licensed Product In-Licenses promptly following the effective date of the termination of this License Agreement. The Prosecution and Maintenance and enforcement and defense rights and obligations of the Parties with respect to any Patents licensed or sublicensed to Bluebird pursuant to this Section 10.4(e) will be discussed and agreed to by the Parties, with the understanding that such Prosecution and Maintenance and enforcement and defense rights and obligations will be substantially similar to those set forth in Sections 6, with the roles of Bluebird and Celgene reversed (and such other changes as are appropriate from the context, and taking into account any rights retained by a Third Party licensor of Celgene to Prosecute and Maintain or enforce and defend any Patent sublicensed to Bluebird under this Section 10.4(e)). Bluebird will abide, and will cause all its Affiliates and applicable sublicensees to abide, by all requirements of each Celgene Licensed Product In-License under which Bluebird is sublicensed under this Section 10.4(e) in all material respects (and in any case in all respects in the case that failure to so abide would result in a breach under the Celgene Licensed Product In-License), to the extent applicable to sublicensees thereunder and to the extent disclosed by Celgene to Bluebird, with the understanding that disclosure by Celgene of any Celgene Licensed Product In-License to Bluebird will be deemed disclosure of such requirements of such Celgene Licensed Product In-License to Bluebird.

(f) *Trademarks*. Subject to Bluebird paying commercially reasonable compensation to Celgene for the license to be granted pursuant to this Section 10.4(f) (such compensation to either be mutually agreed to or determined through arbitration as provided in Section 10.4(g) below, and such compensation to be reduced by [\*\*\*] from what would be commercially reasonable compensation if this License Agreement is terminated by Bluebird pursuant to Section 10.2(a)), Celgene will exclusively license to Bluebird any registered or unregistered trademarks or internet domain names that are specific to and solely used for the Licensed Product worldwide (it being understood that the foregoing will not include any trademarks or internet domain names that contain the corporate or business name(s) of Celgene).

(g) *Baylor Product License*. If the Licensed Product is subject to a Baylor Product License, then Celgene will, at Bluebird’s written request, assign to Bluebird the applicable Baylor Product License in accordance with the terms of Section 13.5 thereof, provided that if such Baylor Product License applies to other products, Celgene will assign or sublicense to Bluebird that portion of the Baylor Product License that applies to the Licensed Product, and with the consequences therein stated (that is, Celgene will remain responsible for all payments accruing thereunder before the assignment, and Bluebird will be responsible for all payments accruing thereunder after such assignment).

(h) *Commercially Reasonable Compensation*. If the Parties are unable to agree on the amount of commercially reasonable compensation payable by Bluebird to Celgene pursuant to Sections 10.4(d), 10.4(e) or 10.4(f) within ten (10) days of the effective date of termination of this License Agreement, [\*\*\*]

(i) *Country Termination*. If this License Agreement is terminated only with respect to a specific country pursuant to Section 10.2(b), the provisions of this Section 10.4 will apply only with respect to such terminated country.



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10.5 Survival. In addition to the termination consequences set forth in Section 10.4, the following provisions will survive termination or expiration of this License Agreement: Sections 1, 3.3 (mutatis mutandis with respect to licenses granted to Bluebird under Section 10.4), 3.7, 3.8, 4.4, 5, 8, 9.3, 9.4, 9.6, 9.7, 10.4, and 11. Termination or expiration of this License Agreement will not relieve the Parties of any liability or obligation which accrued hereunder prior to the effective date of such termination or expiration nor preclude either Party from pursuing all rights and remedies it may have hereunder or at law or in equity with respect to any breach of this License Agreement nor prejudice either Party’s right to obtain performance of any obligation. All other rights and obligations will terminate upon expiration of this License Agreement.

10.6 Right to Set-off. Notwithstanding anything to the contrary in this License Agreement, each Party has the right at all times to retain and set off against all amounts due and owing to the other Party as determined in a final judgment any damages recovered by such Party for any Losses incurred by such Party.

## **11. General Provisions.**

11.1 Cumulative Remedies and Irreparable Harm. All rights and remedies of the Parties hereunder will be cumulative and in addition to all other rights and remedies provided hereunder or available by agreement, at law or otherwise. Each Party acknowledges and agrees that breach of any of the terms or conditions of this License Agreement would cause irreparable harm and damage to the other and that such damage may not be ascertainable in money damages and that as a result thereof the non-breaching Party would be entitled to seek from a court equitable or injunctive relief restraining any breach or future violation of the terms contained herein by the breaching Party without the necessity of proving actual damages or posting bond. Such right to equitable relief is in addition to whatever remedies either Party may be entitled to as a matter of law or equity, including money damages.

### **11.2 Business Combination and IP.**

(a) *Bluebird Business Combination*. Notwithstanding anything to the contrary herein, for purposes of this License Agreement, no Know-How, Materials, Patents, Regulatory Data, Regulatory Filings or Regulatory Approvals not Controlled by Bluebird or any of its Affiliates prior to a Business Combination of Bluebird will be Controlled for purposes of this License Agreement after such Business Combination of Bluebird, other than (i) Applicable Bluebird In-Licenses to the extent in effect immediately prior to such Business Combination of Bluebird, (ii) Collaboration IP, and (iii) any Patent that claims priority, directly or indirectly, to any other Patent first Controlled before such Business Combination of Bluebird will be Controlled thereafter no matter when such Patent is filed or issued.

(b) *Celgene Business Combination*. Notwithstanding anything to the contrary herein, for purposes of this License Agreement, no Know-How, Materials, Patents, Regulatory Data, Regulatory Filings or Regulatory Approvals not Controlled by Celgene or any of its Affiliates prior to a Business Combination of Celgene will be Controlled for purposes of this License Agreement after such Business Combination of Celgene, other than Collaboration IP, and except that any Patent that claims priority, directly or indirectly, to any other Patent first Controlled before such Business Combination of Celgene will be Controlled thereafter no matter when such Patent is filed or issued.

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11.3 Relationship of Parties. Nothing in this License Agreement is intended or will be deemed to constitute a partnership, agency, employer-employee or joint venture relationship between the Parties. No Party will incur any debts or make any commitments for the other, except to the extent, if at all, specifically provided therein. There are no express or implied third party beneficiaries hereunder (except for Bluebird Indemnitees and Celgene Indemnitees for purposes of Section 9.6).

11.4 Compliance with Law. Each Party will perform or cause to be performed any and all of its obligations or the exercise of any and all of its rights hereunder in good scientific manner and in compliance with all applicable Law. Without limiting the foregoing, Bluebird will comply with all applicable Laws and regulations (including U.S. Foreign Corrupt Practices Act and any other applicable anti-bribery or anti-kickback laws or regulations).

11.5 Force Majeure. Neither Party will be liable to the other for failure of or delay in performing obligations set forth in this License Agreement (other than any obligation to pay monies when due), and neither will be deemed in breach of such obligations, if such failure or delay is due to natural disasters or any causes reasonably beyond the control of such Party; provided that the Party affected will promptly notify the other of the force majeure condition and will exert reasonable efforts to eliminate, cure or overcome any such causes and to resume performance of its obligations as soon as possible.

11.6 Governing Law. This License Agreement will be governed by and construed in accordance with the Laws of the State of New York, without respect to its conflict of laws rules, provided that any dispute relating to the scope, validity, enforceability or infringement of any Patents or Know-How will be governed by, and construed and enforced in accordance with, the substantive Laws of the jurisdiction in which such Patents or Know-How apply.

11.7 Counterparts; Facsimiles. This License Agreement may be executed in one or more counterparts, each of which will be deemed an original, and all of which together will be deemed to be one and the same instrument. Facsimile or PDF execution and delivery of this License Agreement by either Party will constitute a legal, valid and binding execution and delivery of this License Agreement by such Party

11.8 Headings. All headings in this License Agreement are for convenience only and will not affect the meaning of any provision hereof.

11.9 Waiver of Rule of Construction. Each Party has had the opportunity to consult with counsel in connection with the review, drafting and negotiation of this License Agreement. Accordingly, the rule of construction that any ambiguity in this License Agreement will be construed against the drafting party will not apply.

11.10 Interpretation. Whenever any provision of this License Agreement uses the term “including” (or “includes”), such term will be deemed to mean “including without limitation” (or “includes without limitations”). “Herein,” “hereby,” “hereunder,” “hereof” and other equivalent words refer to this License Agreement as an entirety and not solely to the particular portion of this License Agreement in which any such word is used. All definitions set forth herein will be deemed applicable whether the words defined are used herein in the singular or the plural.

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Unless otherwise provided, all references to Sections and Appendices in this License Agreement are to Sections and Appendices of this License Agreement. References to any Sections include Sections and subsections that are part of the related Section (e.g., a section numbered “Section 2.1” would be part of “Section 2”, and references to “Section 2.1” would also refer to material contained in the subsection described as “Section 2.1(a)”).

11.11 Binding Effect. This License Agreement will inure to the benefit of and be binding upon the Parties, their Affiliates, and their respective lawful successors and assigns.

11.12 Assignment. This License Agreement may not be assigned by either Party, nor may either Party delegate its obligations or otherwise transfer licenses or other rights created by this License Agreement, except as expressly permitted hereunder or otherwise without the prior written consent of the other Party, which consent will not be unreasonably withheld, delayed or conditioned; provided that without consent (i) Celgene may assign this License Agreement to (x) an Affiliate or (y) its successor in connection with the merger, consolidation, or sale of all or substantially all of its assets, and (ii) Bluebird may assign this License Agreement to (x) an Affiliate or (y) its successor in connection with the merger, consolidation, or sale of all or substantially all of its assets or that portion of its business pertaining to the subject matter of this License Agreement; provided further that, except in the case where a Party is involved in a merger or consolidation where it is the surviving entity and no assets of such Party that are subject to this License Agreement have been transferred as a result of such merger or consolidation, (a) such assigning Party provides the other Party to this Agreement with at least thirty (30) business days advance written notice of such assignment(s) and the assigning Party agrees in a written agreement delivered prior to such assignment(s) to the non-assigning Party (and upon which such non-assigning Party may rely) to remain fully liable for the performance of its obligations under this Agreement by its assignee(s), (b) the assignee(s) agree in a written agreement delivered prior to such assignment(s) to the non-assigning Party (and upon which such non-assigning Party may rely) to assume performance of all such assigned obligations, (c) in the case of any assignment by Bluebird, all Licensed IP licensed to Celgene under this License Agreement will be transferred to such assignee(s) effective as of such assignment(s), (d) all of the matters referred to in clauses (a), (b) and (c), as applicable, will be set forth in documentation reasonably acceptable to the non-assigning Party prior to any such assignment(s) (and with such reasonable acceptance not to be unreasonably withheld, conditioned or delayed) and in all cases will provide the non-assigning Party with the full benefits of its rights under this Agreement (after taking into account all risks involving applicable counter-party performance and bankruptcy and insolvency risks, including those involving contractual rejection under 11 USC §365) as if no such assignment(s) had occurred, and (e) in the case of any assignment, the assigning Party will reimburse the non-assigning Party for all of the legal fees and expenses incurred by such non-assigning Party in connection with the matters set forth in clause (d) of this sentence in an aggregate amount not to exceed [\*\*\*], and provided, further, that if Bluebird wishes to assign any Licensed IP to its Affiliates, it will be permitted to do so conditioned on each such Affiliate becoming a party to this License Agreement, in the form of an amendment to this License Agreement executed by Celgene, Bluebird and such Affiliate, pursuant to which such Affiliate would agree to assume all obligations hereunder, and grant to Celgene all rights hereunder, with respect to the Licensed IP. The terms of this Agreement will be binding upon and will inure to the benefit of the successors, heirs, administrators and permitted assigns of the Parties. Any purported assignment in violation of this Section 11.12 will be null and void *ab initio*.

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11.13 Notices. All notices, requests, demands and other communications required or permitted to be given pursuant to this License Agreement will be in writing and will be deemed to have been duly given upon the date of receipt if delivered by hand, recognized international overnight courier, confirmed facsimile transmission, or registered or certified mail, return receipt requested, postage prepaid to the applicable address or facsimile number set forth in Section 13.14 of the Master Collaboration Agreement. Either Party may change its designated address and facsimile number by notice to the other Party in the manner provided in this Section 11.13

11.14 Amendment and Waiver. This License Agreement may be amended, supplemented, or otherwise modified only by means of a written instrument signed by both Parties; provided that any unilateral undertaking or waiver made by one Party in favor of the other will be enforceable if undertaken in a writing signed by the Party to be charged with the undertaking or waiver. Any waiver of any rights or failure to act in a specific instance will relate only to such instance and will not be construed as an agreement to waive any rights or fail to act in any other instance, whether or not similar.

11.15 Severability. In the event that any provision of this License Agreement will, for any reason, be held to be invalid or unenforceable in any respect, such invalidity or unenforceability will not affect any other provision hereof, and the Parties will negotiate in good faith to modify this License Agreement to preserve (to the extent possible) their original intent.

11.16 Entire Agreement. This License Agreement, together with the Master Collaboration Agreement, is the sole agreement with respect to the subject matter and supersedes all other agreements and understandings between the Parties with respect to same (including Confidential Agreement). In the event of any conflict between the terms of this License Agreement and the terms of the Master Collaboration Agreement, the terms of this License Agreement will control.

11.17 Force Majeure. Neither Celgene nor Bluebird will be liable for failure of or delay in performing obligations set forth in this License Agreement (other than any obligation to pay monies when due), and neither will be deemed in breach of such obligations, if such failure or delay is due to natural disasters or any causes reasonably beyond the control of Celgene or Bluebird and without the fault or negligence of the Party so failing or delaying; provided that the Party affected will promptly notify the other of the force majeure condition and will exert reasonable efforts to eliminate, cure or overcome any such causes and to resume performance of its obligations as soon as possible.

*[Remainder of this Page Intentionally Left Blank]*

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IN WITNESS WHEREOF, the Parties have caused this License Agreement to be executed by their respective duly authorized officers as of the License Agreement Effective Date.

BLUEBIRD BIO, INC.

By: \_\_\_\_\_  
(Signature)

Name: \_\_\_\_\_

Title: \_\_\_\_\_

Date: \_\_\_\_\_

CELGENE CORPORATION

By: \_\_\_\_\_  
(Signature)

Name: \_\_\_\_\_

Title: \_\_\_\_\_

Date: \_\_\_\_\_

---

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**Appendix A**

**Additional Defined Terms**

“Elected Candidate”<sup>20</sup> means the Optioned Candidate selected by Celgene under the Master Collaboration Agreement that specifically targets the following Target Antigen: [ ].

<sup>20</sup> *To be updated by the Parties to specifically identify the candidate that is the subject of the option election .*

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**Appendix B**

**Applicable New In-Licenses**

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**Appendix C**

**Applicable Pre-Existing In-Licenses**



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**Appendix D**

**Certain Manufacturing Definitions**

[\*\*\*]

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**Appendix E**

**Press Release**

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**Appendix F**

**Certain Patents within the Licensed IP Controlled  
by Bluebird as of the License Agreement Effective Date**

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**Appendix G**

**Bluebird Agreements**

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[\*\*\*]

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**Appendix I**

**Redacted Version of License Agreement**

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**Schedule 9.2**

**Exceptions to Bluebird’s Representations and Warranties in Section 9.2**

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**Exhibit B**

**Co-Development, Co-Promote and Profit Share Agreement**



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**Co-Development, Co-Promote and Profit Share Agreement**

**by and between**

**bluebird bio, Inc.**

**and**

**Celgene Corporation**

[            ]

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- Appendix B Applicable New In-Licenses
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- Appendix D Certain Manufacturing Definitions
- Appendix E Co-Co In-Licenses
- Appendix F Profit & Loss Share
- Appendix G Press Release
- Appendix H Certain Patents Within the Licensed IP as of the CCPS Agreement Effective Date
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### **Co-Development, Co-Promote and Profit Share Agreement**

This Co-Development, Co-Promote and Profit Share Agreement (this “CCPS Agreement”), dated as of [ ] (the “CCPS Agreement Effective Date”), is made by and between bluebird bio, Inc., a Delaware corporation (“Bluebird”), and Celgene Corporation, a Delaware (“Celgene”). Each of Bluebird and Celgene may be referred to herein as a “Party” or together as the “Parties.”

WHEREAS, Bluebird has developed and owns or has rights to certain Patents and technology relating to developing innovative gene therapies for genetic disorders;

WHEREAS, Celgene is a biopharmaceutical company focused on acquiring, Developing and Commercializing innovative anti-cancer agents; and

WHEREAS, Bluebird and Celgene are parties to that certain Master Collaboration Agreement (dated [ ], 2013) (the “Master Collaboration Agreement”) pursuant to which Celgene has an option to take a license to Product Candidates;

WHEREAS, pursuant to the terms of the Master Collaboration Agreement, Celgene has exercised its option to select a Product Candidate to be an Optioned Candidate by delivering to Bluebird a Celgene Option Notice and payment of the applicable Initial Option Fee (such Optioned Candidate, as defined more fully in Appendix A, the “Elected Candidate”);

WHEREAS, pursuant to Section 5.3 of the Master Collaboration Agreement, Bluebird has delivered a Bluebird Option Notice to co-promote and co-Develop the Optioned Candidate in the U.S.; and

WHEREAS, the Parties now wish to enter into an exclusive arrangement whereby Bluebird and Celgene will co-Develop Licensed Product and Commercialize Licensed Product in the U.S. as part of a profit share arrangement, and Celgene will have exclusive rights to Commercialize Licensed Product in the ROW, all on the terms and conditions set forth here.

NOW, THEREFORE, in consideration of the mutual covenants contained herein, and for other good and valuable consideration, the amount and sufficiency of which are hereby acknowledged, the Parties hereby agree as follows:

#### **1. Definitions.**

The following terms and their correlatives will have the meanings set forth below. Capitalized terms used, but not defined, herein will have the meanings ascribed to such terms in the Master Collaboration Agreement.

1.1 “Applicable Bluebird In-Licenses” means the Applicable Pre-Existing In-Licenses, the Applicable New In-Licenses, and any Co-Co In-Licenses where Bluebird is a contracting party.

1.2 “Applicable New In-Licenses” means all New In-Licenses of Bluebird or its Affiliates necessary or useful for the research, Development and/or Commercialization of Elected Candidate and Licensed Product that Celgene has elected to list on Appendix B as of the CCPS Agreement Effective Date, plus any other New In-License of Bluebird or its Affiliates that Celgene has elected to include as an Applicable New In-License pursuant to Section 10.7(b).

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1.3 “Applicable Pre-Existing In-Licenses” means all Pre-Existing In-Licenses necessary or useful for the research, Development and/or Commercialization of Elected Candidate and Licensed Product, and any extensions or expansions of the scope of such Pre-Existing In-Licenses, including those listed on Appendix C.

1.4 “Biosimilar Product” means, with respect to a Licensed Product in any country, any biosimilar product sold by a Third Party not authorized by or on behalf of Celgene, its Affiliates or Sublicensees, (i) that is a biosimilar biological product, as defined in 21 USC 379j-51 (or any successor or replacement thereof), a similar biological medicinal product, as defined in Annex I to Directive 2001/83/EC (or any successor or replacement thereof), or any similar biosimilar or generic product under the Laws of any country or jurisdiction, or (ii) regarding which Regulatory Approval is obtained by referencing Regulatory Data of such Licensed Product.

1.5 “Bluebird In-Licensed IP” means all Patents, Materials and Know-How in-licensed by Bluebird pursuant to Applicable Bluebird In-Licenses, including any extensions or expansions of the scope thereof.

1.6 “Bluebird Licensed IP” means all (i) Patents, Materials and Know-How Controlled at any time by Bluebird or any of its Affiliates (including any applicable Collaboration IP and Bluebird Technology) other than pursuant to an Applicable Bluebird In-License and (ii) Bluebird In-Licensed IP, in each case to the extent necessary or useful to Develop Elected Candidate and Develop and Commercialize Licensed Product.

1.7 “Bluebird Regulatory Rights” means all Regulatory Data, Regulatory Filings and Regulatory Approvals for Elected Candidate and Licensed Product worldwide Controlled at any time by Bluebird or any of its Affiliates.

1.8 “Bluebird Technology” means all Bluebird Solely Owned IP and all of Bluebird’s right, title and interest in and to Joint IP.

1.9 “Celgene Licensed IP” means (i) Celgene Licensed Product IP, and (ii) Celgene Licensed Product In-Licensed IP.

1.10 “Celgene Licensed Product In-License” means [\*\*\*]

1.11 “Celgene Licensed Product In-Licensed IP” means [\*\*\*]

1.12 “Celgene Licensed Product IP” means [\*\*\*]

1.13 “Celgene Other In-License” means [\*\*\*]

1.14 “Celgene Regulatory Rights” means all Regulatory Data, Regulatory Filings and Regulatory Approvals for Elected Candidate and Licensed Product worldwide Controlled at any time by Celgene or any of its Affiliates.

1.15 “Celgene Technology” means all Celgene Solely Owned IP and all of Celgene’s right, title and interest in and to Joint IP.

1.16 “Commercialization” means any and all activities directed to the Manufacturing, marketing, detailing, promotion and securing of reimbursement of a product after Regulatory Approval has been obtained (including making, having made, using, importing, selling and offering for sale such product), and will include post-approval clinical studies, post-launch

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marketing, promoting, detailing, marketing research, distributing, customer service, administering and commercially selling such product, importing, exporting or transporting such product for commercial sale, and all regulatory compliance with respect to the foregoing.

1.17 “Commercially Reasonable Efforts” means, with respect to the Development or Commercialization of Licensed Product by a Party, that level of efforts and resources that such Party would normally devote to the Development or Commercialization, as the case may be, of a product owned by it or to which it has rights of the type it has hereunder, which is of a similar commercial potential at a similar stage in its lifecycle, in each case taking into account issues of safety and efficacy, product profile, the proprietary position, the then current competitive environment for such product and the likely timing of such product’s entry into the market, the pricing and launching strategy for the respective product, the regulatory environment and status of such product, and other relevant scientific, technical and commercial factors.

1.18 “Control” or “Controlled” means, with respect to any Know-How, Material, Patent, Regulatory Data, Regulatory Filings and Regulatory Approvals, the possession (whether by ownership or license, other than by a license or sublicense granted pursuant to this CCPS Agreement) by a Party or its Affiliates of the ability to grant to the other Party a license or access as provided herein to such item, without violating the terms of any agreement or other arrangement with any Third Party or, other than under Applicable Bluebird In-Licenses, being obligated to pay any royalties or other consideration therefor (“Additional Payments”). For clarity, Other In-Licenses are not “Controlled” for purposes of this CCPS Agreement, unless and only after such Other In-License is converted into an Applicable New In-License pursuant to Section 10.7(b). Notwithstanding the foregoing, as provided in Section 10.7(a), if on or after the CCPS Agreement Effective Date and for such time as the other Party agrees to pay and does in fact pay all Additional Payments with respect to such Party’s access or license to any Know-How, Material, Patent, Regulatory Data, Regulatory Filings and Regulatory Approvals (other than that in-licensed by Bluebird pursuant to an Other In-License), such Know-How, Material, Patent, Regulatory Data, Regulatory Filings and Regulatory Approvals will be deemed to be included in the definition of “Control”.

1.19 “Covers”, with reference to (i) a Patent, means that the making, using, selling, offering for sale or importing of a product or practice of a method would infringe a Valid Claim of such Patent in the country in which such activity occurs, and (ii) Materials or Know-How, means that the Manufacture, Development or Commercialization of a product incorporates, embodies or otherwise makes use of such Materials or Know-How.

1.20 “EU” means the organization of member states of the European Union as it may be constituted from time to time.

1.21 “EU Regulatory Event” means, with respect to a Licensed Product, the earlier to occur of [\*\*\*]

1.22 “First Commercial Sale” means [\*\*\*]

1.23 “First Indication” means [\*\*\*]

1.24 “GAAP” means U.S. generally accepted accounting principles or International Financial Reporting Standards, consistently applied, as designated and used by the applicable Party.

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1.25 “In-License Payments” means any amounts paid or payable under any Applicable Bluebird In-License that are incurred by Bluebird solely and directly as a result of the grant of a sublicense thereunder under this CCPS Agreement to Celgene, any of Celgene’s contract Third Parties under Section 10.5, or any further Sublicensees of Celgene (including of Celgene’s Affiliates that are granted sublicenses) under this CCPS Agreement. Any such payments will include (i) any amounts paid or payable under any Applicable Bluebird In-License solely and directly as a result of the grant of a sublicense (or an option thereto) by Bluebird to Celgene, [\*\*\*]

1.26 “Licensed IP” means Bluebird Licensed IP and Celgene Licensed IP.

1.27 “Licensed Product” means any product that constitutes or incorporates an Elected Candidate (including all modified and improved versions thereof), in all forms, presentations, and formulations (including manner of delivery and dosage). A modified or improved version of an Elected Candidate constituted or incorporated in a product will be deemed a “Modified Licensed Product” for purposes of Section 11.2 if it is Covered by patentable technology Controlled by Bluebird that (i) is first discovered, created, conceived, developed or reduced to practice after the later of (a) the CCPS Agreement Effective Date and (b) the end of the Collaboration Program Term, (ii) requires the submission of a new BLA with respect to such modified or improved Elected Candidate, and (iii) materially contributes to the Elected Candidate being approved for a new indication or new patient population. For clarity, “Modified Licensed Products” are Licensed Products hereunder for all purposes other than Section 11.2.

1.28 “Manufacturing” means the production, manufacture, processing, filling, finishing, packaging, labeling, shipping and holding of product or any intermediate thereof, including process development, process qualification and validation, scale-up, commercial manufacture and analytic development, product characterization, stability testing, quality assurance and quality control. With reference to Elected Candidate and Licensed Product, Manufacturing includes Vector and associated Payload supply.

1.29 “Net Sales” means [\*\*\*]

1.30 “Pivotal Study” means (i) a Phase 3 Study that is intended by Celgene to be submitted (together with any other registration trials that are prospectively planned when such Phase 3 Study is initiated) for Regulatory Approval in the U.S. or the EU, or (ii) any other clinical study that is designed to establish that a pharmaceutical product is safe and efficacious for its intended use, and to determine warnings, precautions, and adverse reactions that are associated with such pharmaceutical product in the dosage range to be prescribed, which clinical study is a registration trial intended to be sufficient for filing an application for a Regulatory Approval for the Licensed Product in the U.S. or another country or some or all of an extra-national territory, solely as evidenced by the acceptance for filing for a Regulatory Approval for such product after completion of such study.

1.31 “Regulatory Exclusivity Period” means with respect to a Licensed Product in a country, the period of time during which (a) Celgene or any of its Affiliates or Sublicensees has been granted the exclusive legal right by a Regulatory Authority (or is otherwise entitled to the

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exclusive legal right by operation of Law) in such country to market and sell the Licensed Product, or (b) the data and information submitted by Celgene or any of its Affiliates or Sublicensees to the relevant Regulatory Authority in such country for purposes of obtaining Regulatory Approval may not be disclosed, referenced or relied upon in any way by such Regulatory Authority (including by relying upon the Regulatory Authority’s previous findings regarding the safety or effectiveness of the Licensed Product) to support the Regulatory Approval or marketing of any product by a Third Party in such country.

1.32 “ROW” means the world other than the United States.

1.33 “ROW Administration” means administration of Licensed Product to a patient when located in the ROW.

1.34 “ROW Development & Commercialization Program” means the program under this CCPS Agreement for the Development of Elected Candidate and Licensed Product in the ROW, the Commercialization of Licensed Product in the ROW, and all Manufacturing (including Manufacturing of Vectors and associated Payloads) therefor.

1.35 “ROW Development Plan” means the Development plan for the Development of Elected Candidate and Licensed Product for ROW Administration during a given calendar year and the two (2) succeeding calendar years.

1.36 “Second Indication” means [\*\*\*]

1.37 “Selling Party” means a Party and its Sublicensees (including such Party’s Affiliates that are granted sublicenses pursuant to Section 10.3(c)).

1.38 “Sublicensee” means any person or entity (including Affiliates of the applicable Party) that is granted a sublicense as permitted by Section 10.3 (or an option to take such a sublicense), either directly by a Party or indirectly by any other Sublicensee hereunder.

1.39 “U.S. Administration” means administration of Licensed Product to a patient when located in the United States.

1.40 “U.S. Commercialization Budget” means the budget for conducting Commercialization in accordance with the U.S. Commercialization Plan during a given calendar year and the two (2) succeeding calendar years, as approved by the JGC in accordance with Section 5.3.

1.41 “U.S. Commercialization Plan” means that portion of the Worldwide Commercialization Plan that specifies the Commercialization plan for the Commercialization of Licensed Product for U.S. Administration during a given calendar year and the two (2) succeeding calendar years.

1.42 “U.S. Development Budget” means the budget for conducting Development of Elected Candidate and Licensed Product for U.S. Administration pursuant to the U.S. Development Plan during a given calendar year and the two (2) succeeding calendar years, as approved by the JGC in accordance with Section 4.3.

1.43 “U.S. Development Plan” means the Development plan for the Development of Elected Candidate and Licensed Product for U.S. Administration during a given calendar year and the two (2) succeeding calendar years, as approved by the JGC in accordance with Section 4.2.



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1.44 “U.S. Development & Commercialization Program” means the program under this CCPS Agreement for the Development of Elected Candidate and Licensed Product in the United States, the Commercialization of Licensed Product in the United States, and all Manufacturing (including Manufacturing of Vectors and associated Payloads) therefor.

1.45 “Valid Claim” means, with respect to a particular country, (i) any claim of an issued and unexpired Patent in such country that (a) has not been held revoked, unenforceable or invalid by a decision of a court or governmental agency of competent jurisdiction, which decision is unappealable or unappealed within the time allowed for appeal and (b) has not been abandoned, disclaimed, denied or admitted to be invalid or unenforceable through reissue or disclaimer or otherwise in such country, or (ii) a claim of a pending Patent application that has not been finally abandoned or finally rejected or expired and which has been pending [\*\*\*] from the date of filing of the earliest priority Patent application to which such pending Patent application is entitled to claim benefit.

1.46 “Vector Supplies” means supplies of Vectors and associated Payloads Manufactured for incorporation into Elected Candidate and Licensed Product for Development or Commercialization thereof.

1.47 “Worldwide Commercialization Plan” means the Commercialization Plan that specifies the Commercialization plan for the Commercialization of Licensed Product for U.S. Administration and ROW Administration during a given calendar year and the two (2) succeeding calendar years.

1.48 “Worldwide Manufacturing Plan” means the Manufacturing plan for the Elected Candidate and Licensed Product for Development for both U.S. Administration and ROW Administration.

Definitions for each of the following terms are found in the body of this CCPS Agreement or the Appendices hereto as indicated below:

<u>Defined Terms</u>	<u>Location</u>
Additional Bluebird IP	Section 10.7(a)
Allowable Expenses	Appendix F
Allocable Manufacturing Overhead	Appendix D
Allocable Overhead	Appendix F
[***]	
[***]	
Bluebird Indemnitees	Section 11.6(a)
Budgeted U.S. Development Costs	Section 4.3
Business Acquisition	Section 10.4
Business Party	Section 10.4
Business Program	Section 10.4
CCPS Agreement Term	Section 12.1
Celgene Indemnitees	Section 11.6(b)

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<u>Defined Terms</u>	<u>Location</u>
Commercial Supplies	Appendix D
Competitive Infringement [***]	Section 14.1
Cost of Goods Sold or COGS [***]	Appendix F
Development & U.S. Commercialization Program	Section 8.3(a)
Distribution Costs	Appendix F
Elected Candidate	Appendix A
Fully Burdened Manufacturing Cost	Appendix D
Gross Profit	Appendix F
Gross Sales	Appendix F
Indemnification Claim Notice	Section 11.6(c)
Indemnified Party	Section 11.6(c)
Information Request	Section 5.6(g)
JGC	Section 3.1(a)
Joint IP	Section 12.2
Losses	Section 11.6(a)
Major EU Countries	Section 1.21
Manufacturing and Supply Agreement	Section 7.4(b)(ii)
Marketing Costs	Appendix F
Milestone Event	Section 11.2(a)
Milestone Payment	Section 11.2(a)
Modified Licensed Product [***]	Section 1.24
Operating Profits or Losses	Appendix F
Other Operating Income/Expense	Appendix F
Profit & Loss Share	Section 11.4
ROW Post-Approval Manufacturing Plan	Section 7.3
Sales Costs	Appendix F
Sales Returns and Allowances	Appendix F
Solely Owned IP	Section 12.1
Specific Patent	Section 13.3
Third Party Claims	Section 11.6(a)
U.S. Administration Liabilities	Section 16.8
U.S. Development Costs	Appendix F

## **2. Overview.**

2.1 General. During the CCPS Agreement Term, the Parties will conduct the Development and Commercialization of Elected Candidate and Licensed Product worldwide on the terms and conditions set forth in this CCPS Agreement.

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**2.2 Roles and Responsibilities; Diligence.**

(a) The JGC will assign to each Party roles and responsibilities for performing the U.S. Development & Commercialization Program. Each Party, directly or through one or more of its Affiliates, Sublicensees or permitted subcontractors, will use Commercially Reasonable Efforts to perform the obligations assigned to such Party by the JGC under the U.S. Development & Commercialization Program. Each Party will reasonably cooperate with the other Party in performing such obligations.

(b) Celgene will assume sole responsibility for, and control of, Developing Elected Candidate and Licensed Product in the Field outside of the United States, and will establish a ROW Development & Commercialization Program for that purpose. Bluebird will reasonably cooperate with Celgene in such ROW Development & Commercialization Program.

**2.3 Technical Assistance.** During the Collaboration Program Term, Bluebird will reasonably cooperate with Celgene to provide all technical assistance, and to transfer to Celgene any additional Know-How licensed to Celgene under Section 10.1, requested by Celgene to facilitate the transfer of Development efforts related to Elected Candidate and Licensed Product. Such cooperation will include providing Celgene with reasonable access by teleconference or in-person at Bluebird’s facilities to Bluebird personnel involved in the research and Development of Elected Candidate to provide Celgene with a reasonable level of technical assistance and consultation in connection with the transfer of such Know-How. Following the Collaboration Program Term, Bluebird will reasonably cooperate with Celgene to provide reasonable amounts of technical assistance, including to transfer to Celgene any additional Know-How licensed to Celgene under Section 10.1, with respect to Elected Candidate or Licensed Product as reasonably requested by Celgene with reasonable advance notice to Bluebird. Any dispute with respect to the amount and completeness of the technical assistance and cooperation to be provided by Bluebird under this Section 2.3 will be referred to and finally resolved by binding arbitration by a mutually agreeable, disinterested, conflict-of-interest-free individual not affiliated or consulting with either Party. Any such arbitration will be conducted under the then-current rules of the American Arbitration Association.

**3. Governance and Joint Governance Committee.**

**3.1 Joint Governance Committee.**

(a) *Governance Committee.* As soon as practicable following the CCPS Agreement Effective Date, the Parties will establish a Joint Governance Committee, comprised of three (3) representatives of Bluebird and three (3) representatives of Celgene (the “JGC”). Each Party may replace its representatives on the JGC or its Program Director at any time upon written notice to the other Party. With the consent of the other Party (such consent not to be unreasonably withheld, delayed or conditioned), each Party may invite non-voting employees and consultants to attend meetings of the JGC, subject to their agreement to be bound to the same extent as a permitted subcontractor under Section 2.4.

(b) *Meetings.* While in existence, the JGC will meet each calendar quarter and, at a minimum, two (2) of such meetings each calendar year will be in person (which in-person meeting will be held at locations mutually agreed by the Parties). In addition, either Party can

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call a meeting of the JGC on five (5) business days prior written notice. Meetings of the JGC will be effective only if at least one (1) representative of each Party is present or participating. Each Party will be responsible for all of its own expenses of participating in the meetings. The Parties will endeavor to schedule the calendar quarterly meetings of the JGC at least six (6) months in advance. The Parties will alternate in preparing and circulating a meeting agenda prior to each such meeting. The Party that prepared the agenda (or called the meeting) will prepare written minutes of such meeting, and the preparing Party will circulate such minutes within fifteen (15) days after such meeting. The Parties will agree on the minutes of each meeting promptly, but in no event later than the next meeting of the JGC.

(c) *Responsibilities*. The JGC will supervise the overall performance of the Development and Commercialization of Elected Candidate and Licensed Product for U.S. Administration, and within such scope will:

(i) Make all decisions regarding the Parties’ performance of the U.S. Development & Commercialization Program (except as otherwise expressly provided in this CCPS Agreement), including, subject to Section 2.2, which Party will have which responsibilities under the U.S. Development & Commercialization Program (taking into account each Party’s reasonably available resources and expertise (either directly or through Third Party contracting));

(ii) Review and seek to coordinate the U.S. Development & Commercialization Program with the ROW Development & Commercialization Program;

(iii) Address all matters specifically delegated to the JGC pursuant to this CCPS Agreement;

(iv) Form such other committees as the JGC may deem appropriate, and require that such committees meet at such times and places, provided that such committees may make recommendations to the JGC but may not be delegated JGC decision-making authority;

(v) Address such other matters relating to the activities of the Parties under this CCPS Agreement as either Party may bring before the JGC, including any matters that are expressly for the JGC to decide as provided in this CCPS Agreement; and

(vi) Attempt to resolve any disputes on an informal basis.

(d) *Decision-making*. The three (3) JGC representatives of each Party will collectively have one (1) vote, and the JGC will make decisions only by unanimous consent of each Party with respect to its vote, and each Party will act reasonably in exercising its vote. [\*\*\*]

(e) *Limits on JGC Authority*. Each Party will retain the rights, powers and discretion granted to it under this CCPS Agreement and no such rights, powers, or discretion will be delegated to or vested in the JGC unless such delegation or vesting of rights is expressly provided for in this CCPS Agreement or the Parties expressly so agree in writing. The JGC will not have the power to, nor will the Party having the tie-breaking vote in the JGC have the power to (i) amend, modify or waive compliance with this CCPS Agreement (other than as expressly permitted hereunder), (ii) alter, increase or expand the Parties’ rights or obligations under this CCPS Agreement (other than as permitted by Section 2.2), (iii) determine that a Party has

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fulfilled any obligations under this CCPS Agreement or that a Party has breached any obligation under this CCPS Agreement, (iv) make a decision that is expressly stated to require the mutual agreement of the Parties, or (v) determine that milestone events required for the payment of milestone payments have or have not occurred. For avoidance of doubt, the JGC will have no right to supervise or direct the Development and Commercialization of Elected Candidate or Licensed Product for ROW Administration, and Celgene will have sole decision making authority with respect to such Development and Commercialization, including with respect to the ROW Development & Commercialization Program.

(f) *Term.* The JGC will cease to exist upon the end of the CCPS Agreement Term, unless the Parties elect to extend the JGC upon termination of expiration of this CCPS Agreement.

#### **4. Development.**

4.1 Generally. As of and after the CCPS Agreement Effective Date, subject to the terms and conditions of this CCPS Agreement, the Parties will assume through the JGC joint responsibility for Development of Elected Candidate and Licensed Product for U.S. Administration, under the U.S. Development & Commercialization Program, and Celgene will assume responsibility for Development of Elected Candidate and Licensed Product for ROW Administration, under the ROW Development & Commercialization Program. Notwithstanding the foregoing, if the initial Phase 1 Study with respect to Optioned Candidate has not been completed as of the CCPS Agreement Effective Date, at Celgene’s election, Bluebird will continue to be responsible for the performance of such initial Phase 1 Study under the oversight of the JSC under the Master Collaboration Agreement until completion of such initial Phase 1 Study. In the event Bluebird continues, at Celgene’s election, to continue to be responsible for the performance of such initial Phase 1 Study, Bluebird will be responsible for the costs of performing such initial Phase 1 Study until the earlier to occur of (i) completion of such initial Phase 1 Study and (ii) expiration or termination of the Collaboration Program Term; following the end of the Collaboration Program Term, Bluebird’s out-of-pocket costs of performing such initial Phase 1 Study incurred after the end of the Collaboration Program Term will be Development costs hereunder and, (a) if incurred for Licensed Product for U.S. Administration, will be subject to Section 4.3 and Section 11.4, and (b) if incurred for Licensed Product solely for ROW Administration, Celgene will reimburse Bluebird for such costs within thirty (30) days of Celgene’s receipt of Bluebird’s written invoice therefor.

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4.2 Development Plan. Promptly after the CCPS Agreement Effective Date, Celgene will prepare an initial U.S. Development Plan, and the JGC will review and approve such initial U.S. Development Plan, with the goal of coordinating and harmonizing the U.S. Development Plan with the ROW Development Plan. Thereafter, Celgene will update the U.S. Development Plan each calendar year, and the JGC will review and approve any such update or any other amendment to the U.S. Development Plan. In addition, either Party may request at any time that the JGC consider and approve other updates to the U.S. Development Plan. Promptly after the CCPS Agreement Effective Date, Celgene will prepare an initial ROW Development Plan and will provide it to the JGC for purposes of discussion and the goal of coordinating and harmonizing the U.S. Development Plan and the ROW Development Plan. Thereafter, Celgene will update the ROW Development Plan each year and submit it to the JGC for purposes of discussion and the goal of coordinating and harmonizing the U.S. Development Plan and ROW Development Plan. Notwithstanding anything in this CCPS Agreement to the contrary, the Parties acknowledge and agree that (i) Bluebird may decline to perform any Development activity proposed to be conducted by Bluebird in Worldwide Commercialization Plan (excluding the completion of a Phase 1 Study pursuant to Section 4.1 and excluding Manufacturing of Vectors and associated Payloads), and (ii) the U.S. Development Plan will not include, and Bluebird will have no obligation to perform, any such Development activity that Bluebird has declined to perform (other than the completion of a Phase 1 Study pursuant to Section 4.1 and Manufacture of Vectors and associated Payloads), provided that once Bluebird has agreed to perform a Development activity, it will be obligated to perform, and cannot decline to perform, such activity. Further:

(a) The JGC will set the required form and contents of the U.S. Development Plan. The JGC will seek to coordinate and harmonize the U.S. Development Plan and the ROW Development Plan.

(b) Neither Party (itself or by or through any others, including any Affiliates or Sublicensees) will take any material action regarding the Development of Elected Candidate or Licensed Product for U.S. Administration unless described in the U.S. Development Plan, provided that the foregoing will not restrict Celgene from taking any action regarding the Development of Elected Candidate or Licensed Product for ROW Administration.

(c) All Development of Elected Candidate and Licensed Product for U.S. Administration will be conducted under the supervision of the JGC and as part of the U.S. Development & Commercialization Program.

(d) All Development of Elected Candidate and Licensed Product for ROW Administration will be conducted under the sole control of Celgene and as part of the ROW Development & Commercialization Program. At each calendar quarter meeting of the JGC, Celgene will provide the JGC with an update on the Development of Elected Candidate and Licensed Product by Celgene for ROW Administration. During such meeting, Celgene will disclose to Bluebird all material information regarding such Development.

(e) Celgene will prepare and maintain, and will cause its Affiliates and Sublicensees to prepare and maintain, reasonably complete and accurate records regarding the Development of Elected Candidate and Licensed Product for ROW Administration. At each calendar quarter

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meeting of the JGC, Celgene will provide the JGC with a reasonably detailed report regarding such efforts. Such report will contain sufficient detail to enable Bluebird to assess Celgene’s compliance with its Development and Commercialization obligations hereunder, including information with respect to the following: (i) the design, status and results of any animal studies and clinical trials for Licensed Product; and (ii) any regulatory milestones, and any Regulatory Approvals achieved, for Licensed Product. In addition to the foregoing, Celgene will provide Bluebird with such additional information regarding any such activities as Bluebird may reasonably request from time to time.

4.3 Development Budget and Costs. Promptly after the CCPS Agreement Effective Date, and concurrently with the preparation of the U.S. Development Plan, Celgene will prepare an initial U.S. Development Budget, which U.S. Development Budget will specify estimated U.S. Development Costs for each calendar year covered by such U.S. Development Budget (as updated pursuant to the following sentence, the “Budgeted U.S. Development Costs”), and the JGC will review and approve, where practicable, such initial U.S. Development Budget at least six (6) months in advance of such U.S. Development Costs being incurred. [\*\*\*]

## **5. Commercialization.**

5.1 Generally. Subject to the terms and conditions of this CCPS Agreement, (i) the Parties will assume through the JGC joint responsibility for Commercialization of Licensed Product for U.S. Administration under the U.S. Development & Commercialization Program, and (ii) Celgene will assume sole responsibility for Commercialization of Licensed Product for ROW Administration (including all costs and expenses arising therefrom).

5.2 Commercialization Plan. At such times as the JGC will deem appropriate, the JGC will direct the Parties to mutually prepare a Worldwide Commercialization Plan, and the JGC will review and approve such initial Worldwide Commercialization Plan. Thereafter, the JGC will have one or the other Party (or both) update the Worldwide Commercialization Plan each calendar year, and the JGC will review and approve any such update or any other amendment to the Worldwide Commercialization Plan. Notwithstanding anything in this CCPS Agreement to the contrary, the Parties acknowledge and agree that (i) Bluebird may decline to perform any Commercialization activity proposed to be conducted by Bluebird in the Worldwide Commercialization Plan (other than Manufacturing of Vectors and associated Payloads), and (ii) the Worldwide Commercialization Plan will not include, and Bluebird will have no obligation to perform, any such Commercialization activity that Bluebird has declined to perform, provided that once Bluebird has agreed to perform a Commercialization activity, it will be obligated to perform, and cannot decline to perform, such activity. In addition, either Party may request at any time that the JGC consider and approve other updates to the Worldwide Commercialization Plan. Further:

(a) The JGC will set the required form and contents of the Worldwide Commercialization Plan. The Worldwide Commercialization Plan will reflect a singular marketing and sales approach worldwide, and will specify, among other things, the number of sales reps in the U.S. for each Party, allocation of regions in the U.S. for each Parties’ sales force, creation of marketing materials, planning for conferences, and other marketing activities.

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(b) Neither Party (itself or by or through any others, including any Affiliates or Sublicensees) will take any material action regarding the Commercialization of Licensed Product unless described in the Worldwide Commercialization Plan or approved by the JGC.

(c) All Commercialization of Licensed Product for U.S. Administration will be conducted under the supervision of the JGC and as part of the U.S. Development & Commercialization Program.

(d) Celgene will have final decision making authority for all Commercialization activities worldwide, including timing of launch and pricing and the Worldwide Development Plan.

5.3 U.S. Commercialization Budget. At such times as the JGC will deem appropriate, and concurrently with the preparation of the initial Worldwide Commercialization Plan, Celgene will prepare an initial U.S. Commercialization Budget, and the JGC will review and approve such initial U.S. Commercialization Budget. [\*\*\*]

5.4 Commercialization in the ROW. Celgene, directly or through one or more of its Affiliates or Sublicensees, will use Commercially Reasonable Efforts, (i) to Develop Licensed Product in the Field for ROW Administration and to obtain Regulatory Approvals therefor; and (ii) to Commercialize Licensed Product in the Field for ROW Administration after obtaining such Regulatory Approval, in each country in the ROW where Commercializing Licensed Product would be warranted by using Commercially Reasonable Efforts.

5.5 Branding. Subject to further mutual written agreement of the Parties, to the extent permitted by applicable Law and applicable Regulatory Authorities, (i) all Licensed Product sold or distributed for U.S. Administration will have the corporate brands of each Party displayed on an equally prominent basis, and (ii) all Licensed Product sold or distributed for ROW Administration will have the corporate brand of Bluebird displayed on a reasonably prominent basis. At such time as the JGC will deem appropriate, the Parties will enter into appropriate trademark licensing agreements to achieve the foregoing.

#### 5.6 Training; Details.

(a) Celgene will direct the training of both Parties' sales representatives and will prepare and implement, in consultation with Bluebird, a training program and training materials for such sales representatives. In addition, Celgene will specify the conduct and content of details (including detail scripts) for the Licensed Product. Bluebird will cause each of its sales representatives assigned to promote the Licensed Product to attend and complete the training program developed by Celgene for the Licensed Product in the United States to assure a consistent, focused promotional strategy and message as and to the extent consistent with applicable Law.

(b) Each Party will be solely responsible for recruiting, hiring and maintaining its sales force of sales representatives for promotion of the Licensed Product in accordance with its standard procedures and the requirements of this CCPS Agreement. Each Party will be responsible for the activities of its sales representatives, including compliance by its sales representatives with training and detailing requirements. In particular, each Party will provide its sales representatives assigned to promote the Licensed Product with the level of oversight,



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management, direction and sales support with respect to the promotion of Licensed Product necessary to effectively and efficiently promote the Licensed Product in accordance with the terms of this CCPS Agreement and applicable Law. If Celgene raises any concern with Bluebird regarding the performance or fitness of any Bluebird sales representative, Bluebird will address such concerns in a manner consistent with Celgene’s instructions, including removal of such sales representative from the promotion of the Licensed Product.

(c) Each Party’s sales representatives assigned to promote the Licensed Product will utilize only promotional materials that have been approved by the JGC. All detailing activities conducted by each Party’s sales representatives will be consistent in all material respects with the promotional materials so approved. Each Party will train and instruct their respective sales representatives to make only those statements and claims regarding the Licensed Product, including as to efficacy and safety, that are consistent with the Licensed Product labeling and accompanying inserts and the approved promotional materials.

(d) Bluebird will have the right, but not the obligation, to provide [\*\*\*] of the total sales representatives used by both Parties for promotion of Licensed Product. The Worldwide Commercialization Plan will set forth the precise number of Bluebird sales representatives consistent with the foregoing. If Bluebird is not at any particular time able to provide, for any reason, the number of sales representatives specified in the Worldwide Commercialization Plan, then Celgene will have the right to make up such shortfall using its sales representatives until such time as Bluebird is able to provide its agreed upon number of sales representatives. Bluebird will engage sales representatives having the minimum qualifications set forth in Schedule 5.6. [\*\*\*]

(e) Each Party will provide the JGC with a report, as soon as practicable but in no event later than forty-five (45) days following the end of each calendar quarter during the Term, setting forth the number of details made by its sales representatives of Licensed Product in the United States during such calendar quarter. Costs and expenses for sales representatives will be charged to the Profit & Loss Share on an FTE basis.

(f) Each Party will maintain records and otherwise establish procedures to ensure compliance with all applicable Laws and professional requirements that apply to the promotion and marketing of the Licensed Product, including compliance with the PhRMA Code on Interactions with Healthcare Professionals.

(g) Celgene will have sole authority to execute medical and scientific affairs and programs, including professional symposia and other educational activities, and medical affairs studies based upon approved protocols. Celgene will have sole authority over all medical affairs activities relating to the Licensed Product, including medical information support and medical communications and publishing activities. The Parties acknowledge that each Party may receive requests for medical information concerning the Licensed Product from members of the medical professions and consumers. Celgene will have the exclusive right to respond to questions and requests for information about the Licensed Product received from such Persons that (i) warrant a response beyond the understanding of the sales representatives or (ii) are beyond the scope of the Licensed Product labels and inserts (each such request, an “Information Request”). If Information Requests are received by Bluebird, the request will be referred to Celgene’s medical information department or appointed Third Party vendor to which Celgene has instructed Bluebird in writing to refer Information Requests.

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**6. Regulatory.**

6.1 Generally. Subject to Section 6.2 and the last sentence of Section 4.1, as of and after the CCPS Agreement Effective Date, subject to the terms and conditions of this CCPS Agreement, the Parties will assume through the JGC joint responsibility for all regulatory matters regarding seeking Regulatory Approval for Elected Candidate and Licensed Product for U.S. Administration, including interacting with Regulatory Authorities in connection therewith, before and after Regulatory Approval of Licensed Product. Celgene will have sole responsibility for all regulatory matters regarding seeking Regulatory Approval for Elected Candidate and Licensed Product for ROW Administration, including interacting with Regulatory Authorities in connection therewith, before and after Regulatory Approval of Licensed Product. Further:

(a) Prior to Regulatory Approval of Licensed Product for U.S. Administration, any such regulatory activities for Elected Candidate and such Licensed Product will be included in and will be part of the U.S. Development Plan (and thus subject to Section 4.2(a)) and the U.S. Development & Commercialization Program.

(b) Prior to Regulatory Approval of Licensed Product for ROW Administration, any such regulatory activities for Elected Candidate and such Licensed Product will be included in and will be part of the ROW Development Plan and the ROW Development & Commercialization Program.

(c) After any such Regulatory Approval for such Licensed Product for U.S. Administration, any such regulatory activities for U.S. Administration will be included in and will be part of the Worldwide Commercialization Plan and the U.S. Development & Commercialization Program.

(d) After any such Regulatory Approval for such Licensed Product for ROW Administration, any such regulatory activities for ROW Administration will be included in and will be part of the Worldwide Commercialization Plan and the ROW Development & Commercialization Program.

(e) Neither Party (itself or by or through any others, including any Affiliates or Sublicensees) will take any material action regarding any such regulatory activities unless described in the U.S. Development Plan, ROW Development Plan or the U.S. Commercialization Plan.

(f) Celgene will deploy and administer any REMS or other safety monitoring activity implemented for the Licensed Product, and be responsible for all pharmacovigilance activities for the Licensed Product.

6.2 Roles. Subject to Section 6.1, Celgene will take the lead and have final authority with respect to any regulatory activities for seeking Regulatory Approval for Elected Candidate and Licensed Product worldwide. Bluebird will have the right (i) to review and provide comments on all Regulatory Data, Regulatory Filings and Regulatory Approvals for U.S. Administration regarding such activities, which comments will be included if reasonable, and (ii) participate in all meeting with any Regulatory Authorities in the United States regarding such activities.

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6.3 Ownership. All Regulatory Filings for Elected Candidate and Licensed Product worldwide will be made by Celgene, in Celgene’s name, and all Regulatory Filings and Regulatory Approvals for Elected Candidate and Licensed Product worldwide will be solely owned by Celgene.

**7. Manufacture and Supply.**

7.1 Generally. As of and after the CCPS Agreement Effective Date, subject to the terms and conditions of this CCPS Agreement, (i) the Parties will assume through the JGC joint responsibility for (1) Manufacture of Elected Candidate and Licensed Product for Development and (2) Manufacture of Licensed Product for Commercialization for U.S. Administration, each under the Development & U.S. Commercialization Program, and (ii) Celgene will assume sole responsibility for Manufacturing Licensed Product for Commercialization for ROW Administration and, subject to Section 7.4, Celgene will purchase Vector Supply from Bluebird or its designee for such purpose.

7.2 Manufacturing for Development and Commercialization for U.S. Administration. Prior to Regulatory Approval of Licensed Product in any country, any Manufacturing activities for Development of Elected Candidate and such Licensed Product will be included in and will be part of the Worldwide Manufacturing Plan. After any such Regulatory Approval for such Licensed Product in the United States, any Manufacturing activities for Commercialization of Licensed Product for U.S. Administration will be included in and will be part of the U.S. Commercialization Plan and the U.S. Development and Commercialization Program. Neither Party (itself or by or through any others, including any Affiliates or Sublicensees) will take any material action regarding any such Manufacturing activities unless described in the Worldwide Manufacturing Plan or the U.S. Commercialization Plan, unless approved by the JGC.

7.3 Manufacturing for ROW Administration. Prior to Regulatory Approval of Licensed Product in any country in the ROW, Celgene will provide to the JGC a Manufacturing plan for the ROW in form and substance at least as detailed as the applicable section of the U.S. Commercialization Plan (including covering the applicable three-year time period) (the “ROW Post-Approval Manufacturing Plan”). Celgene (itself or by or through any others, including any Affiliates or Sublicensees) will not materially deviate from the then current ROW Post-Approval Manufacturing Plan when Manufacturing Licensed Product for Commercialization for ROW Administration without first notifying the JGC in writing and providing an updated ROW Post-Approval Manufacturing Plan.

7.4 Vector Manufacturing. Notwithstanding this Section 7:

(a) Generally. Bluebird will have the sole right to Manufacture Vector Supply for the Development and Commercialization of Elected Candidate and Licensed Product worldwide, and Celgene will have no rights with respect thereto except as provided in Section 7.4(b)(iv). Except as provided in Section 7.4(b)(iv) or in the Manufacturing Supply Agreement, neither Celgene nor any Affiliate of Celgene (nor any others on behalf of or under license or sublicense from Celgene or any of its Affiliates) will Manufacture (i) any Vector and associated Payload for Licensed Product or (ii) Licensed Product, except for the Manufacture of Licensed Product using Vector Supply supplied by or on behalf of Bluebird. Except as provided in Section 7.4(b)(iv) or in the Manufacturing Supply Agreement, Celgene and its Affiliates and Sublicensees will purchase all Vector Supply exclusively from Bluebird or its designee.

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(b) *Vector Supply Terms.*

(i) Except as provided in this Section 7.4(b)(iv) or in the Manufacturing Supply Agreement, Bluebird and its Affiliates will Manufacture, or cause a Third Party to Manufacture, all Vector Supply for all Elected Candidate and Licensed Product required for clinical Development and Commercialization in the Field worldwide, and will have the right to make all necessary decisions regarding arrangements with Third Party manufacturers, provided that Bluebird will reasonably consult with Celgene with respect to all such arrangements and obtain Celgene’s prior written consent, which will not be unreasonably withheld, conditioned or delayed. [\*\*\*]

(ii) The Parties will enter into a “Manufacturing and Supply Agreement,” between each other or among the Parties and an Affiliate or a Third Party, covering Vector Supply as soon as reasonably practicable after the CCPS Agreement Effective Date, which agreement will be consistent with and supersede the terms of this Section 7.4(b) and will otherwise be subject in all respects to the terms and conditions of this CCPS Agreement.

(iii) The cost to Celgene of Vector Supply for Commercialization for ROW Administration will equal [\*\*\*], unless otherwise agreed by the Parties in writing. The cost of Vector Supply for Commercialization for U.S. Administration will be included in the Cost of Goods Sold. The cost of Vector Supply for Development will be included in the U.S. Development Costs, subject to adjustment as provided therein.

(iv) The Manufacturing and Supply Agreement will include the terms set forth in Appendix I, including terms permitting Celgene to establish “back-up” and/or “second source” rights for Vector Supply and license grants from Celgene to Bluebird under the Celgene Licensed IP to the extent necessary or useful for Bluebird to Manufacture Vector Supply. [\*\*\*]

(v) At Celgene’s request, Bluebird will cooperate with Celgene’s reasonable requests, at Celgene’s cost and expense, to engage in a technology transfer to allow Celgene, in accordance with Section 7.4(b), to Manufacture Vector Supply (through the first commercial batch of Vector Supply) itself or by through its designated Third Party manufacturer, by transferring all Know-How, Materials, technology and trade secrets Controlled by Bluebird or its Affiliates that are necessary to Manufacture Vector Supply, thereby enabling Celgene (or such Third Party) to Manufacture the Vector Supply.

(vi) Any purchase of Vector Supply from Bluebird or its designee will expressly not include any license rights to any Know-How or Patents, but instead all licenses (implied, by exhaustion or otherwise) will arise under Section 10.1, if and as applicable.

(vii) For the purpose of this CCPS Agreement, certain words and phrases (and their correlatives) relating to Manufacturing will have the meanings set forth on Appendix D.

**8. Supporting Provisions for Development and Commercialization.**

8.1 Co-Co Licenses. In the event that through the JGC the Parties identify Patents, Know-How or Materials of a Third Party that are necessary to Develop and Commercialize Elected Candidate and Licensed Product worldwide, upon JGC recommendation, one or the

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other Party (or both) will use commercially reasonable efforts to obtain a license or other rights to such Patents, Know-How or Materials for use in connection with the performance of such Development and Commercialization (“Co-Co In-Licenses”). Prior to entering into any Co-Co In-License, the contracting Party will provide a draft copy to the other Party and the other Party will have the right to review and provide comments to such proposed Co-Co In-License. Neither Party will enter into a Co-Co In-License without the prior approval of the JGC, provided that Celgene will be free to enter into any Co-Co In-License for ROW Administration notwithstanding this Section 8.1. If a Party enters into any Co-Co In-Licenses during the CCPS Agreement Term, Appendix F hereto will be updated accordingly to include such Co-Co In-Licenses.

8.2 Records. Each Party will maintain, or cause to be maintained, records of its activities under this CCPS Agreement (including the Development & U.S. Commercialization Program) in sufficient detail and in good manner appropriate for research. Development, Commercialization, scientific, Patent and regulatory purposes, that will properly reflect all work included in the Development & U.S. Commercialization Program and under this CCPS Agreement, for a period of at least ten (10) years after the creation of such records. Each Party will have the right to request a copy of any such records.

8.3 Materials.

(a) Each Party will, during the CCPS Agreement Term, as a matter of course under the U.S. Development & Commercialization Program or ROW Development & Commercialization Program (collectively the “Development & U.S. Commercialization Program”) or upon the other Party’s reasonable written request, furnish to each other samples of Materials that are in such Party’s Control and are necessary for the other Party to carry out its responsibilities hereunder.

(b) Each Party will use such Materials only in accordance with the Development & U.S. Commercialization Program and otherwise in accordance with the terms and conditions of this CCPS Agreement and any instructions provided by the Party furnishing the Materials. Except with the prior written consent of the supplying Party (such consent not to be unreasonably withheld, delayed or conditioned), the Party receiving any Materials will not distribute or otherwise allow the release of Materials to any Affiliate (other than wholly-owned subsidiaries) or Third Party, except for subcontracting as permitted hereunder. All Materials delivered to the receiving Party will remain the sole property of the supplying Party and will be used in compliance with all applicable Law. The Materials supplied under this CCPS Agreement will be used with prudence and appropriate caution in any experimental work because not all of their characteristics may be known.

8.4 Permitted Subcontracting. Each Party may subcontract any of its activities to be performed under the Development & U.S. Commercialization Program to an Affiliate or Third Party, provided that any such Affiliate or Third Party will have entered into a written agreement with such Party that includes terms and conditions protecting and limiting use and disclosure of Confidential Information and Materials and Know-How at least to the same extent as under this CCPS Agreement, and requiring such Affiliate or Third Party and its personnel to assign to such Party all right, title and interest in and to any Patents, Know-How and Materials created,

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conceived or developed in connection with the performance of subcontracted activities to the extent required to research, Develop, Manufacture and Commercialize Elected Candidate and Licensed Product, provided that with respect to Third Parties that are academic or other non-commercial Persons, a Party will be required only to use commercially reasonable efforts to obtain such assignment. Any such subcontracting activities will be described in the reports for the Collaboration Program required by Section 8.5.

8.5 Reports. The Parties will prepare and provide to the other Party such reports regarding their activities under this CCPS Agreement as the JGC may reasonably require. In addition, each Party will disclose to the other Party information regarding those activities as such Party may reasonable request. Without limiting the foregoing, each Party will prepare and maintain, and will cause its Affiliates and Sublicensees to prepare and maintain, reasonably complete and accurate records regarding the Development of Elected Candidate and Licensed Product, and Commercialization of Licensed Product worldwide after Regulatory Approval therefor. Each Party will provide to the other Party a reasonably detailed report regarding such efforts at least once every calendar year (and more frequently if required by the JGC). Such report will contain sufficient detail to enable a Party to assess the other Party’s compliance with its Development and Commercialization obligations hereunder (including under the Development & U.S. Commercialization Program), including information with respect to the following: (i) the design, status and results of any animal studies and clinical trials for Licensed Product; (ii) any regulatory milestones, and any Regulatory Approvals achieved, for Licensed Product; and (iii) activities with respect to selling, promoting, supporting, detailing and marketing of Licensed Product.

**9. In-Licenses.**

**9.1 Applicable Bluebird In-Licenses and Other IP.**

(a) *Maintenance of Applicable Bluebird In-Licenses*. Bluebird (i) will duly perform and observe all of its obligations under the Applicable Bluebird In-Licenses in all material respects and maintain in full force and effect the Applicable Bluebird In-Licenses, and (ii) will not, without Celgene’s prior written consent (such consent not to be unreasonably withheld, conditioned or delayed), (1) amend, modify, restate, cancel, supplement or waive any provision of any Applicable Bluebird In-License, or grant any consent thereunder, or agree to do any of the foregoing, or (2) exercise any right to terminate any Applicable Bluebird In-License in each case ((1) and (2)) that would reasonably be expected to adversely affect in any respect the rights of Celgene under this CCPS Agreement, provided that Bluebird will provide prior written notice to Celgene of all of the foregoing notwithstanding whether or not any of the foregoing would reasonably be expected to adversely affect in any respect the rights of Celgene under this CCPS Agreement. Bluebird will provide Celgene with written notice as promptly as practicable (and in any event within five (5) business days) after becoming aware of any of the following: (A) any material breach or default by Bluebird or any of its Affiliates of any covenant, agreement or other provision of any Applicable Bluebird In-License, (B) any notice or claim from the counterparty to any Applicable Bluebird In-License terminating or providing notice of termination of any Applicable Bluebird In-License, (C) any notice or claim alleging any breach of default under any Applicable Bluebird In-License, or (D) the existence of any facts, circumstances or events which alone or together with other facts, circumstances or events would

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reasonably be expected (with or without the giving of notice or passage of time or both) to give rise to a breach of or default under or right to terminate any Applicable Bluebird In-License. If Bluebird fails to pay any amounts due under any Applicable Bluebird In-License and if such nonpayment would permit the counterparty to such Applicable Bluebird In-License to terminate or suspend the same or any rights thereunder, Celgene will have the right, but not the obligation, in its sole discretion, to pay such amounts on Bluebird’s behalf, and any amounts so paid by Celgene may be taken by Celgene as a credit against any amounts payable to Bluebird under this CCPS Agreement.

(b) *Maintenance of Co-Co In-Licenses*. The contracting Party to any Co-Co In-License (i) will duly perform and observe all of its obligations under the Co-Co In-License in all material respects and maintain in full force and effect the Co-Co In-License, and (ii) will not, without the other Party’s prior written consent (such consent not to be unreasonably withheld, conditioned or delayed), (1) amend, modify, restate, cancel, supplement or waive any provision of any Co-Co In-License, or grant any consent thereunder, or agree to do any of the foregoing, or (2) exercise any right to terminate any Co-Co In-License in each case ((1) and (2)) that would reasonably be expected to adversely affect in any respect the rights of the non-contracting Party under this CCPS Agreement, provided that the contracting Party will provide prior written notice to the non-contracting Party of all of the foregoing notwithstanding whether or not any of the foregoing would reasonably be expected to adversely affect in any respect the rights of the non-contracting Party under this CCPS Agreement. The contracting Party to any Co-Co In-License will provide the other Party with written notice as promptly as practicable (and in any event within five (5) business days) after becoming aware of any of the following: (A) any material breach or default by such contracting Party or any of its Affiliates of any covenant, agreement or other provision of the Co-Co In-License, (B) any notice or claim from the counterparty to the Co-Co In-License terminating or providing notice of termination of the Co-Co In-License, (C) any notice or claim alleging any breach of default under the Co-Co In-License, or (D) the existence of any facts, circumstances or events which alone or together with other facts, circumstances or events would reasonably be expected (with or without the giving of notice or passage of time or both) to give rise to a breach of or default under or right to terminate the Co-Co In-License. If the contracting Party to a Co-Co In-License fails to pay any amounts due under such Co-Co In-License and if such nonpayment would permit the counterparty to such Co-Co In-License to terminate or suspend the same or any rights thereunder, the other Party will have the right, but not the obligation, in its sole discretion, to pay such amounts on the other Party’s behalf, and any amounts so paid by such other Party may be taken by such other Party as a credit against any amounts payable to the other Party under this CCPS Agreement.

(c) [\*\*\*]

(d) *Applicable Bluebird In-License Requirements*. Celgene will abide, and will cause all its Affiliates and applicable Sublicensees to abide, by all requirements of each Applicable Bluebird In-License in all material respects (and in any case in all respects in the case that failure to so abide would result in a breach under the Applicable Bluebird In-License), to the extent applicable to sublicensees thereunder and to the extent disclosed by Bluebird to Celgene, with the understanding that disclosure by Bluebird of any Applicable Bluebird In-License to Celgene will be deemed disclosure of such requirements of such Applicable Bluebird In-License to

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Celgene. In the event of a termination of any Applicable Bluebird In-License, Bluebird agrees, to the extent requested by Celgene, to reasonably assist Celgene in securing a direct license from the applicable licensor under any Patents, Materials and Know-How that was licensed to Bluebird and sublicensed to Celgene hereunder prior to such termination. In addition, Bluebird agrees, if requested by Celgene, to reasonably assist Celgene in securing a standby license from the applicable licensor under any Patents, Materials and Know-How that are licensed to Bluebird and sublicensed to Celgene.

(e) *Applicable Co-Co In-License Requirements*. Each non-contracting Party to a Co-Co In-License will abide, and will cause all its Affiliates and applicable Sublicensees to abide, by all requirements of each such Co-Co In-License in all material respects (and in any case in all respects in the case that failure to so abide would result in a breach under the Co-Co In-License), to the extent applicable to sublicensees thereunder and to the extent disclosed by the contracting Party to the non-contracting Party, with the understanding that disclosure by the contracting Party of any Co-Co In-License to the non-contracting Party will be deemed disclosure of such requirements of such Co-Co In-License to the non-contracting Party. In the event of a termination of any Co-Co In-License, the contracting Party agrees, to the extent requested by the non-contracting Party, to reasonably assist the non-contracting Party in securing a direct license from the applicable licensor under any Patents, Materials and Know-How that was licensed to the contracting Party and sublicensed to the non-contracting Party hereunder prior to such termination. In addition, the contracting Party agrees, if requested by the non-contracting Party, to reasonably assist the non-contracting Party in securing a standby license from the applicable licensor under any Patents, Materials and Know-How that are licensed to the contracting Party and sublicensed to the non-contracting Party hereunder.

(f) [\*\*\*]

## **10. License Grants.**

10.1 Development and Commercialization Licenses by Bluebird. Subject to the terms and conditions of this CCPS Agreement, Bluebird hereby grants to Celgene:

(a) a co-exclusive (with Bluebird and its Affiliates) license, with the right to sublicense only as permitted by Section 10.3, under Bluebird Licensed IP and Bluebird Regulatory Rights, (i) to Develop (including for clarity Manufacture) Elected Candidate and Licensed Product for U.S. Administration and (ii) to Commercialize (including for clarity Manufacture) Licensed Product for U.S. Administration;

(b) a worldwide, exclusive (even as to Bluebird) license, with the right to sublicense only as permitted by Section 10.3, under Bluebird Licensed IP and Bluebird Regulatory Rights, (i) Develop (including for clarity Manufacture (other than Vectors)) Elected Candidate and Licensed Product for ROW Administration and (ii) to Commercialize (including for clarity Manufacture (other than Vectors)) Licensed Product for ROW Administration; and

(c) a worldwide, co-exclusive (with Bluebird and its Affiliates) license, with the right to sublicense only as permitted by Section 10.3, under Bluebird Licensed IP and Bluebird Regulatory Rights, to Manufacture Vectors and associated Payloads for Licensed Product for ROW Administration.



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Further, (i) the foregoing licenses to Bluebird Regulatory Rights include the right to reference same, (ii) the licenses to Commercialize granted in this Section 10.1 will cover only the sale and offer for sale of Licensed Product in finished form and not the sale or offer for sale of Vectors and associated Payloads (other than as and to the extent incorporated in the Licensed Product), and (iii) rights to Manufacture Vectors and associated Payloads are included within the scope of the licenses granted to Celgene under this Section 10.1, which rights are subject to the terms and conditions of Section 7.4(b).

#### 10.2 Development and Commercialization Covenant Not To Sue by Celgene.

(a) Subject to the terms and conditions of this CCPS Agreement, Celgene agrees that neither it nor its Affiliates will sue, assert any claim against, or otherwise participate in any action or proceeding against Bluebird or any of its Affiliates, sublicensees, contractors (including suppliers and manufacturers) or agents, or cause or authorize any Person to do any of the foregoing, under the Celgene Licensed IP and Celgene Regulatory Rights, with respect to Bluebird’s (i) Development (including for clarity Manufacture) of Elected Candidate and Licensed Product for U.S. Administration and (ii) Commercialization (including for clarity Manufacture) of Licensed Product for U.S. Administration, all as part of the Development & U.S. Commercialization Program; and (iii) Manufacture of Vectors and associated Payloads for Licensed Product for ROW Administration.

(b) Celgene will require that any Person that takes after the CCPS Agreement Effective Date any license or right in or to any Celgene Licensed IP and Celgene Regulatory Rights that is subject to the covenant not to sue in Section 10.2(a) is subject to the covenants not to sue set forth in this Section 10.2.

For clarity, (i) the foregoing covenants not to sue regarding Celgene Regulatory Rights includes the right to reference same, (ii) such covenants not to sue with respect to the Commercialization granted in this Section 10.2 will cover only the sale and offer for sale of Licensed Product in finished form, and (iii) Manufacture of Vectors and associated Payloads is included within the scope of the covenants not to sue granted to Bluebird under this Section 10.2.

#### 10.3 Licensing and Sublicensing Rights.

(a) *Transfer.* The licenses and covenants granted in Sections 10.1 and 10.2 are transferable only upon a permitted assignment of this CCPS Agreement in accordance with Section 13.13.

(b) *Other Licenses.* Either Party can grant licenses to its own Licensed IP to its Affiliates and other Third Parties, subject to the terms of this CCPS Agreement (including the exclusivity and co-exclusivity provided for in the licenses granted in Sections 10.1 and 10.2).

(c) *Sublicenses.* The licenses and covenants granted in Sections 10.1 and 10.2 may be sublicensed, in full or in part, by the licensee Party by a written agreement to its Affiliates and Third Parties (with the right to sublicense through multiple tiers), provided, that as a condition precedent to and requirement of any such sublicense:

(i) Celgene will obtain Bluebird’s written consent prior to granting to a Third Party any sublicense of the licenses granted by Bluebird in Section 10.1 with respect to the Development or Commercialization of Licensed Product for U.S. Administration (such consent not to be unreasonably withheld, delayed or conditioned).

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(ii) Bluebird will obtain Celgene’s written consent prior to granting to a Third Party any sublicense of the covenant not to sue granted by Celgene in Section 10.2, or any other right to license, with respect to the Development or Commercialization of Licensed Product for U.S. Administration (such consent not to be unreasonably withheld, delayed or conditioned).

(iii) The licensee Party will provide the licensor Party with a copy of any sublicense agreement with a non-Affiliated Sublicensee within thirty (30) days of execution thereof, and to the extent permitted under any Applicable Bluebird In-License, such sublicense agreement may be redacted as necessary to protect commercially sensitive information;

(iv) The licensor Party will be responsible for any and all obligations of such Sublicensee as if such Sublicensee were such licensee Party hereunder;

(v) Any such Sublicensee will agree in writing to be bound by substantially identical obligations as such licensee Party hereunder with respect to the activities of such Sublicensee hereunder (and not with respect to the activities of any other), including any Know-How disclosure obligations such licensee Party has to the licensor Party hereunder with respect to the activities of such Sublicensee hereunder (but excluding payment obligations); and

(vi) The licensor Party will be made an express third-party beneficiary of any such Sublicensee’s obligations under such sublicense agreement that relate to compliance with the terms and conditions of this CCPS Agreement.

10.4 Exclusivity. During the CCPS Agreement Term, neither Party nor its Affiliates (nor any others on behalf of or with, or under license or sublicense from, such Party or any of its Affiliates) will research, Develop, Manufacture or Commercialize any products (including Vectors and associated Payloads) to be used in the Field (which, for the purposes of this Section 10.4, will include all indications and will not be limited to cancer) that specifically target the same Target Antigen as Elected Candidate, other than pursuant to this CCPS Agreement (which includes, for avoidance of doubt, research, development, Manufacture and Commercialization of improved and modified versions of the Licensed Product by Celgene) or any other Development & U.S. Commercialization Agreement (if against the same Target Antigen) (which includes, for avoidance of doubt, research, development, Manufacture and Commercialization of improved and modified versions of the Licensed Product pursuant to this CCPS Agreement). Notwithstanding this Section 10.4, if (i) a Business Combination occurs with respect to either Party with a Third Party or (ii) a Party acquires a Third Party (including by a merger or consolidation) so that such Third Party becomes an Affiliate over which the acquiring Party has control (as defined in the definition of Affiliate), or (iii) a Party acquires all or substantially all of the assets of a Third Party (including any Subsidiaries or divisions thereof) (each of (i), (ii) and (iii), a “Business Acquisition”; such Party, the “Business Party”), and, in each case, the Third Party (or any of such Third Party’s Affiliates or any successors or assigns of such Third Party or such Third Party’s Affiliates, other than the Business Party and its Affiliates as of the Business Acquisition) (a) already has, or the acquired assets contain, as applicable, a program that existed prior to, or was planned prior to and is demonstrably to be implemented shortly after, the Business Acquisition or (b) initiates and pursues a new program following such Business Acquisition, in each case that would otherwise violate this Section 10.4 (a “Business Program”),

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then such Third Party (or any of such Third Party’s Affiliates or any successors or assigns of such Third Party or such Third Party’s Affiliates, other than the Business Party and its Affiliates as of the Business Acquisition), as applicable, will be permitted to initiate, pursue and continue such Business Program after such Business Acquisition and such initiation, pursuit and continuation will not constitute a violation of this Section 10.4; provided however that (A) none of the Bluebird Licensed IP or Celgene Licensed IP, as the case may be, or other Patents, Materials or Know-How Controlled by the other Party and, in each case, licensed to the Business Party will be used in the Business Program, and (B) the research or Development activities required under this CCPS Agreement will be conducted separately from any research or Development activities directed to such Business Program, including the maintenance of separate lab notebooks and records (password-protected to the extent kept on a computer network) and separate personnel working on each of the activities under this CCPS Agreement and the activities covered under such Business Program

10.5 Contract Manufacturers. Subject to the terms and conditions of this CCPS Agreement, either Party will have the right to appoint by a written agreement “contract manufacturers”, meaning any Third Party or Affiliate of such Party that Manufactures Licensed Product (or components thereof, including for Bluebird, Vectors and associated Payloads) for re-sale, but who itself is not a “Sublicensee” hereunder and thereby exercises “have made” rights granted by the other Party under Section 10.1 or Section 10.2, as applicable, as well as “contract research organizations” and other providers performing services on a Party’s behalf, none of which will be deemed a “Sublicensee” hereunder. Such Party will be responsible for any such contract manufacturer, contract research organization or service provider hereunder, and further will require any such contract manufacturer, contract research organization or service provider to agree in writing to comply with Sections 4.4 and 15.

10.6 No Implied Rights. No license, sublicense or other right is or will be created or granted hereunder by implication, estoppel or otherwise. Any licenses, sublicenses or rights will be granted only as expressly provided in this CCPS Agreement. Neither Party will practice or otherwise use any Licensed IP of the other Party other than in accordance with the licenses granted in Section 10.1 and Section 10.2, as applicable.

10.7 Additional IP; Other In-Licenses.

(a) *Additional IP*. Except as set forth in Section 10.7(b), Celgene may, on or after the CCPS Agreement Effective Date, elect to include within the scope of the Bluebird Licensed IP any Know-How, Material, Patent, Regulatory Data, Regulatory Filings or Regulatory Approvals (“Additional Bluebird IP”), that would be Controlled by Bluebird but for required payments of Additional Payments to a Third Party, by (i) providing notice to Bluebird of same and (ii) agreeing to pay and in fact paying all Additional Payments with respect to Celgene’s access or license to such Additional Bluebird IP. Following Bluebird’s receipt of such notice and subject to Celgene’s performance of its obligations to pay any Additional Payments with respect to Celgene’s access or license to such Additional Bluebird IP, such Additional Bluebird IP will be deemed Bluebird Licensed IP hereunder. For avoidance of doubt, this Section 10.7(a) does not apply to Know-How, Materials, Patents, Regulatory Data, Regulatory Filings or Regulatory Approvals licensed to Bluebird under the Applicable Bluebird In-Licenses, all of which are deemed Controlled by Bluebird notwithstanding the terms of this Section 10.7(a).

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(b) *Other In-Licenses*. Celgene may, on or after the CCPS Agreement Effective Date, elect to convert any Other In-License to an Applicable New In-License by providing notice to Bluebird of same. Upon Bluebird’s receipt of such notice, such Other In-License will be an Applicable New In-License hereunder, Appendix B will automatically be updated to include such New In-License and the provisions of this CCPS Agreement applicable to New In-Licenses, including Section 11.1, will apply with respect to such Other In-License.

10.8 Section 365(n) of the Bankruptcy Code. All rights and licenses granted pursuant to any section of this CCPS Agreement are, and will be deemed to be, rights and licenses to “intellectual property” (as defined in Section 101(35A) of title 11 of the United States Code and of any similar provisions of applicable Laws under any other jurisdiction (the “Bankruptcy Code”). Each Party agrees that the other Party, as a licensee of rights and licenses under this CCPS Agreement, will retain and may fully exercise all of its rights and elections under the Bankruptcy Code. The Parties further agree that, in the event of the commencement of a bankruptcy proceeding by or against a Party under the Bankruptcy Code or analogous provisions of applicable Law outside the United States, the other Party will be entitled to a complete duplicate of (or complete access to, as appropriate) any intellectual property licensed to it and all embodiments of such intellectual property, which, if not already in its possession, will be promptly delivered to it (a) upon any such commencement of a bankruptcy proceeding upon such other Party’s written request therefor, unless the Party involved in the bankruptcy proceeding elects to continue to perform all of its obligations under this CCPS Agreement or (b) if not delivered under clause (a), following the rejection of this CCPS Agreement by the Party in the bankruptcy proceeding upon written request therefor by the other Party.

## **11. Payments and Royalties.**

### **11.1 Payments for In-Licenses.**

(a) *United States*. With respect to the Development and Commercialization of Elected Candidate and Licensed Product for U.S. Administration hereunder, if any payments become due under any Applicable Pre-Existing In-License, Applicable New In-Licenses, Co-Co In-Licenses or Celgene Licensed Product In-License during the CCPS Agreement Term, the contracting Party thereto will pay same and such payment will be treated as U.S. Development Expenses or Allowable Expenses, as appropriate, provided [\*\*\*]

(b) *ROW*. With respect to the Development and Commercialization of Elected Candidate and Licensed Product for ROW Administration hereunder (including the Manufacture of Vectors and associated Payloads therefor pursuant to Section 7.4):

(i) *Applicable Pre-Existing In-Licenses*. If any In-License Payment becomes due under any Applicable Pre-Existing In-License during the CCPS Agreement Term, Bluebird will pay same, provided that Celgene will reimburse Bluebird for any such In-License Payment applicable to ROW Administration within thirty (30) days of Celgene’s receipt of Bluebird’s written invoice therefor, which In-License Payments (other than payments that are royalties) will not exceed [\*\*\*], and subject to Section 13.1. Any such reimbursement by Celgene to Bluebird (1) is in addition to and not in lieu of the other payments required by this Section 11 and (2) will not be subject to Section 11.3(d).

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(ii) *Applicable New In-Licenses*. Celgene may elect to take a sublicense under any New In-License of Bluebird or its Affiliates and upon such election, such New In-License will be an Applicable New In-License hereunder for all purposes. For the purposes of determining the Parties’ respective payment obligations, all Applicable New In-Licenses as of and following the CCPS Agreement Effective Date will be listed on Appendix B. If any In-License Payment becomes due under any Applicable New In-License during the CCPS Agreement Term with respect to ROW Administration, Bluebird will pay same and, subject to Section 13.1, Celgene will reimburse Bluebird for (i) [\*\*\*] of such payment that are royalties, which royalties will be subject to Section 11.3(d), and (ii) [\*\*\*] of such payment that are not royalties, in each case ((i) and (ii)) within thirty (30) days of receipt of Bluebird’s written invoice therefor. If Celgene elects to convert an Other In-License to an Applicable In-License pursuant to Section 10.7(b), Celgene will reimburse Bluebird for [\*\*\*] of any In-License Payments that became due under such Applicable New In-License during the CCPS Agreement Term with respect to ROW Administration to the same extent as if such Applicable New In-License was designated as such as of the CCPS Agreement Effective Date, including with respect to applicable Patent Costs in accordance with Section 6.1, provided that Bluebird provides Celgene with a reasonable accounting of same. If any In-License Payments are royalties due under any Applicable New In-License during the CCPS Agreement Term with respect to Licensed Product for ROW Administration, such royalties will be subject to Section 11.3(d). To the extent that any grant of a sublicense by Celgene or any Sublicensees under an Applicable New In-License triggers a payment obligation under such Applicable New In-License, Bluebird will pay same and Celgene will reimburse Bluebird for [\*\*\*] of such payment within thirty (30) days of receipt of Bluebird’s written invoice therefor. To the extent that any grant of a sublicense by Bluebird or any Sublicensees under a Celgene Licensed Product In-License triggers a payment obligation under such Celgene Licensed Product In-License, Celgene will pay same and Bluebird will reimburse Celgene for [\*\*\*] of such payment within thirty (30) days of receipt of Celgene’s written invoice therefor.

(iii) If any payments become due under any Co-Co In-Licenses during the CCPS Agreement Term with respect to Licensed Product for ROW Administration, the contracting Party will pay same, and further if Bluebird is the contracting Party, Celgene will reimburse Bluebird for such payment within thirty (30) days upon receipt of Bluebird’s written invoice therefor, subject to Section 13.1. Any such reimbursement by Celgene to Bluebird (1) is in addition to and not in lieu of the other payments required by this Section 11 and (2) will not be subject to Section 11.3(d). If any payments are royalties due under any Co-Co In-License during the CCPS Agreement Term with respect to Licensed Product for ROW Administration, such royalties will be subject to Section 11.3(d).

(iv) If any payments become due under any Celgene Licensed Product In-License with respect to Licensed Product for ROW Administration, Bluebird will be responsible for [\*\*\*] of such payments as provided in Section 4.1(e) of the Master Collaboration Agreement, provided that if any such payments are royalties with respect to Licensed Product for ROW Administration, such royalties will be subject to Section 11.3(d).

(c) [\*\*\*]

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11.2 Milestone Payments.

(a) *Generally.* Celgene will make milestone payments (each, a “Milestone Payment”) to Bluebird upon the occurrence of each of the milestones events (each, a “Milestone Event”) as set forth below in this Section 11.2. Each of the Milestone Payments will be payable to Bluebird by Celgene within forty-five (45) days of the achievement of the specified Milestone Event, and such payments when owed or paid will be non-refundable and non-creditable, and not subject to set-off, except as otherwise set forth in Sections 4.3(c), 9.1(a), 9.1(b), 17.3(c) and 17.6 hereof or Sections 4.1(e), 4.3 and 12.6 of the Master Collaboration Agreement. Except with respect to Modified Licensed Products, each of the Milestone Payments are payable only once in total under this CCPS Agreement, whether achieved by one or more Licensed Products. Notwithstanding the foregoing, Bluebird will be entitled to receive [\*\*\*] of the Milestone Payments below, other than the Milestone Payment for the first Milestone Event ([\*\*\*]), for [\*\*\*] for each new Modified Licensed Product.

(b) *Development Milestones.*

<u>Milestone Event</u>	<u>Milestone Payment</u>
[***]	
[***]	

11.3 Royalties for Licensed Product for ROW Administration.

(a) *Rates.* Subject to the remainder of this Section 11.3, Celgene will pay to Bluebird running royalties, on a Licensed Product-by-Licensed Product basis, based on the total aggregate annual Net Sales by Selling Parties of such Licensed Product for ROW Administration in a given calendar year based on the Royalty Rate in the table set forth below.

<u>Annual Net Sales of Each Licensed Product for ROW Administration</u>	<u>Royalty Rate</u>
[***]	

By way of example, in a given calendar year, if the aggregate annual Net Sales for a Licensed Product for ROW Administration is [\*\*\*] the following royalty payment would be payable for those Net Sales under this Section 11.3(a): [\*\*\*]

The Parties acknowledge and agree that for the purposes of calculating royalties under this Section 11.3(a), the country of sale for Licensed Product will be deemed to be the country in which such Licensed Product is administered to a patient.

(b) *Royalty Term.* Royalties under Section 11.3(a) will be payable, on a Licensed Product-by-Licensed Product and country-by-country basis, on the Net Sales of any Licensed Product for ROW Administration if at least one of the following two (2) conditions apply:

(i) if one or more Valid Claims within any of Patents included within the Bluebird Licensed IP Covers in such country such Licensed Product for ROW Administration; or

(ii) [\*\*\*]

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(c) *Royalty Reduction*. If Licensed Product is royalty-bearing only on account of Section 11.3(b)(ii), then the royalty rates set forth in Section 11.3(a) with respect to Net Sales attributable to Licensed Product will be reduced by [\*\*\*]

(d) *Third Party Royalty Payments – ROW Administration*. As provided in Section 11.1(b), if Celgene (or its Sublicensee) is required to pay to a Third Party under any New In-License or Co-Co License or any Celgene Licensed Product In-License, any royalties for Commercialization of Licensed Product for ROW Administration, or if Celgene or its Sublicensee, in its reasonable judgment, is required to obtain a license from any Third Party under any Patent Covering Licensed Product in order to Develop or Commercialize such Licensed Product for ROW Administration, and if Celgene (or its Sublicensee) is required to pay to such Third Party under such license any royalties, and the infringement of such Patent cannot reasonably be avoided by Celgene or its Sublicensee, or if Celgene (or its Sublicensee) is required by a court of competent jurisdiction to pay royalties or lost profits to a Third Party based on a Patent as a result of the such Commercialization (and the infringement of such Patent cannot reasonably be avoided by Celgene or its Sublicensee), then the amount of Celgene’s royalty obligations under this Section 11.3 will be reduced by [\*\*\*] of the amount of such royalties paid to such Third Party, provided however, that the royalties payable under Section 11.3(a) will not be reduced in any such event below [\*\*\*] of the amounts set forth in Section 11.3(a) (but as may be further reduced pursuant to Section 11.3(c) or 11.3(e)) for each royalty tier. Any royalties payable under any Applicable Pre-Existing In-Licenses may not be deducted under this Section 11.3(d) from royalties owed to Bluebird. Any royalties payable under any Applicable New In-Licenses, Celgene Licensed Product In-Licenses and Co-Co Licenses may be deducted under this Section 11.3(d) from royalties owed to Bluebird. Celgene (or its Sublicensee) will use its commercially reasonable efforts to minimize the amount of any of the foregoing payments owed to Third Parties. Prior to Celgene or its Sublicensee exercising its reasonable judgment under this Section 11.3(d), Celgene will provide Bluebird with written notice of a potential need to obtain any license from Third Parties. The Parties will discuss the best course of action to resolve such potential license requirement(s). For clarity, the Parties acknowledge and agree that, notwithstanding anything in this CCPS Agreement to the contrary, no royalties or other amounts payable by Celgene (or its Sublicensee) to a Third Party with respect to Licensed Product for U.S. Administration may act to reduce the amount of Celgene’s royalty obligations under this Section 11.3.

(e) [\*\*\*]

(f) *Additional Royalty Provisions*. The royalties payable under Section 11.3(a) will be subject to the following:

(i) only one royalty will be payable hereunder with respect to each Licensed Product unit;

(ii) royalties when owed or paid hereunder will, except as provided in Section 11.3(b), be non-refundable and non-creditable and not subject to set-off, except as otherwise provided in [\*\*\*] 9.1(b), 17.3(d) and 17.6 hereof or Sections 4.1(e), 4.3 and 12.6 of the Master Collaboration Agreement; and

(iii) except as expressly set forth in Section 11.3(c), Section 11.3(d) and Section 11.3(e), no other royalty deductions are permitted hereunder

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11.4 Profit & Loss Share for Licensed Product for U.S. Administration. The Parties will share in Operating Profit or Loss with respect to Licensed Product for U.S. Administration as follows: Bluebird will bear (and be entitled to) fifty percent (50%), and Celgene will bear (and be entitled to) fifty percent (50%) (the “Profit & Loss Share”). Procedures for calendar quarterly reporting of actual results and review and discussion of potential discrepancies, quarterly reconciliation, reasonable forecasting, and other finance and accounting matters, are set forth in Appendix F, and to the extent not set forth in Appendix F, will be established by the JGC, subject to Section 11.5(e).

11.5 Payment Terms for Milestones and Royalties Due Hereunder. [\*\*\*]

11.6 *Mutual Convenience of the Parties*. The royalty and other payment obligations set forth hereunder have been agreed to by the Parties for the purpose of reflecting and advancing their mutual convenience, including the ease of calculating and paying royalties and other amounts to Bluebird.

## **12. Ownership and Inventorship of IP.**

12.1 Solely-Owned IP. Subject to Section 12.2, as between the Parties, each Party will own and retain all right, title and interest in and to any and all Know-How and Patents arising therefrom that are discovered, created, conceived, developed or reduced to practice solely by or on behalf of such Party under or in connection with this CCPS Agreement, including as part of the Development & U.S. Commercialization Program (“Solely Owned IP”). Subject to the licenses hereunder and the other terms and conditions of this CCPS Agreement, each Party will be solely responsible for the Prosecution and Maintenance, and the enforcement and defense, of any Patents within its Solely Owned IP, and the other Party will have no rights with respect thereto.

12.2 Joint IP. The Parties will jointly own any and all Know-How and Patents arising therefrom that are discovered, created, conceived, developed or reduced to practice jointly by or on behalf of the Parties under or in connection with this CCPS Agreement, including as part of the Development & U.S. Commercialization Program (“Joint IP”). Each Party will have an undivided one-half interest in and to Joint IP. Each Party will exercise its ownership rights in and to such Joint IP, including the right to license and sublicense or otherwise to exploit, transfer or encumber its ownership interest, without an accounting or obligation to, or consent required from, the other Party, but subject to the licenses hereunder and the other terms and conditions of this CCPS Agreement, including Section 10.4. At the reasonable written request of a Party, the other Party will in writing grant such consents and confirm that no such accounting is required to effect the foregoing regarding Joint IP. Each Party, for itself and on behalf of its Affiliates, licensees and sublicenses, and employees, subcontractors, consultants and agents of any of the foregoing, hereby assigns (and to the extent such assignment can only be made in the future hereby agrees to assign), to the other Party a joint and undivided interest in and to all Joint IP. The Prosecution and Maintenance, and the enforcement and defense, of any Patents within Joint IP will be jointly managed by the Parties on mutually agreeable terms to be entered into by the Parties at the time any such Patents are first filed, provided that (i) all recoveries and Patent Costs arising from the enforcement or defense of any Patents within Joint IP, absent further



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agreement, will be shared by the Parties in accordance with Section 14.2 (provided that sufficient advance written notice of any such Patent Costs is given to the Party not incurring same) and (ii) Patent Costs incurred in connection with the Prosecution and Maintenance of Patents within Joint IP will be apportioned as set forth in Sections 13.1 and 13.3, provided that in each case ((i) and (ii)), and all recoveries and Patent Costs arising from those activities, absent further agreement, will be shared equally by the Parties (provided that sufficient advance written notice of any such Patent Costs is given to the Party not incurring same), provided that if either Party elects not to pay any such Patent Costs for any such Patent, the Parties will meet and agree upon an equitable way to treat such Patent.

12.3 Inventorship. Inventorship determination for all Patents worldwide arising from any Know-How discovered, created, conceived, developed or reduced to practice by or on behalf of the Parties under or in connection with this CCPS Agreement and thus the ownership thereof will be made in accordance with applicable United States patent Laws.

12.4 Allocation. Notwithstanding Sections 12.1 – 12.3, the Patent Committee may allocate ownership of a particular item of intellectual property to improve the prospects of obtaining patent protection with respect to such item of intellectual property, even if such allocation is not in accordance with the terms of Sections 12.1 – 12.3, so long as the Parties mutually agree to such allocation.

### **13. Patent Prosecution and Maintenance.**

13.1 Generally. Subject to Sections 13.2 and 13.3, each Party will have the sole right to Prosecute and Maintain Patents within its respective Licensed IP. Bluebird will use commercially reasonable efforts to, where applicable and permitted under applicable Law and upon Celgene’s reasonable request, separate parent Patent applications within the Bluebird Licensed IP into one or more separate Patent applications for Specific Patents, where doing so would not reasonably be expected to materially harm any Patent within the Bluebird Licensed IP or other Patents owned by Bluebird or its Affiliates, provided that the foregoing limitation will not apply to Bluebird Licensed IP that is Collaboration IP. [\*\*\*]

13.2 Input. Each Party will regularly provide the other with copies of all applications for Patents within its respective Licensed IP, and all other material submissions and correspondence with any patent authorities regarding such Patents, in sufficient time to allow for review and comment by the other Party. In addition, each Party will provide the other Party and its counsel with an opportunity to consult with such Party and its counsel regarding Prosecution and Maintenance of any such Patents within the Field, and such Party will consider in good faith all such comments timely made by such other Party and its counsel. In the event of any disagreement between the Parties, the licensor Party will have the final decision-making authority with respect to the matter involved as long as the licensor Party acts in good faith.

13.3 Specific Patents. For any Patent within the Bluebird Licensed IP [\*\*\*] (each “Specific Patent”), the following will apply: upon Celgene’s written request, and provided that Bluebird reasonably agrees with Celgene that the following Prosecution and Maintenance activities would not materially harm any other Patent within the Bluebird Licensed IP or other Patents owned by Bluebird or its Affiliates (other than Collaboration IP), Celgene will control the Prosecution and Maintenance of the Specific Patents, and notwithstanding anything in

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Section 13.1 to the contrary, Celgene will be solely responsible for the payment of all related Patent Costs. In addition, Celgene will provide Bluebird and its counsel with an opportunity to consult with Celgene and its counsel regarding Prosecution and Maintenance of any such Specific Patents, and Celgene will include or reflect all reasonable comments timely made by Bluebird and its counsel. Celgene acknowledges and agrees that Bluebird may grant similar rights to other exclusive Third Party licensees under any Patent within the Bluebird Licensed IP that has claims Covering only a product that is not a Licensed Product (or its manufacture or use) and no other product (or its manufacture or use), other than Specific Patents. If the Parties cannot agree whether or not any Patent within the Bluebird Licensed IP is a Specific Patent, or if Bluebird claims that the foregoing Prosecution and Maintenance activities would materially harm any other Patent within the Bluebird Licensed IP or other Patents owned by Bluebird or any of its Affiliates, either of the Parties may refer such dispute to a mutually agreeable, disinterested, conflict-of-interest-free individual not affiliated or consulting with either Party and who has at least fifteen (15) years of patent prosecution experience in the pharmaceutical field. Any such arbitration will be conducted under the then-current rules of the American Arbitration Association, and the decision of the arbitrator will be final.

13.4 Election Not to Prosecute or Maintain or Pay Patent Costs. If a Party elects not (i) to Prosecute or Maintain any Patents within its respective Licensed IP in any particular country before the applicable filing deadline or continue such activities once filed in a particular country, or (ii) to pay the Patent Costs associated with Prosecution or Maintenance of any Patents within the Licensed IP as required by Section 13.1, then in each such case such first Party will so notify the other Party, promptly in writing and in good time to enable any deadlines by which an action must be taken to preserve such Patent in such country to be met. Upon receipt of each such notice by such first Party, such other Party will have the right, but not the obligation, to notify such first Party in writing on a timely basis that such other Party will continue the Prosecution or Maintenance of such Patent on terms the Parties shall mutually agree; it being understood that only U.S. Patents controlled by Celgene will be subject to this sentence. Notwithstanding the foregoing, upon receipt of each such notice by Bluebird, Celgene will have the right, but not the obligation, to notify Bluebird in writing on a timely basis that Celgene will assume control of the Prosecution or Maintenance of such Patent within the Bluebird Licensed IP, and bear the Patent Costs thereafter incurred by Celgene with respect thereto. In addition, Celgene will provide Bluebird and its counsel with an opportunity to consult with Celgene and its counsel regarding Prosecution and Maintenance of any such Patents, and Celgene will include or reflect all reasonable comments timely made by Bluebird and its counsel. If after making such election, Celgene elects not to pay the Patent Costs associated with Prosecution or Maintenance of any such Patent, then in each such case Celgene will so notify Bluebird and on the ninetieth (90<sup>th</sup>) day after Bluebird’s receipt of such notice such Patent will no longer be licensed to Celgene hereunder and will no longer be included within the “Bluebird Licensed IP” hereunder.

13.5 Third Party Rights. To the extent that a Third Party licensor of a Party has retained any right to Prosecute or Maintain any Patent within such Party’s Licensed IP licensed to the other Party hereunder, or otherwise be involved in such activities, such Party will use commercially reasonable efforts to cause such Third Party licensor to take the actions specified by this Section 13 (including Sections 13.6 and 13.7) in a manner consistent with the in-license applicable thereto, but such Party will not be deemed to be in breach of its obligations under this Section 13 if, after using such commercially reasonable efforts, it is unable to comply with such obligations because of actions taken or not taken by such Third Party licensor.

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13.6 Patent Extensions. Subject to the remainder of this Section 13.6, if any election for patent term restoration or extension, supplemental protection certificate or any of their equivalents may be made with respect to any Patent within the Licensed IP, after consultation through the JGC. If the Parties are not able to reach mutual agreement, (i) Celgene will have the sole right to make the final decision whether or not to seek such patent term restoration or extension, supplemental protection certificate or any of their equivalents with respect to Specific Patents and Patents within the Collaboration IP licensed to Celgene hereunder and the Celgene Licensed IP, and (ii) Bluebird will have the sole right to make the final decision whether or not to seek such patent term restoration or extension, supplemental protection certificate or any of their equivalents with respect to all other Patents within the Bluebird Licensed IP.

13.7 Regulatory Exclusivity Periods. With respect to any Patent listings required for any Regulatory Exclusivity Periods for Product, the Parties will mutually agree on which Patents within the Licensed IP to list, provided that if the Parties are not able to agree, Celgene will have the right to make the final decision, and provided further that the exercise of such right by Celgene will not increase or otherwise change the rights or obligations of the Parties hereunder.

13.8 Cooperation. Each Party will reasonably cooperate with the other Party in the Prosecution and Maintenance of Patents within the Licensed IP. Such cooperation includes promptly executing all documents, or requiring inventors, subcontractors, employees and consultants and agents of such Party and its Affiliates and Sublicensees to execute all documents, as reasonable and appropriate so as to enable the Prosecution and Maintenance of any such Patents in any country.

13.9 Patent Marking. For Licensed Product for U.S. Administration, the JGC will determine the Patent marking requirements in accordance with applicable Law. For Licensed Product for ROW Administration, Celgene will mark, and will cause all other Selling Parties to mark, Product with all Patents within the Bluebird Licensed IP in accordance with applicable Law, which marking obligation will continue for as long as (and only for as long as) required under applicable Law.

13.10 Common Interest Disclosures. With regard to any information or opinions disclosed pursuant to this CCPS Agreement by one Party to the other Party regarding Prosecution and Maintenance of Patent within the Licensed IP, or enforcement of intellectual property and/or technology by or against Third Parties, Bluebird and Celgene agree that they have a common legal interest in determining the ownership, scope, validity and/or enforcement of the Licensed IP, and whether, and to what extent, Third Party intellectual property rights may affect the conduct of the Development and Commercialization of any Licensed Product, and have a further common legal interest in defending against any actual or prospective Third Party claims based on allegations of misuse or infringement of intellectual property rights relating to the Development or Commercialization of any Licensed Product. Accordingly, the Parties agree that all such information and materials obtained by the Parties from each other will be used solely for purposes of the Parties' common legal interests with respect to the conduct of the Agreement. All such information and materials will be treated as protected by the attorney-client privilege.

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the work product privilege, and any other privilege or immunity that may otherwise be applicable. By sharing any such information and materials, neither Party intends to waive or limit any privilege or immunity that may apply to the shared information and materials. Neither Party will have the authority to waive any privilege or immunity on behalf of the other Party without such other Party’s prior written consent, nor will the waiver of privilege or immunity resulting from the conduct of one Party be deemed to apply against any other Party. This Section 13.10 will be subject to any right granted by either Party to any Third Party, provided that the grant of such right to such Third Party does not conflict with the other Party’s rights or the first Party’s obligations under this CCPS Agreement.

**14. Patent Enforcement and Defense.**

14.1 Notice. Each Party will promptly notify, in writing, the other Party upon learning of any actual or suspected Competitive Infringement of any Patents within the Licensed IP by a Third Party, or of any claim of invalidity, unenforceability, or non-infringement of any Patents within the Licensed IP, and will, along with such notice, supply the other Party with any evidence in its possession pertaining thereto. For purposes of this CCPS Agreement, “Competitive Infringement” means any allegedly infringing activity in the Field (which, for the purposes of this definition, will include all indications and will not be limited to cancer) with respect to a Patent within the Licensed IP, which activity (i) falls within the scope then in effect of the licenses granted by Bluebird to Celgene as set forth in Sections 10.1 and 10.2, (ii) is subject to Section 14.2(f), or (iii) would be competitive with a Licensed Product and targets the same Target Antigen as such Licensed Product.

14.2 Enforcement and Defense. [\*\*\*]

**15. Confidentiality.**

The Parties acknowledge and agree that terms of this CCPS Agreement and all Materials, ideas and information of any kind, whether in written, oral, graphical, machine-readable or other form, whether or not marked as confidential or proprietary, which are transferred, disclosed or made available by a Party or at the request of a Party, including any of the foregoing of Third Parties, will be subject to the provisions of Section 10 of the Master Collaboration Agreement. The Parties agree to issue the joint press release on Appendix G promptly following the CCPS Agreement Effective Date. A redacted version of this CCPS Agreement is attached hereto as Appendix M.

**16. Warranties; Limitations of Liability; Indemnification.**

16.1 Representations and Warranties. Each Party represents and warrants to the other as of the CCPS Agreement Effective Date that it has the legal right and power to enter into this CCPS Agreement, to extend the rights and licenses granted or to be granted to the other in this CCPS Agreement, and to fully perform its obligations hereunder.

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16.2 Additional Representations and Warranties of Bluebird. Except as set forth in Schedule 16.2, Bluebird represents and warrants to Celgene that, as of the CCPS Agreement Effective Date:

(a) *Licensed IP*. Appendix H sets forth a complete and accurate list of all Patents included in the Bluebird Licensed IP, indicating the owner, licensor and/or co-owner(s), if applicable, and, for any Elected Candidate and Licensed Product-relevant subject matter or Materials, if no Patent is specifically licensed, a list of all subject matter or Materials that are included in the Bluebird Licensed IP, including those licensed under a materials use license or equivalent. Bluebird Controls the Patents listed on Appendix H and the Know-How within the Bluebird Licensed IP, and is entitled to grant the licenses specified herein. Bluebird has not granted to any Third Party any rights or licenses under such Patents or Know-How within the Bluebird Licensed IP that would conflict with the licenses granted to Celgene hereunder.

(b) *Third Party Agreements*. The Applicable Bluebird In-Licenses are valid and binding obligations of Bluebird and, to the Knowledge of Bluebird, the applicable licensor, enforceable against Bluebird and, to the Knowledge of Bluebird, the applicable licensor, in accordance with their terms, except as may be limited by general principles of equity (regardless of whether considered in a proceeding at law or in equity) and by applicable bankruptcy, insolvency, moratorium and other similar Laws of general application relating to or affecting creditors’ rights generally. Neither Bluebird nor any of its Affiliates has received any notice of any counterparty’s intention to terminate any Applicable Bluebird In-License in whole or in part or any notice requesting any amendment, alteration or modification of such Applicable Bluebird In-License or any sublicense or assignment thereunder. There is no breach or default, or event which upon notice or the passage of time, or both, would give rise to any breach or default, in the performance of any Applicable Bluebird In-License by Bluebird or any of its Affiliates or, to the Knowledge of Bluebird, the counterparty thereto, and Bluebird has not received any notice of any such breach, default or event. Except for the Applicable Bluebird In-Licenses, neither Bluebird nor any of its Affiliates is a party to any license, sublicense or other agreement pursuant to which Bluebird or such Affiliate has received a license or other rights relating to the Elected Candidate or Licensed Product. All Patents and Know-How licensed to Bluebird under the Applicable Bluebird In-Licenses are Controlled by Bluebird for purposes of the licenses granted to Celgene under this CCPS Agreement.

(c) *Patents*. To Bluebird’s Knowledge, the Patents listed on Appendix H have been procured or are being procured from the respective patent offices in accordance with applicable Law. None of the Patents included in the Bluebird Licensed IP is or has been involved in any opposition, cancellation, interference, reissue or reexamination proceeding, and no Bluebird Licensed IP is the subject of any judicial, administrative or arbitral order, award, decree, injunction, lawsuit, proceeding or stipulation. Neither Bluebird nor any of its Affiliates has received any notice alleging that the Patents in the Bluebird Licensed IP are invalid or unenforceable, or challenging Bluebird’s ownership of or right to use any such rights.

(d) *No Conflicts*. The execution, delivery and performance by Bluebird of this CCPS Agreement and the consummation of the transactions contemplated hereby will not result in any violation of, conflict with, result in a breach of or constitute a default under any understanding, contract or agreement to which Bluebird is a party or by which it is bound. Neither Bluebird nor any of its Affiliates has entered into any agreement or otherwise licensed, granted, assigned, transferred, conveyed or otherwise encumbered or disposed of any right, title or interest in or to any of its assets, including any intellectual property rights, that would in any way conflict with or impair the scope of any rights or licenses granted to Celgene hereunder.

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(e) *Outlicenses.* Appendix J sets forth a complete and accurate list of all agreements relating to the licensing, sublicensing or other granting of rights by Bluebird to any Person with respect to the Bluebird Licensed IP and the Field, and Bluebird has provided complete and accurate copies of all such agreements to Celgene. Except for the Applicable Bluebird In-Licenses, Bluebird and its Affiliates are not subject to any payment obligations to Third Parties as a result of the execution or performance of this CCPS Agreement. Neither Bluebird nor any of its Affiliates has granted any liens or security interests on the Bluebird Licensed IP and the Bluebird Licensed IP is free and clear of any mortgage, pledge, claim, security interest, covenant, easement, encumbrance, lien or charge of any kind.

(f) *No Proceedings.* There is no action, suit, proceeding or investigation pending or, to the Knowledge of Bluebird, currently threatened in writing against or affecting Bluebird that questions the validity of this CCPS Agreement or the right of Bluebird to enter into this CCPS Agreement or consummate the transactions contemplated hereby.

(g) *No Infringement.* Neither Bluebird nor any of its Affiliates has received any notice of any claim that any Patent, Know-How or other intellectual property Controlled by a Third Party would be infringed or misappropriated by the production, use, research, Development, Manufacture or Commercialization of the Elected Candidate or Licensed Product pursuant to this CCPS Agreement, and, to the Knowledge of Bluebird, there are no Patents, Know-How or other intellectual property owned by a Third Party and not included in the Bluebird Licensed IP or Bluebird In-Licensed IP that are necessary for the production, use, research, Development, Manufacture or Commercialization of Elected Candidate or Licensed Product.

16.3 Disclaimers. Without limiting the respective rights and obligations of the Parties expressly set forth herein, each Party specifically disclaims any guarantee that any Licensed Product will be successful, in whole or in part. EXCEPT AS OTHERWISE EXPRESSLY PROVIDED IN THIS CCPS AGREEMENT, THE PARTIES MAKE NO REPRESENTATIONS AND EXTEND NO WARRANTY OF ANY KIND, EITHER EXPRESS OR IMPLIED, WITH RESPECT TO ANY PATENTS, KNOW-HOW, ELECTED CANDIDATE OR LICENSED PRODUCT, INCLUDING WARRANTIES OF VALIDITY OR ENFORCEABILITY OF ANY PATENT RIGHTS, TITLE, QUALITY, MERCHANTABILITY, FITNESS FOR A PARTICULAR USE OR PURPOSE, PERFORMANCE, AND NONINFRINGEMENT OF ANY THIRD PARTY PATENTS OR OTHER INTELLECTUAL PROPERTY RIGHTS.

16.4 [\*\*\*]

16.5 Performance by Others. The Parties recognize that each Party may perform some or all of its obligations under this CCPS Agreement through Affiliates and permitted subcontractors provided, however, that each Party will remain responsible and liable for the performance by its Affiliates and permitted subcontractors and will cause its Affiliates and permitted subcontractors to comply with the provisions of this CCPS Agreement in connection therewith.

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16.6 Indemnification.

(a) *Indemnification by Celgene.* Celgene will indemnify Bluebird, its Affiliates and their respective directors, officers, employees, Third Party licensors and agents, and their respective successors, heirs and assigns (collectively, “Bluebird Indemnitees”), and defend and save each of them harmless, from and against any and all losses, damages, liabilities, costs and expenses (including reasonable attorneys’ fees and expenses) (collectively, “Losses”) in connection with any and all suits, investigations, claims or demands of Third Parties (collectively, “Third Party Claims”) against the Bluebird Indemnitees arising from or occurring as a result of: (i) the material breach by Celgene of any term of this CCPS Agreement; (ii) any gross negligence or willful misconduct on the part of Celgene in performing its obligations under this CCPS Agreement; (iii) the Development or Commercialization by or on behalf of Celgene or any of its Affiliates or Sublicensees of Elected Candidate or Licensed Product for ROW Administration, and (iv) [\*\*\*] except in each case for those Losses for which Bluebird has an obligation to indemnify Celgene pursuant to Section 11.6(b), as to which Losses each Party will indemnify the other to the extent of their respective liability; provided, however, that Celgene will not be obligated to indemnify Bluebird Indemnitees for any Losses to the extent that such Losses arise as a result of gross negligence or willful misconduct on the part of an Bluebird Indemnitee.

(b) *Indemnification by Bluebird.* Bluebird will indemnify Celgene, its Affiliates and their respective directors, officers, employees and agents, and their respective successors, heirs and assigns (collectively, “Celgene Indemnitees”), and defend and save each of them harmless, from and against any and all Losses in connection with any and all Third Party Claims against the Celgene Indemnitees arising from or occurring as a result of: (i) the material breach by Bluebird of any term of this CCPS Agreement; (ii) any gross negligence or willful misconduct on the part of Bluebird in performing its obligations under this CCPS Agreement; or (iii) the Development by or on behalf of Bluebird or any of its Affiliates or Sublicensees of Elected Candidate or Licensed Product, except in each case for those Losses for which Celgene has an obligation to indemnify Bluebird pursuant to Section 11.6(a), as to which Losses each Party will indemnify the other to the extent of their respective liability for the Losses; provided, however, that Bluebird will not be obligated to indemnify Celgene Indemnitees for any Losses to the extent that such Losses arise as a result of gross negligence or willful misconduct on the part of a Celgene Indemnitee.

(c) *Notice of Claim.* All indemnification claims provided for in Sections 11.6(a) and 11.6(b) will be made solely by such Party to this CCPS Agreement (the “Indemnified Party”). The Indemnified Party will promptly notify the indemnifying Party (an “Indemnification Claim Notice”) of any Losses or the discovery of any fact upon which the Indemnified Party intends to base a request for indemnification under Section 11.6(a) and 11.6(b), but in no event will the indemnifying Party be liable for any Losses that result from any delay in providing such notice. Each Indemnification Claim Notice must contain a description of the claim and the nature and estimated amount of such Loss (to the extent that the nature and amount of such Loss is known at such time). The Indemnified Party will furnish promptly to the indemnifying Party copies of all papers and official documents received in respect of any Losses and Third Party Claims.

(d) *Defense, Settlement, Cooperation and Expenses .*

(i) *Control of Defense.* At its option, the indemnifying Party may assume the defense of any Third Party Claim by giving written notice to the Indemnified Party within thirty

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(30) days after the indemnifying Party’s receipt of an Indemnification Claim Notice, provided however that (A) the Third Party Claim solely seeks monetary damages and (B) the indemnifying Party expressly agrees in writing that as between the indemnifying Party and the Indemnified Party, the indemnifying Party will be solely obligated to satisfy and discharge the Third Party Claim in full and is able to reasonably demonstrate that it has sufficient financial resources (the matters described in (A) and (B), the “Litigation Conditions”). The assumption of the defense of a Third Party Claim by the indemnifying Party will not be construed as an acknowledgment that the indemnifying Party is liable to indemnify the Indemnified Party in respect of the Third Party Claim, nor will it constitute a waiver by the indemnifying Party of any defenses it may assert against the Indemnified Party’s claim for indemnification. Upon assuming the defense of a Third Party Claim, the indemnifying Party may appoint as lead counsel in the defense of the Third Party Claim any legal counsel selected by the indemnifying Party (the indemnifying Party will consult with the Indemnified Party with respect to a possible conflict of interest of such counsel retained by the indemnifying Party). The Indemnified Party may, at any time, assume the defense of a Third Party Claim if at any time the Litigation Conditions are not satisfied with respect to such Claim. In the event the indemnifying Party assumes the defense of a Third Party Claim, the Indemnified Party will immediately deliver to the indemnifying Party all original notices and documents (including court papers) received by the Indemnified Party in connection with the Third Party Claim. Should the indemnifying Party assume the defense of a Third Party Claim, except as provided in Section 11.6(d)(ii) the indemnifying Party will not be liable to the Indemnified Party for any legal costs or expenses subsequently incurred by such Indemnified Party in connection with the analysis, defense or settlement of the Third Party Claim. In the event that it is ultimately determined that the indemnifying Party is not obligated to indemnify, defend or hold harmless the Indemnified Party from and against the Third Party Claim, the Indemnified Party will reimburse the indemnifying Party for any and all costs and expenses (including attorneys’ fees and costs of suit) and any Third Party Claims incurred by the indemnifying Party in its defense of the Third Party Claim.

(ii) *Right to Participate in Defense.* Without limiting Section 11.6(d)(i), any Indemnified Party will be entitled to participate in, but not control, the defense of such Third Party Claim and to employ counsel of its choice for such purpose; provided, however, that such employment will be at the Indemnified Party’s own cost and expense unless (i) the employment thereof has been specifically authorized by the indemnifying Party in writing, (ii) the indemnifying Party has failed to assume the defense and employ counsel in accordance with Section 11.6(d)(i) (in which case the Indemnified Party will control the defense), (iii) the interests of the Indemnified Party and the indemnifying Party with respect to such Third Party Claim are sufficiently adverse to prohibit the representation by the same counsel of both Parties under applicable Law, ethical rules or equitable principles, or (iv) the indemnifying Party no longer satisfies the Litigation Conditions, in which case the indemnifying Party will assume [\*\*\*] percent ([\*\*\*]%) of any such costs and expenses of counsel for the Indemnified Party.

(iii) *Settlement.* With respect to any Third Party Claims that relate solely to the payment of money damages in connection with a Third Party Claim and that will not result in the Indemnified Party’s becoming subject to injunctive or other relief or otherwise adversely affecting the business of the Indemnified Party in any manner, and as to which the indemnifying Party will have acknowledged in writing the obligation to indemnify the Indemnified Party



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hereunder, and subject to the Litigation Conditions being satisfied, the indemnifying Party will have the sole right to agree to the entry of any judgment, enter into any settlement or otherwise dispose of such Loss, on such terms as the indemnifying Party, in its sole discretion, will deem appropriate. With respect to all other Losses in connection with Third Party Claims, where the indemnifying Party has assumed the defense of the Third Party Claim in accordance with Section 11.6(d)(i), the indemnifying Party will have authority to agree to the entry of any judgment, enter into any settlement or otherwise dispose of such Loss provided it obtains the prior written consent of the Indemnified Party (such consent not to be unreasonably withheld, delayed or conditioned). The indemnifying Party will not be liable for any settlement or other disposition of a Loss by an Indemnified Party that is reached without the prior written consent of the indemnifying Party. Regardless of whether the indemnifying Party chooses to defend or prosecute any Third Party Claim, no Indemnified Party will admit any liability with respect to or settle, compromise or discharge, any Third Party Claim without the prior written consent of the indemnifying Party, such consent not to be unreasonably withheld, delayed or conditioned.

(iv) *Cooperation*. If the indemnifying Party chooses to defend or prosecute any Third Party Claim, the Indemnified Party will, and will cause each other Indemnified Party to, cooperate in the defense or prosecution thereof and will furnish such records, information and testimony, provide such witnesses and attend such conferences, discovery proceedings, hearings, trials and appeals as may be reasonably requested in connection therewith. Such cooperation will include access during normal business hours afforded to indemnifying Party to, and reasonable retention by the Indemnified Party of, records and information that are reasonably relevant to such Third Party Claim, and making Indemnified Parties and other employees and agents available on a mutually convenient basis to provide additional information and explanation of any material provided hereunder, and the indemnifying Party will reimburse the Indemnified Party for all its reasonable out-of-pocket costs and expenses in connection therewith.

(v) *Costs and Expenses*. Except as provided above in this Section 11.6(d), the costs and expenses, including attorneys’ fees and expenses, incurred by the Indemnified Party in connection with any claim will be reimbursed on a calendar quarter basis by the indemnifying Party, without prejudice to the indemnifying Party’s right to contest the Indemnified Party’s right to indemnification and subject to refund in the event the indemnifying Party is ultimately held not to be obligated to indemnify the Indemnified Party.

16.7 Insurance. Each Party will maintain at its sole cost and expense, an adequate liability insurance or self-insurance program (including product liability insurance) to protect against potential liabilities and risk arising out of activities to be performed under this CCPS Agreement, and any agreement related hereto and upon such terms (including coverages, deductible limits and self-insured retentions) as are customary in the U.S. pharmaceutical industry for the activities to be conducted by such Party under this CCPS Agreement. Subject to the preceding sentence, such liability insurance or self-insurance program will insure against all types of liability, including personal injury, physical injury or property damage arising out of the manufacture, sale, use, distribution or marketing of Licensed Product. The coverage limits set forth herein will not create any limitation on a Party’s liability to the other under this CCPS Agreement.

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16.8 U.S. Administration Liabilities. In the event that either Party (i) incurs any Losses in connection with a Third Party Claim for personal injury or death caused by the use of Licensed Product for U.S. Administration, or (ii) is required to make payments to any Third Party in order to acquire a license or other rights under Patents or Know-How necessary for the Development, Manufacture or Commercialization of Licensed Product for U.S. Administration (collectively, “U.S. Administration Liabilities”), such U.S. Administrative Losses arising from or occurring as a result of the performance, in good faith, of the Development, Manufacture or Commercialization of Licensed Product for U.S. Administration in accordance with this CCPS Agreement will be charged to such Party’s Operating Profit or Loss under the Profit & Loss Share, provided that Operating Profit or Loss will not include U.S. Administration Liabilities of a Party or its Affiliates: (1) that are caused by a breach of this CCPS Agreement by such Party or its Affiliates; (2) incurred with respect to or allocable to products other than Licensed Product for U.S. Administration; or (3) that are subject to indemnification by such Party pursuant to Section 16.6 (and for clarity, if a Third Party makes a Third Party Claim directly against Bluebird (or any of its Affiliates) or Celgene (or any of its Affiliates), respectively, that would otherwise be indemnified by Bluebird or Celgene, respectively, if such Third Party Claim had been made against the other Party (or any of its Affiliates), then U.S. Administration Liabilities incurred by Bluebird or Celgene in connection with such direct Third Party Claim will not be included in the calculation of Operating Profit or Loss).

## 17. Term and Termination.

17.1 Term. This CCPS Agreement will commence as of the CCPS Agreement Effective Date and, unless sooner terminated in accordance with the terms hereof or by mutual written consent, will continue on a country-by-country basis, until there are no more payments owed one or the other Party on Licensed Product in such country (the longest such period of time for any Licensed Product hereunder, the “CCPS Agreement Term”); for clarity, unless sooner terminated in accordance with the terms hereof or by mutual written consent, this CCPS Agreement Term will continue in all events until Licensed Product is no longer being Developed or Commercialized in the United States. Upon there being no more such payments hereunder for any such Licensed Product in such country (other than the United States), the licenses contained in Section 10.1 will become fully paid up and will remain exclusive with respect to such Licensed Product in such country.

### 17.2 Termination by Bluebird.

(a) *Breach*. Bluebird will have the right to terminate this CCPS Agreement in full upon delivery of written notice to Celgene in the event of any material breach by Celgene of any terms and conditions of this CCPS Agreement in a manner that fundamentally frustrates the transactions contemplated by this CCPS Agreement, provided that such termination will not be effective if such breach, has been cured within [\*\*\*] after written notice thereof is given by Bluebird to Celgene specifying the nature of the alleged breach (or, if such default cannot be cured within such [\*\*\*] after such notice if Celgene commences actions to cure such default within such [\*\*\*] and thereafter diligently continues such actions, but fails to cure the default by the end of such [\*\*\*]); provided, however, that to the extent such material breach involves the failure to make a payment when due, such breach must be cured within [\*\*\*] after written notice thereof is given by Bluebird to Celgene.

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(b) [\*\*\*]

(c) *Termination of the Profit & Loss Share.* Bluebird will have the right to terminate the Profit & Loss Share by delivering written notice to Celgene, such termination to be effective [\*\*\*] following the date of such notice. Promptly following such notice, the Parties will enter into a license agreement with respect to the United States and the ROW, which agreement will be substantially identical to the License Agreement attached as Exhibit A to the Master Collaboration Agreement, with such changes that the Parties may, acting reasonably, mutually agree are required in order to address any specific facts or circumstances existing at the time of such termination. The Parties will enter into such license agreement no later than the effective date of such termination and, if such license agreement is not entered into prior the expiration of such [\*\*\*], upon execution, the effective date of such license agreement will be deemed to be the effective date of such termination. For clarity, (i) termination of the Profit & Loss Share pursuant to this Section 17.2(c) will not release Bluebird from any obligation or liability which, at the time of the effective date of such termination, has already accrued to Celgene or which is attributable to a period prior to the effective date of such termination, and (ii) any events that have already occurred before the effective date of such termination (such as achievement of any milestones) will not trigger any payment obligation by Celgene to Bluebird under such executed license agreement (other than, for clarity, the Milestone Payment based on the Pivotal Study if not already paid or accrued under this CCPS Agreement).

### 17.3 Termination by Celgene.

(a) *Breach.* Celgene will have the right to terminate this CCPS Agreement in full upon delivery of written notice to Bluebird in the event of any material breach by Bluebird of any terms and conditions of this CCPS Agreement in a manner that fundamentally frustrates the transactions contemplated by this CCPS Agreement, provided that such termination will not be effective if such breach has been cured within [\*\*\*] after written notice thereof is given by Celgene to Bluebird specifying the nature of the alleged breach (or, if such default cannot be cured within such [\*\*\*] period, within [\*\*\*] after such notice if Bluebird commences actions to cure such default within such [\*\*\*] period and thereafter diligently continues such actions, but fails to cure the default by the end of such [\*\*\*]).

(b) *Discretionary Termination.* Beginning with [\*\*\*], Celgene will have the right to terminate this CCPS Agreement in full, at its discretion for any reason, by delivering written notice to Bluebird, such termination to be effective [\*\*\*] following the date of such notice.

(c) [\*\*\*]

(d) *Alternative to Termination Under Section 17.3(a).* If Celgene has the right to terminate this CCPS Agreement under Section 17.3(a) or 17.3(c) (including expiration of all applicable cure periods thereunder), in lieu of exercising such termination right, Celgene may elect once by written notice to Bluebird before the end of such applicable cure period to have this CCPS Agreement continue in full force and effect and instead have, starting immediately after the end of such applicable cure period, any future Milestone Payments set forth in Section 11.2(b) and the royalty rates set forth in the table set forth in Section 11.3(a) be reduced by [\*\*\*], provided that such reduction will not apply if such future Milestone Payments and royalty rates have already been reduced pursuant to Section 11.4(c) of the Master Collaboration Agreement.

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17.4 Effects of Termination or Expiration. Upon termination (but not expiration pursuant to Section 17.1) of this CCPS Agreement for any reason:

(a) *Wind Down*. Celgene will responsibly wind-down, in accordance with accepted pharmaceutical industry norms and ethical practices, any on-going clinical studies for which it has responsibility hereunder in which patient dosing has commenced or, if reasonably practicable and requested by Bluebird, allow Celgene, its Affiliates or its Sublicensees to complete such trials. Celgene will be responsible for any costs associated with such wind-down. Bluebird will pay all costs incurred by either Party to complete such studies should Bluebird request that such studies be completed.

(b) *Sublicenses*. A termination of this CCPS Agreement will not automatically terminate any sublicense granted by Celgene pursuant to Section 10.3 for Commercialization rights with respect to a non-Affiliated Sublicensee, provided that (i) such Sublicensee is not then (a) in material breach of any provision of this CCPS Agreement or (b) in material breach of the applicable sublicense agreement or otherwise in breach of such sublicense agreement in a manner that would give rise to a right of termination on the part of Celgene, (ii) if Bluebird terminates this CCPS Agreement pursuant to Section 17.2(a) for Celgene’s failure to fulfill its payment obligations hereunder, such Sublicensee agrees to and does pay to Bluebird all outstanding amounts that accrued as a result of such Sublicensee’s activities under the sublicense, (iii) Bluebird will have the right to step into the role of Celgene as sublicensor under any such sublicense executed after the CCPS Agreement Effective Date, with all the rights that Celgene had under such sublicense, solely with respect to the Bluebird Licensed IP, prior to termination of this CCPS Agreement (including the right to receive any payments to Celgene by such Sublicensee that accrue from and after the date of the termination of this CCPS Agreement solely with respect to the Bluebird Licensed IP), (iv) such Sublicensee will pay to Bluebird all amounts that Celgene would have been obligated to pay to Bluebird hereunder with respect to such Sublicensee’s activities had this CCPS Agreement not terminated (less any amounts received by Bluebird in clause (iii) above) and (v) the survival of such sublicense will not result in an imposition of any additional obligations on the part of Bluebird that are not included within the scope of this CCPS Agreement. Celgene will include in any sublicense agreement executed after the CCPS Agreement Effective Date that relates solely to the Bluebird Licensed IP a provision in which said Sublicensee acknowledges its obligations to Bluebird under this Section 17.4(b).

(c) *Cessation of Rights*. Except as otherwise expressly provided in this Section 17, all rights and licenses granted by Bluebird to Celgene in Section 10.1 will terminate, and all rights granted by Celgene to Bluebird in Section 10.2 will terminate, and Celgene and its Affiliates and Sublicensees will cease all use of Bluebird Licensed IP and all Development and Commercialization of Elected Candidate and Licensed Product.

(d) *Regulatory Approvals*. To the extent permitted by applicable Law, and subject to Bluebird paying commercially reasonable compensation to Celgene for the assets to be transferred pursuant to this Section 17.4(d) (such compensation to either be mutually agreed to or determined through arbitration as provided in Section 17.4(h) below, and such compensation to

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be reduced by [\*\*\*] from what would be commercially reasonable compensation if this CCPS Agreement is terminated by Bluebird pursuant to Section 17.2(a)), all Regulatory Approvals and other regulatory filings and communications owned (in whole or in part) or otherwise Controlled by Celgene and its Affiliates and Sublicensees solely relating to the Elected Candidate and/or Licensed Product, and all other documents solely relating to and necessary to further Develop and Commercialize Elected Candidate and Licensed Product, as such items exist as of the effective date of such termination (including all solely related completed and ongoing clinical studies) will be assigned to Bluebird, and Celgene will provide to Bluebird one (1) copy of the foregoing and all documents contained in or referenced in any such items, together with the raw and summarized data for any clinical studies (and where reasonably available, electronic copies thereof). In the event of failure to obtain assignment, subject to the Parties agreeing on commercially reasonable compensation for the right to access and reference, Celgene hereby consents and grants to Bluebird the right to access and reference (without any further action required on the part of Celgene, whose authorization to file this consent with any Regulatory Authority is hereby granted) any such item.

(e) *Licenses*. Subject to Bluebird paying (i) commercially reasonable compensation to Celgene for the licenses to be granted pursuant to subsection (1) of this Section 17.4(e) (such compensation to either be mutually agreed to or determined through arbitration as provided in Section 17.4(h) below, and such compensation to be reduced by [\*\*\*] from what would be commercially reasonable compensation if this CCPS Agreement is terminated by Bluebird pursuant to Section 17.2(a)), and (ii) amounts payable to Celgene’s licensors as set forth below, Celgene will grant to Bluebird and its Affiliates (1) a worldwide, perpetual and irrevocable, nontransferable (except in connection with a permitted assignment of this CCPS Agreement in accordance with Section 18.12), exclusive license, with the right to grant sublicenses through multiple tiers (subject to Section 10.3, *mutatis mutandis*), under Celgene Licensed Product IP, and (2) an exclusive sublicense under the Celgene Licensed Product In-Licensed IP, in each case ((1) and (2)) to the extent such Celgene Licensed Product IP and Celgene Licensed Product In-Licensed IP are used in or Cover the Licensed Product as of the effective date of termination and to the extent such Celgene Licensed Product IP and Celgene Licensed Product In-Licensed IP exist as of the effective date of such termination (including in each case any additions, divisions, continuations, continuations-in-part, invention certificates, substitutions, reissues, reexaminations, extensions, registrations, supplementary protection certificates and renewals of such Celgene Licensed Product IP and Celgene Licensed Product In-Licensed IP), solely to the extent necessary to research, Develop, Manufacture and Commercialize the Elected Candidate and Licensed Product. With respect to grants of a sublicense under subsection (2) above, Bluebird will be responsible for all amounts payable to the applicable licensor that are attributable to Bluebird as a sublicensee thereunder under this CCPS Agreement, and Celgene will pay same and Bluebird will reimburse Celgene for [\*\*\*] percent ([\*\*\*]%) of such payments within thirty (30) days of receipt of Celgene’s written invoice therefor. Celgene will provide Bluebird with copies of all applicable Celgene Licensed Product In-Licenses promptly following the effective date of the termination of this License Agreement. The Prosecution and Maintenance and enforcement and defense rights and obligations of the Parties with respect to any Patents licensed or sublicensed to Bluebird pursuant to this Section 17.4(e) will be discussed and agreed to by the Parties, with the understanding that such Prosecution and Maintenance and

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enforcement and defense rights and obligations will be substantially similar to those set forth in Section 13, with the roles of Bluebird and Celgene reversed (and such other changes as are appropriate from the context, and taking into account any rights retained by a Third Party licensor of Celgene to Prosecute and Maintain or enforce and defend any Patent sublicensed to Bluebird under this Section 17.4(e)).

(f) *Trademarks*. Subject to Bluebird paying commercially reasonable compensation to Celgene for the license to be granted pursuant to this Section 17.4(f) (such compensation to either be mutually agreed to or determined through arbitration as provided in Section 17.4(h) below, and such compensation to be reduced by [\*\*\*] from what would be commercially reasonable compensation if this CCPS Agreement is terminated by Bluebird pursuant to Section 17.2(a)), Celgene will exclusively license to Bluebird any registered or unregistered trademarks or internet domain names that are specific to and solely used for the Licensed Product worldwide (it being understood that the foregoing will not include any trademarks or internet domain names that contain the corporate or business name(s) of Celgene).

(g) *Baylor Product License*. If the Licensed Product is subject to a Baylor Product License, then Celgene will, at Bluebird’s written request, assign to Bluebird the applicable Baylor Product License in accordance with the terms of Section 13.5 thereof, provided that if such Baylor Product License applies to other products, Celgene will assign or sublicense to Bluebird that portion of the Baylor Product License that applies to the Licensed Product, and with the consequences therein stated (that is, Celgene will remain responsible for all payments accruing thereunder before the assignment, and Bluebird will be responsible for all payments accruing thereunder after such assignment).

(h) *Commercially Reasonable Compensation*. If the Parties are unable to agree on the amount of commercially reasonable compensation payable by Bluebird to Celgene pursuant to Sections 17.4(d), 17.4(e) or 17.4(f) within ten (10) days of the effective date of termination of this CCPS Agreement, [\*\*\*]

(i) *Country Termination*. If this CCPS Agreement is terminated only with respect to a specific country pursuant to Section 11.2(b) or Section 11.3(c), the provisions of this Section 17.4 will apply only with respect to such terminated country.

17.5 Survival. In addition to the termination consequences set forth in Section 17.4, the following provisions will survive termination or expiration of this CCPS Agreement: Sections 1, 4.3, 8.2, 2.3(c)(ii), 10.3(c) (*mutatis mutandis* with respect to licenses granted to Bluebird under Section 17.4, but excluding subsections (i) and (ii) of Section 10.3(c)) 10.6, 10.8, 11.5, 11.6, 12, 15, 16.3, 16.4, 16.6, 16.7, 16.8, 17.4, 17.5, 17.6 and 18, and Appendix F (to the extent required to provide for a true up of Operating Profit and Losses during the term of this CCPS Agreement following termination of this CCPS Agreement). Termination or expiration of this CCPS Agreement will not relieve the Parties of any liability or obligation which accrued hereunder prior to the effective date of such termination or expiration nor preclude either Party from pursuing all rights and remedies it may have hereunder or at law or in equity with respect to any breach of this CCPS Agreement nor prejudice either Party’s right to obtain performance of any obligation. All other rights and obligations will terminate upon expiration of this CCPS Agreement.

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17.6 Right to Set-off. Notwithstanding anything to the contrary in this CCPS Agreement, each Party has the right at all times to retain and set off against all amounts due and owing to the other Party as determined in a final judgment any damages recovered by such Party for any Losses incurred by such Party.

## **18. General Provisions.**

18.1 Cumulative Remedies and Irreparable Harm. All rights and remedies of the Parties hereunder will be cumulative and in addition to all other rights and remedies provided hereunder or available by agreement, at law or otherwise. Each Party acknowledges and agrees that breach of any of the terms or conditions of this CCPS Agreement would cause irreparable harm and damage to the other and that such damage may not be ascertainable in money damages and that as a result thereof the non-breaching Party would be entitled to seek from a court equitable or injunctive relief restraining any breach or future violation of the terms contained herein by the breaching Party without the necessity of proving actual damages or posting bond. Such right to equitable relief is in addition to whatever remedies either Party may be entitled to as a matter of law or equity, including money damages.

### **18.2 Business Combination and IP.**

(a) *Bluebird Business Combination*. Notwithstanding anything to the contrary herein, for purposes of this CCPS Agreement, no Know-How, Materials, Patents, Regulatory Data, Regulatory Filings or Regulatory Approvals not Controlled by Bluebird or any of its Affiliates prior to a Business Combination of Bluebird will be Controlled for purposes of this CCPS Agreement after such Business Combination of Bluebird, other than (i) Applicable Bluebird In-Licenses to the extent in effect immediately prior to such Business Combination of Bluebird, (ii) Collaboration IP, and (iii) any Patent that claims priority, directly or indirectly, to any other Patent first Controlled before such Business Combination of Bluebird will be Controlled thereafter no matter when such Patent is filed or issued.

(b) *Celgene Business Combination*. Notwithstanding anything to the contrary herein, for purposes of this CCPS Agreement, no Know-How, Materials, Patents, Regulatory Data, Regulatory Filings or Regulatory Approvals not Controlled by Celgene or any of its Affiliates prior to a Business Combination of Celgene will be Controlled for purposes of this CCPS Agreement after such Business Combination of Celgene, other than Collaboration IP, and except that any Patent that claims priority, directly or indirectly, to any other Patent first Controlled before such Business Combination of Celgene will be Controlled thereafter no matter when such Patent is filed or issued.

18.3 Relationship of Parties. Nothing in this CCPS Agreement is intended or will be deemed to constitute a partnership, agency, employer-employee or joint venture relationship between the Parties. No Party will incur any debts or make any commitments for the other, except to the extent, if at all, specifically provided therein. There are no express or implied Third Party beneficiaries hereunder (except as set forth in Section 10.2 and except for Bluebird Indemnitees and Celgene Indemnitees for purposes of Section 16.6).

18.4 Compliance with Law. Each Party will perform or cause to be performed any and all of its obligations or the exercise of any and all of its rights hereunder in good scientific manner

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and in compliance with all applicable Law. Without limiting the foregoing, Bluebird will comply with all applicable Laws and regulations (including U.S. Foreign Corrupt Practices Act and any other applicable anti-bribery or anti-kickback laws or regulations).

18.5 Force Majeure. Neither Party will be liable to the other for failure of or delay in performing obligations set forth in this CCPS Agreement (other than any obligation to pay monies when due), and neither will be deemed in breach of such obligations, if such failure or delay is due to natural disasters or any causes reasonably beyond the control of such Party; provided that the Party affected will promptly notify the other of the force majeure condition and will exert reasonable efforts to eliminate, cure or overcome any such causes and to resume performance of its obligations as soon as possible.

18.6 Governing Law. This CCPS Agreement will be governed by and construed in accordance with the Laws of the State of New York, without respect to its conflict of laws rules, provided that any dispute relating to the scope, validity, enforceability or infringement of any Patents or Know-How will be governed by, and construed and enforced in accordance with, the substantive laws of the jurisdiction in which such Patents or Know-How apply.

18.7 Counterparts; Facsimiles. This CCPS Agreement may be executed in one or more counterparts, each of which will be deemed an original, and all of which together will be deemed to be one and the same instrument. Facsimile or PDF execution and delivery of this CCPS Agreement by either Party will constitute a legal, valid and binding execution and delivery of this CCPS Agreement by such Party

18.8 Headings. All headings in this CCPS Agreement are for convenience only and will not affect the meaning of any provision hereof.

18.9 Waiver of Rule of Construction. Each Party has had the opportunity to consult with counsel in connection with the review, drafting and negotiation of this CCPS Agreement. Accordingly, the rule of construction that any ambiguity in this CCPS Agreement will be construed against the drafting Party will not apply.

18.10 Interpretation. Whenever any provision of this CCPS Agreement uses the term “including” (or “includes”), such term will be deemed to mean “including without limitation” (or “includes without limitations”). “Herein,” “hereby,” “hereunder,” “hereof” and other equivalent words refer to this CCPS Agreement as an entirety and not solely to the particular portion of this CCPS Agreement in which any such word is used. All definitions set forth herein will be deemed applicable whether the words defined are used herein in the singular or the plural. Unless otherwise provided, all references to Sections and Appendices in this CCPS Agreement are to Sections and Appendices of this CCPS Agreement. References to any Sections include Sections and subsections that are part of the related Section (*e.g.*, a section numbered “Section 2.1” would be part of “Section 2”, and references to “Section 2.1” would also refer to material contained in the subsection described as “Section 2.1(a)”).

18.11 Binding Effect. This CCPS Agreement will inure to the benefit of and be binding upon the Parties, their Affiliates, and their respective lawful successors and assigns.

18.12 Assignment. This CCPS Agreement may not be assigned by either Party, nor may either Party delegate its obligations or otherwise transfer licenses or other rights created by this



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CCPS Agreement, except as expressly permitted hereunder or otherwise without the prior written consent of the other Party, which consent will not be unreasonably withheld, delayed or conditioned; provided that without consent (i) Celgene may assign this CCPS Agreement to (x) an Affiliate or (y) its successor in connection with the merger, consolidation, or sale of all or substantially all of its assets, and (ii) Bluebird may assign this CCPS Agreement to (x) an Affiliate or (y) its successor in connection with the merger, consolidation, or sale of all or substantially all of its assets or that portion of its business pertaining to the subject matter of this CCPS Agreement; provided further that, except in the case where a Party is involved in a merger or consolidation where it is the surviving entity and no assets of such Party that are subject to this CCPS Agreement have been transferred as a result of such merger or consolidation, (a) such assigning Party provides the other Party to this Agreement with at least thirty (30) business days advance written notice of such assignment(s) and the assigning Party agrees in a written agreement delivered prior to such assignment(s) to the non-assigning Party (and upon which such non-assigning Party may rely) to remain fully liable for the performance of its obligations under this Agreement by its assignee(s), (b) the assignee(s) agree in a written agreement delivered prior to such assignment(s) to the non-assigning Party (and upon which such non-assigning Party may rely) to assume performance of all such assigned obligations, (c) in the case of any assignment(s) by Bluebird, all Bluebird Licensed IP licensed to Celgene under this CCPS Agreement will be transferred to such assignee(s) effective as of such assignment(s), (d) all of the matters referred to in clauses (a), (b) and (c), as applicable, will be set forth in documentation reasonably acceptable to the non-assigning Party prior to any such assignment(s) (and with such reasonable acceptance not to be unreasonably withheld, conditioned or delayed) and in all cases will provide the non-assigning Party with the full benefits of its rights under this Agreement (after taking into account all risks involving applicable counter-party performance and bankruptcy and insolvency risks, including those involving contractual rejection under 11 USC §365) as if no such assignment(s) had occurred, and (e) in the case of any assignment(s), the assigning Party will reimburse the non-assigning Party for all of the legal fees and expenses incurred by such non-assigning Party in connection with the matters set forth in clause (D) of this sentence in an aggregate amount not to exceed [\*\*\*], and provided, further, that if Bluebird wishes to assign any Bluebird Licensed IP to its Affiliates, it will be permitted to do so conditioned on each such Affiliate becoming a party to this CCPS Agreement, in the form of an amendment to this CCPS Agreement executed by Celgene, Bluebird and such Affiliate, pursuant to which such Affiliate would agree to assume all obligations hereunder, and grant to Celgene all rights hereunder, with respect to the Bluebird Licensed IP. The terms of this Agreement will be binding upon and will inure to the benefit of the successors, heirs, administrators and permitted assigns of the Parties. Any purported assignment in violation of this Section 18.12 will be null and void *ab initio*.

18.13 Notices. All notices, requests, demands and other communications required or permitted to be given pursuant to this CCPS Agreement will be in writing and will be deemed to have been duly given upon the date of receipt if delivered by hand, recognized international overnight courier, confirmed facsimile transmission, or registered or certified mail, return receipt requested, postage prepaid to the applicable address or facsimile number in Section 13.14 in the Master Collaboration Agreement. Either Party may change its designated address and facsimile number by notice to the other Party in the manner provided in this Section 13.14.

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18.14 Amendment and Waiver. This CCPS Agreement may be amended, supplemented, or otherwise modified only by means of a written instrument signed by both Parties; provided that any unilateral undertaking or waiver made by one Party in favor of the other will be enforceable if undertaken in a writing signed by the Party to be charged with the undertaking or waiver. Any waiver of any rights or failure to act in a specific instance will relate only to such instance and will not be construed as an agreement to waive any rights or fail to act in any other instance, whether or not similar.

18.15 Severability. In the event that any provision of this CCPS Agreement will, for any reason, be held to be invalid or unenforceable in any respect, such invalidity or unenforceability will not affect any other provision hereof, and the Parties will negotiate in good faith to modify this CCPS Agreement to preserve (to the extent possible) their original intent.

18.16 Entire Agreement. This CCPS Agreement, together with the Master Collaboration Agreement, is the sole agreement with respect to the subject matter and supersedes all other agreements and understandings between the Parties with respect to same (including Confidential Agreement). In the event of any conflict between the terms of this CCPS Agreement and the terms of the Master Collaboration Agreement, the terms of this CCPS Agreement will control.

18.17 Force Majeure. Neither Celgene nor Bluebird will be liable for failure of or delay in performing obligations set forth in this CCPS Agreement (other than any obligation to pay monies when due), and neither will be deemed in breach of such obligations, if such failure or delay is due to natural disasters or any causes reasonably beyond the control of Celgene or Bluebird and without the fault or negligence of the Party so failing or delaying; provided that the Party affected will promptly notify the other of the force majeure condition and will exert reasonable efforts to eliminate, cure or overcome any such causes and to resume performance of its obligations as soon as possible.

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IN WITNESS WHEREOF, the Parties have caused this Co-Development, Co-Promote and Profit Share Agreement to be executed by their respective duly authorized officers as of the CCPS Agreement Effective Date.

BLUEBIRD BIO, INC.

By: \_\_\_\_\_  
(Signature)

Name: \_\_\_\_\_

Title: \_\_\_\_\_

Date: \_\_\_\_\_

CELGENE CORPORATION

By: \_\_\_\_\_  
(Signature)

Name: \_\_\_\_\_

Title: \_\_\_\_\_

Date: \_\_\_\_\_

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**Appendix A**

**Additional Defined Terms**

“Elected Candidate”<sup>1</sup> means the Optioned Candidate selected by Celgene under the Master Collaboration Agreement that specifically targets the following Target Antigen: [                    ].

<sup>1</sup>     *To be updated by the Parties to specifically identify the candidate that is the subject of the option election .*

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**Appendix B**

**Applicable New In-Licenses**

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**Appendix C**

**Applicable Pre-Existing In-Licenses**

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**Appendix D**

**Certain Manufacturing Definitions**

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**Appendix E**

**Co-Co In-Licenses**



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**Appendix F**

**Profit & Loss Share**

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**Appendix G**

**Press Release**

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**Appendix H**

**Certain Patents within the Licensed IP Controlled  
by Bluebird as of the CCPS Agreement Effective Date**

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**Appendix J**

**Bluebird Agreements**

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**Appendix K**

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**Appendix L**

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**Appendix M**

**Redacted Version of CCPS Agreement**



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**Schedule 4.3(b)**

**Cost Allocation**

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**Schedule 5.6**

**Minimum BlueBird Sales Representative Qualifications**

- BS in Business or Science; 5+ years sales experience in pharmaceutical/biotechnology industry with at least two years of related hematology/oncology sales strongly preferred (or proven success in medical field).
- May not be debarred or disqualified by the FDA (or subject to a similar sanction by any Regulatory Authority outside the United States), or the subject of an FDA debarment or disqualification investigation or proceeding (or similar proceeding by any Regulatory Authority outside the United States), or convicted, indicted or charged with any crime that would constitute grounds for FDA debarment or disqualification (or similar sanctions by any Regulatory Authority outside the United States).
- Proven track record that demonstrates top sales accomplishments.
- Demonstrated ability to understand and communicate technical clinical material clearly and effectively.
- Demonstrated ability to develop critical relationships with physicians, nurses and ancillary staff within academic hospitals, clinics, and private practice facilities.
- Demonstrated understanding of oncology therapeutic area, products and marketplace.
- Demonstrated knowledge of healthcare system processes including reimbursement.

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**Schedule 16.2**

**Exceptions to Bluebird’s Representations and Warranties in Section 16.2**

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**Exhibit F**

**Additional Celgene Option Information**

Celgene will provide to Bluebird, along with the Option Exercise Notice:

- The clinical Development plan and commercial launch plan that Celgene is contemplating to achieve Regulatory Approval for such Optioned Candidate, together with the cost estimates for such a clinical program;
- The U.S. Development Budget, which for purposes of this Exhibit F will be for the first twelve (12) months of the Co-Development, Co-Promote and Profit Share Agreement. Celgene may update such U.S. Development Budget within ten (10) business days of first providing the same; and
- Such other supporting information related to the items listed in the foregoing bullet points as Bluebird may reasonably request, to the extent such information is in Celgene’s possession (for clarity, without any obligation to create or generate new information.)

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**Exhibit H**

**Redacted Master Collaboration Agreement**

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**Exhibit I**

**Press Release**

**bluebird bio Announces Global Strategic Collaboration with Celgene to Advance Gene Therapy in Oncology**

*- Separately, Celgene and bluebird bio to collaborate with the Center for Cell and Gene Therapy to advance new and existing CAR T cell programs*

CAMBRIDGE, Mass.—March XX, 2013 — bluebird bio, a privately-held biotechnology company focused on gene therapy, today announced the formation of a broad, global strategic collaboration with Celgene Corporation to discover, develop and commercialize novel disease-altering gene therapies in oncology. The collaboration will focus on applying gene therapy technology to genetically modify a patient’s own T-cells, known as chimeric antigen receptor (CAR) T-cells, to target and destroy cancer cells. The multi-year research and development collaboration has the potential to lead to the development and commercialization of multiple CAR T-cell products. Celgene has an option to license any products resulting from the collaboration after the completion of a Phase 1 clinical study for each such product. bluebird will be responsible for research and development activity through Phase 1 studies.

Simultaneous with this announcement, Celgene and bluebird bio also announced a related strategic collaboration in the CAR T-cell field with the Center for Cell and Gene Therapy at Baylor College of Medicine, Texas Children’s Hospital and The Methodist Hospital, Houston led by Malcolm Brenner, M.D., Ph.D., professor, Department of Molecular and Human Genetics and the director, Center for Cell and Gene Therapy. bluebird bio, Celgene and Dr. Brenner’s team will work collaboratively to advance and develop existing and new products and programs in the CAR T cell field.

“The genetic manipulation of autologous T cells is a new frontier in oncology, one that shows early promise in emerging clinical trials,” said Tom Daniel, President, Research & Early Development at Celgene. “We see strong prospects for this collaboration between Celgene, bluebird bio and Baylor College of Medicine’s experienced leaders in this emerging field, led by Dr. Brenner, to advance this innovative approach to intractable problems in oncology.”

“We believe that our recent advances in the industrialization of our gene therapy platform will drive improvements in the potency, purity, efficiency and scalability of our lentiviral gene therapy programs. These advances provide us with an opportunity to apply our platform, intellectual property and know-how to the development of additional product candidates in indications such as CAR T-cells for cancer,” stated Jeff Walsh, chief operating officer of bluebird bio. “Celgene is a global leader in oncology and, combined with Baylor’s expertise in the CAR T-cell field, we have created a great opportunity to drive innovation in a new and exciting area.”

Financial terms of the agreement include a substantial upfront payment and up to \$225 million per product in potential option fees and clinical and regulatory milestones. bluebird bio also has the right to participate in the development and commercialization of any licensed products resulting from the collaboration through a 50/50 co-development and profit share in the United States in exchange for a reduction of milestones. Royalties would also be paid in regions where there is no profit share including in the United States if bluebird declines to exercise their co-development and profit sharing rights.

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The gene therapy products currently in clinical development at bluebird bio for the treatment of childhood cerebral adrenoleukodystrophy, beta-thalassemia and sickle cell disease are independent of this collaboration.

Cowen and Company contributed as a strategic advisor to bluebird bio on this transaction.

**About CAR T-Cell Therapy**

CAR T-cell therapy represents a promising, emerging approach to treating cancer. Blood is withdrawn from a patient and the T-cells are then extracted from a patient’s blood. These cells are then genetically modified to recognize and attack cancer cells and then re-introduced into the patient’s blood. The patient’s genetically modified cells are intended to bind to and kill the target cancer cells.

**About bluebird bio**

bluebird bio is developing potentially transformative gene therapies for severe genetic and orphan diseases. bluebird bio has two clinical-stage programs in development for childhood cerebral adrenoleukodystrophy (CCALD) and beta-thalassemia/sickle cell disease. Led by a management team with extensive industry experience, bluebird bio is privately held and backed by top-tier life sciences investors. Its operations are located in Cambridge, Mass., San Francisco and Paris, France. For more information, please visit [www.bluebirdbio.com](http://www.bluebirdbio.com).

**About Celgene**

Celgene Corporation, headquartered in Summit, New Jersey, is an integrated global biopharmaceutical company engaged primarily in the discovery, development and commercialization of novel therapies for the treatment of cancer and inflammatory diseases through gene and protein regulation. For more information, please visit [www.celgene.com](http://www.celgene.com).

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## Exhibit L

### Call Option

#### 1. Call Option.

- 1.1 Notice. In the event that, during the Call Option Period, a Call Option Triggering Event occurs, then Bluebird will give Celgene written notice of such Call Option Triggering Event (a “Call Option Triggering Event Notice”). In the event that such Call Option Triggering Event occurring during the Call Option Period is a Bluebird Call Option Triggering Event, Bluebird will not commence a process intended to result in (or does result in) a Corporate Event until after delivery of the Call Option Triggering Event Notice to Celgene. For purposes of this Exhibit L, the “Call Option Period” means the period, if any, beginning on the date of termination of provisions set forth in Section 6.1 through 6.8 of the Agreement and, unless earlier terminated pursuant to Section 1.11 below, ending on the earliest to occur of: (i) expiration of the Initial Collaboration Term, unless Celgene has elected to extend the Initial Collaboration for the First Collaboration Program Extension pursuant to Section 2.1(d), and (ii) expiration of the First Collaboration Program Extension if Celgene has elected to extend the Initial Collaboration for the First Collaboration Program Extension pursuant to Section 2.1(d), even if Celgene has elected to extend the Collaboration Term for Second Collaboration Term Extension; provided that the Parties understand and agree that if the Call Option Period would otherwise terminate prior to the expiration of the applicable Call Option Exercise Period (as such Call Option Exercise Period may be extended by Bluebird from time to time) then the Call Option Period shall automatically be extended such that it shall expire on the same day as the expiration of the applicable Call Option Exercise Period (as such Call Option Exercise Period may be extended by Bluebird from time to time).
- 1.2 Exercise; Diligence Process. Celgene shall have the right, but not the obligation, to exercise the Call Option by delivery of written notice to Bluebird (the “Call Option Exercise Notice”) at any time prior to the expiration of the Call Option Exercise Period. Upon request made by Celgene during the Call Option Exercise Period, Bluebird will use its commercially reasonable efforts to provide Celgene with reasonable access to such scientific, technical, clinical, manufacturing, regulatory and other information and such personnel, at reasonable times and on a reasonable number of occasions, as may be reasonably necessary for Celgene to determine its own internal estimate of the Call Option Exercise Price. For clarity, Bluebird shall be under no obligation to share with Celgene its own internal estimate of the Call Option Exercise Price, if any. If Celgene fails to exercise the Call Option by delivery of written notice to Bluebird or otherwise delivers written notice to Bluebird of its decision not to exercise the Call Option, in either case, prior to the expiration of the Call Option Exercise Period, then (a) the procedures specified in Section 1.6 below will automatically terminate

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without any obligation on either Party to consummate the Call Option and (b) Bluebird shall be free to consummate a Corporate Event without any further obligation to Celgene under this Exhibit L, provided that, if a Corporate Event is not publicly announced within nine (9) months following the Call Option Triggering Event Notice described above, then, if during the remaining portion of the Call Option Period, a subsequent Call Option Triggering Event occurs, the procedures set forth in this Exhibit L will apply with respect to such subsequent Call Option Triggering Event.

- 1.3 Payment. If the Call Option has been exercised as of the expiration of the Call Option Exercise Period, then, effective as of the expiration of the Call Option Exercise Period, Celgene will be required to pay Bluebird at the Call Option Closing (defined below) an amount in cash equal to the Call Option Exercise Price determined in accordance with the procedures specified in Section 1.6 below which Call Option Exercise Price will be binding on both Parties (unless otherwise mutually agreed in accordance with Section 1.6(i)), subject only to the conditions and exceptions specified in Section 1.4 and Section 1.10 below. Subject to satisfaction of the conditions and exceptions specified in Section 1.4 and Section 1.10 below and final determination of the Call Option Exercise Price, Celgene will make such payment, and Bluebird will consummate the matters it is contemplated by this Exhibit L to perform on the date of such payment, all on the later of (a) the date of consummation of the applicable Corporate Event involving Bluebird (the “Other Closing”), and (b) ten (10) business days advance written notice by Bluebird to Celgene of such Other Closing (such later date, the “Call Option Closing”).
- 1.4 Government Approvals. The Call Option Closing and all payment obligations of Celgene set forth in Section 1.3 shall be conditioned upon Receipt of Regulatory Approvals. As used herein, “Receipt of Regulatory Approvals” means that all regulatory approvals required to consummate the transactions contemplated by the Call Option Closing shall have been obtained and shall remain in full force and effect through the Call Option Closing and all statutory waiting periods applicable to the Call Option Closing shall have expired or been terminated. Except for any Non-Required Remedy (defined below), each of Celgene and Bluebird shall use its commercially reasonable good faith efforts to obtain all requisite consents of any court or government authority regarding the exercise of the Call Option, including, if required by federal or state antitrust authorities, promptly taking all steps to secure government antitrust clearance, including cooperating in good faith with any government investigation including the prompt production of documents and information demanded by a second request for documents and of witnesses if requested, to the same extent and in the same manner (including with respect to the payment by Celgene of all fees required to be paid to any government authority in connection with any HSR Filing) as provided in Section 5.9 of this Agreement with respect to any proposed Development & Commercialization Agreement, *mutatis mutandis*. In the event that the Parties make an HSR Filing under this Section 1.4, then the Call Option shall terminate (i) at the election of either Party, immediately upon notice to the other Party, in the event that the United States of America Federal Trade Commission or the United States of America Department of Justice obtains a preliminary injunction under the HSR Act against the Parties to enjoin the

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transactions contemplated by this Exhibit L, or (ii) at the election of either Party, immediately upon written notice to the other Party, in the event that either the Call Option HSR Clearance Date or the Receipt of Regulatory Approvals shall not have occurred on or prior to one hundred twenty (120) days after the effective date of the HSR Filing (including expiration or termination of the applicable waiting period under the HSR Act or the inability to eliminate any Non-Required Remedy as part of any consent or approval). Notwithstanding anything to the contrary in this Agreement, in connection with the obligations contained in this Exhibit L, neither Party nor any of its respective Affiliates shall be required to sell, divest, hold separate, license or agree to any other structural or conduct remedy with respect to, any operations, divisions, businesses, product lines, customers, assets or relationships of such Party or any of its respective Affiliates (any such action, a “Non-Required Remedy”).

- 1.5 Fully Paid-Up, Non-Terminable Exclusive Licenses; Expense Reimbursement. Upon effectiveness of the Call Option Closing, the Parties covenant and agree that the following shall apply:
- a. This Agreement will terminate, provided that the provisions of Sections 12.4(a) and 12.4(c) will not apply with respect to such termination.
  - b. On the six-month anniversary of the Call Option Closing, each Co-Development, Co-Promote and Profit Share Agreement in effect at the time of the Call Option Closing will terminate (provided that, notwithstanding the foregoing, neither Party shall be responsible for any Profit & Loss Share payments that would otherwise have accrued from and after the Call Option Closing, the JGC and all subcommittees shall terminate as of the Call Option Closing and Celgene shall have sole discretion to make (and refrain from making) all decisions of the JGC and all subcommittees as of such date, and Bluebird shall have no further rights or obligations with respect to the performance of Development or Commercialization activities under the U.S. Development Plan or Worldwide Commercialization Plan), and, effective upon such termination, the Parties will enter into a License Agreement in the form attached hereto as Exhibit A with respect to the Optioned Candidate that was the subject of such Co-Development, Co-Promote and Profit Share Agreement, provided that (i) no royalties will be payable under Section 4.3 of the License Agreement and no Milestone Payments will be payable under Section 4.2 of the License Agreement, (ii) with respect to any In-License Payment that becomes due from and after the Call Option Closing, Celgene will reimburse Bluebird for one hundred percent (100%) of such payments within thirty (30) days of receipt of Bluebird’s written invoice therefor, (iii) all recovery-sharing provisions in Section 5.2 and Section 7.2 shall terminate, other than with respect to the provisions of Section 7.2(e) regarding reimbursement of Bluebird, and any other Third Party licensees of Bluebird, on a *pro rata* basis for each of their out-of-pocket costs and expenses incurred as a result of cooperating with Celgene as reasonably requested by Celgene in connection with the action (provided that, for clarity, the rest of the provisions in Section 5.2 and Section 7.2 shall remain in full force and effect, including Bluebird’s right to



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indemnification and reimbursement of Patent Costs if joined as a party to any action controlled by Celgene under Section 7.2), (iv) all of the licenses granted to Celgene to Licensed IP thereunder will become fully-paid up other than as set forth in subsection (ii) above, (v) all of the licenses granted to Celgene to Licensed IP thereunder will become perpetual and non-terminable other than with respect to Bluebird In-Licensed IP if, as a result of a breach by Celgene of its obligations under Section 2.8(c), the applicable Third Party licensor has the right to terminate an Applicable Bluebird In-License pursuant to which such Bluebird In-Licensed IP is licensed to Bluebird, (vi) all obligations of Celgene to commercialize any product will terminate, (vii) neither Celgene nor its Affiliates shall be subject to the exclusivity provision contained in Section 3.4, provided that if Celgene or its Affiliates are no longer pursuing the Development or Commercialization of the applicable Licensed Product (including any reasonable periods of customary delay), then the exclusivity provision contained in Section 3.4 shall no longer apply to Bluebird and its Affiliates (and Celgene will provide notice of same to Bluebird upon Bluebird’s written request), and after such time Celgene will not have any enforcement rights under clause (viii) below with respect to activities under clause (iii) of such “Competitive Infringement” definition, (viii) Celgene will have the exclusive right to institute and prosecute all enforcement claims under Section 7.2 with respect to “Competitive Infringement” (as defined in Section 7.1) for which Celgene has the first right to institute such claims (provided that, for clarity, the rest of the provisions in Section 7.2 shall remain in full force and effect, including Bluebird’s right to participate and be involved in any action controlled by Celgene under Section 7.2, but not a secondary right for Bluebird to institute any such enforcement claim with respect to Competitive Infringement), (ix) the technology transfer obligations under Section 2.3 of the License Agreement will include, but not be limited to, the obligation to transfer to Celgene complete and fully annotated Vector maps and sequences, including details of Payloads and any and all nucleic acid constructs, plasmids, cell lines and associated methodologies and production protocols, and (x) such License Agreement will be appropriately tailored to reflect the above. For clarity, and notwithstanding anything to the contrary herein, no license to Bluebird In-Licensed IP under any License Agreement described in this Section 1.5(b) shall be deemed to be fully-paid up, it being understood and agreed that in no event shall Celgene be relieved of any obligation to reimburse Bluebird for any In-License Payment within thirty (30) days of receipt of Bluebird’s written invoice therefor or any obligation to provide Bluebird with written reports pursuant to Section 4.4 in order to confirm any such required payments.

- c. The Parties will amend each License Agreement to provide that (i) no royalties will be payable under Section 4.3 of the License Agreement and no Milestone Payments will be payable under Section 4.2 of the License Agreement, (ii) with respect to any In-License Payment that becomes due from and after the Call Option Closing, Celgene will reimburse Bluebird for one hundred percent (100%) of such payments within thirty (30) days of receipt of Bluebird’s written invoice therefor, (iii) all recovery-sharing provisions in Section 5.2 and Section 7.2 shall terminate, other than with respect to the provisions of Section 7.2(e) regarding reimbursement of Bluebird, and any other Third

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Party licensees of Bluebird, on a *pro rata* basis for each of their out-of-pocket costs and expenses incurred as a result of cooperating with Celgene as reasonably requested by Celgene in connection with the action (provided that, for clarity, the rest of the provisions in Section 5.2 and Section 7.2 shall remain in full force and effect, including Bluebird’s right to indemnification and reimbursement of Patent Costs if joined as a party to any action controlled by Celgene under Section 7.2), (iv) all of the licenses granted to Celgene to Licensed IP thereunder will become fully-paid up other than as set forth in subsection (ii) above, (v) all of the licenses granted to Celgene to Licensed IP thereunder will become perpetual and non-terminable other than with respect Bluebird In-Licensed IP if, as a result of a breach by Celgene of its obligations under Section 2.8(c), the applicable Third Party licensor has the right to terminate an Applicable Bluebird In-License pursuant to which such Bluebird In-Licensed IP is licensed to Bluebird, (vi) all obligations of Celgene to commercialize any product will terminate, (vii) neither Celgene nor its Affiliates shall be subject to the exclusivity provision contained in Section 3.4, provided that if Celgene or its Affiliates are no longer pursuing the Development or Commercialization of the applicable Licensed Product (including any reasonable periods of customary delay), then the exclusivity provision contained in Section 3.4 shall no longer apply to Bluebird and its Affiliates (and Celgene will provide notice of same to Bluebird upon Bluebird’s written request), and after such time Celgene will not have any enforcement rights under clause (viii) below with respect to activities under clause (iii) of such “Competitive Infringement” definition, (viii) Celgene will have the exclusive right to institute and prosecute all enforcement claims under Section 7.2 with respect to “Competitive Infringement” (as defined in Section 7.1) for which Celgene has the first right to institute such claims (provided that, for clarity, the rest of the provisions in Section 7.2 shall remain in full force and effect, including Bluebird’s right to participate and be involved in any action controlled by Celgene under Section 7.2, but not a secondary right for Bluebird to institute any such enforcement claim with respect to Competitive Infringement), (ix) the technology transfer obligations under Section 2.3 of the License Agreement will include, but not be limited to, the obligation to transfer to Celgene complete and fully annotated Vector maps and sequences, including details of Payloads and any and all nucleic acid constructs, plasmids, cell lines and associated methodologies and production protocols, and (x) such License Agreement will be appropriately tailored to reflect the above. For clarity, and notwithstanding anything to the contrary herein, no license to Bluebird In-Licensed IP under any License Agreement described in this Section 1.5(c) shall be deemed to be fully-paid up, it being understood and agreed that in no event shall Celgene be relieved of any obligation to reimburse Bluebird for any In-License Payment within thirty (30) days of receipt of Bluebird’s written invoice therefor or any obligation to provide Bluebird with written reports pursuant to Section 4.4 in order to confirm any such required payments

- d. The Parties will enter into a License Agreement in the form attached hereto as Exhibit A with respect to each IND Product Candidate, Declined Product Candidate and Pre-IND Product Candidate, provided that (i) no royalties will be payable under Section 4.3 of the License Agreement and no Milestone Payments will be payable under Section 4.2 of the

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License Agreement, (ii) with respect to any In-License Payment that becomes due from and after the Call Option Closing, Celgene will reimburse Bluebird for one hundred percent (100%) of such payments within thirty (30) days of receipt of Bluebird’s written invoice therefor, (iii) all recovery-sharing provisions in Section 5.2 and Section 7.2 shall terminate, other than with respect to the provisions of Section 7.2(e) regarding reimbursement of Bluebird, and any other Third Party licensees of Bluebird, on a *pro rata* basis for each of their out-of-pocket costs and expenses incurred as a result of cooperating with Celgene as reasonably requested by Celgene in connection with the action (provided that, for clarity, the rest of the provisions in Section 5.2 and Section 7.2 shall remain in full force and effect, including Bluebird’s right to indemnification and reimbursement of Patent Costs if joined as a party to any action controlled by Celgene under Section 7.2), (iv) all of the licenses granted to Celgene to Licensed IP thereunder will become fully-paid up other than as set forth in subsection (ii) above, (v) all of the licenses granted to Celgene to Licensed IP thereunder will become perpetual and non-terminable other than with respect Bluebird In-Licensed IP if, as a result of a breach by Celgene of its obligations under Section 2.8(c), the applicable Third Party licensor has the right to terminate an Applicable Bluebird In-License pursuant to which such Bluebird In-Licensed IP is licensed to Bluebird, (vi) all obligations of Celgene to commercialize any product will terminate, (vii) neither Celgene nor its Affiliates shall be subject to the exclusivity provision contained in Section 3.4, provided that with respect to any Pre-IND Candidates any such exclusivity as applied to Bluebird and its Affiliates will be as provided in Section 12.4(c) of this Agreement, and provided further that if Celgene or its Affiliates are no longer pursuing the Development or Commercialization of the applicable Licensed Product (including any reasonable periods of customary delay), then the exclusivity provision contained in Section 3.4 shall no longer apply to Bluebird and its Affiliates (and Celgene will provide notice of same to Bluebird upon Bluebird’s written request), and after such time Celgene will not have any enforcement rights under clause (viii) below with respect to activities under clause (iii) of such “Competitive Infringement” definition, (viii) Celgene will have the exclusive right to institute and prosecute all enforcement claims under Section 7.2 with respect to “Competitive Infringement” (as defined in Section 7.1) for which Celgene has the first right to institute such claims (provided that, for clarity, the rest of the provisions in Section 7.2 shall remain in full force and effect, including Bluebird’s right to participate and be involved in any action controlled by Celgene under Section 7.2, but not a secondary right for Bluebird to institute any such enforcement claim with respect to Competitive Infringement), (ix) the technology transfer obligations under Section 2.3 of the License Agreement will include, but not be limited to, the obligation to transfer to Celgene complete and fully annotated Vector maps and sequences, including details of Payloads and any and all nucleic acid constructs, plasmids, cell lines and associated methodologies and production protocols, and (x) such License Agreement will be appropriately tailored to reflect the above. For clarity, and notwithstanding anything to the contrary herein, no license to Bluebird In-Licensed IP under any License Agreement described in this Section 1.5(d) shall be deemed to be fully-paid up, it being understood and agreed that in no event shall Celgene be relieved of any obligation to reimburse

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Bluebird for any In-License Payment within thirty (30) days of receipt of Bluebird’s written invoice therefor or any obligation to provide Bluebird with written reports pursuant to Section 4.4 in order to confirm any such required payments Celgene will reimburse Bluebird for all costs and expenses incurred by Bluebird in connection with transitioning clinical development responsibilities for such IND Product Candidates and Pre-IND Product Candidates from Bluebird to Celgene, within thirty (30) days of Celgene’s receipt of an invoice and a reasonably detailed invoice of such costs and expenses from Bluebird therefore.

- 1.6 Process for Determination of Call Option Exercise Price. The determination of the Call Option Exercise Price (which determination shall be binding in the event Celgene exercises the Call Option as provided in Section 1.2 above unless otherwise mutually agreed in accordance with Section 1.6(i)) will be made in accordance with the following process:
- a. A panel of three (3) independent third party firms each of which is either (i) a nationally-recognized investment banking firm or (ii) a nationally-recognized valuation firm (each a “Valuation Firm”) will be selected by Bluebird and Celgene as promptly as practicable in accordance with the procedures set forth below, but in no event more than five (5) calendar days after Celgene’s receipt of the Call Option Triggering Event Notice. The Parties will share equally in the costs of the Valuation Firms in this process (regardless of outcome).
  - b. Bluebird and Celgene will mutually appoint one Valuation Firm who (A) in the prior two (2) years has not had, does not have and does not anticipate having any material commercial or other business relationship with Bluebird or Celgene or any of their respective Affiliates and (B) with the respect to the lead representative in charge of such assignment contemplated by this Section 1 on behalf of such Valuation Firm (the “Valuation Manager”), such Valuation Manager has at least ten (10) years professional experience in the valuation of biopharmaceutical development programs as an investment banker, valuation expert or healthcare consultant (such experience referenced in this clause (B), the “Requisite Experience”), which Valuation Firm shall be proposed by Bluebird and approved by Celgene (which approval shall not be unreasonably withheld or delayed) (the “Bluebird Designee”).
  - c. Celgene and Bluebird will mutually appoint one Valuation Firm who (A) in the prior two (2) years has not had, does not have and does not anticipate having any material commercial or other business relationship with Bluebird or Celgene or any of their respective Affiliates, and (B) with respect to the applicable Valuation Manager, such Valuation Manager has the Requisite Experience, which Valuation Firm shall be proposed by Celgene and approved by Bluebird (which approval shall not be unreasonably withheld or delayed) (the “Celgene Designee”).
  - d. Celgene and Bluebird will mutually appoint a third Valuation Firm who (A) in the prior two (2) years has not had, does not have and does not anticipate having any material

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commercial or other business relationship with Bluebird or Celgene or any of their respective Affiliates, and (B) with respect to the applicable Valuation Manager, such Valuation Manager has the Requisite Experience, which Valuation Firm must be mutually approved by both Bluebird and Celgene (which approval shall not be unreasonably withheld or delayed by either Party) (the “Final Designee”).

- e. The engagement of the Valuation Firms selected shall be limited to the determination of the Call Option Exercise Price. In determining the Call Option Exercise Price, the Valuation Firms shall render their judgment as to what an independent Third Party would pay to Bluebird to acquire its rights and assume its obligations (including the unilateral exclusivity afforded to Celgene under the agreements referenced above and the In-License Payments that become due and payable by Celgene from and after the Call Option Closing, but excluding the Target Antigen License and all other rights described in Section 1.8 below), including without limitation the right to receive future payments and perform obligations under any existing Co-Development, Co-Promote and Profit Share Agreement or License Agreement for Licensed Products (which shall take into account the rights and obligations associated solely with the co-promotion of Licensed Products by such independent Third Party and not any other products such Third Party may have the right to promote or sell) as well as for any IND Product Candidate, Declined Product Candidate or Pre-IND Product Candidate that is not the subject of an existing Co-Development, Co-Promote and Profit Share Agreement or License Agreement, as set forth in Section 1.5; it being understood that in no event will the Call Option Exercise Price include any value attributable to the Target Antigen License or other rights described in Section 1.8 below. Each of the Valuation Firms selected shall agree to complete the terms of its engagement within the time period prescribed hereunder. Each of Bluebird and Celgene shall provide reasonable access to its information and personnel, at reasonable times and on a reasonable number of occasions, to the Valuation Firms as may be reasonably necessary for the Valuation Firms to determine the Call Option Exercise Price, upon such Valuation Firm entering into a confidentiality agreement on terms reasonably satisfactory to the disclosing Party.
- f. In the event that Celgene fails to exercise the Call Option by delivery of the Call Option Exercise Notice to Bluebird or otherwise delivers written notice to Bluebird of its decision not to exercise the Call Option, in either case, prior to the expiration of the Call Option Exercise Period, then the procedures specified in this Section 1.6 will automatically terminate, without any obligation on either Party to consummate the Call Option.
- g. In the event that Celgene exercises the Call Option by delivery of the Call Option Exercise Notice to Bluebird prior to the expiration of the Call Option Exercise Period, then no later than the expiration of the Call Option Exercise Period, each Party will have the opportunity to provide the Valuation Firms, on a confidential basis, with a written memorandum (not to exceed [\*\*\*] words), including supporting analysis and documentary evidence where appropriate, which such Party deems appropriate to assist

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the Valuation Firms in determining the Call Option Exercise Price, provided that neither Party shall submit a proposed Call Option Exercise Price. Neither Party shall be entitled to review any such memorandum or supporting analysis and documentary evidence submitted by the other Party.

- h. The Valuation Firms will have the right to meet with the Parties as necessary to inform the Valuation Firms’ determination of the Call Option Exercise Price. Upon the expiration of the Valuation Period (the “Initial Valuation Determination Date”), each of the Bluebird Designee and the Celgene Designee will provide to both Parties their respective determinations of the Call Option Exercise Price. The final Call Option Price will be the average of these two amounts, unless one of the Call Option Prices submitted exceeds the other Call Option Price by [\*\*\*] or more, in which case both Call Option Prices shall then be submitted to the Final Designee which Valuation Firm shall determine, no later than two (2) business days after the Initial Valuation Determination Date (the “Final Valuation Determination Date”), a final Call Option Price [\*\*\*]. The final Option Price, as determined in accordance with the foregoing process, will be binding and enforceable on the Parties.
  - i. Notwithstanding any other provision of this Section 1.6, the Parties may at any time following Celgene’s receipt of the Call Option Triggering Event Notice and during the valuation process but prior to the Initial Valuation Determination Date or the Final Valuation Date, as applicable, engage in negotiations to determine a mutually-satisfactory Call Option Exercise Price. In the event the Parties agree in writing as to the amount of the Call Option Exercise Price, the valuation process described in this Section 1.6 shall terminate and the Call Option Exercise Price shall be the amount agreed to in writing by the Parties which amount shall be binding and enforceable on the Parties.
- 1.7 Miscellaneous Provisions Relating to Exercise of the Call Option. In connection with the exercise and consummation of the Call Option as contemplated herein:
- a. The Parties will take or cause to be taken all such actions as may be reasonably necessary or desirable in order to give effect to the actions described in this Exhibit L and any related transactions, including executing, acknowledging and delivering consents, assignments, waivers and other documents and instruments; furnishing information and copies of documents; filing applications, reports, returns, filings and other documents or instruments with governmental authorities; and otherwise cooperating with the other Party as applicable. For clarity, Bluebird covenants and agrees to transfer all of its rights to receive payments contemplated to be transferred to Celgene at the Call Option Closing free of all liens, charges and other encumbrances (and Bluebird shall be financially responsible for eliminating and satisfying all such liens, charges and encumbrances).
  - b. Except as otherwise expressly provided in this Exhibit L (including the requirement of Celgene to pay all fees required to be paid to any government authority in connection

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with any HSR Filing as set forth in Section 1.4 above), each of the Parties will be responsible for its own costs and expenses incurred in connection with the transactions contemplated by this Exhibit L (whether or not consummated), including all attorneys’ fees and charges, all accounting fees and charges and all investment banking fees, charges or commissions.

- c. Any information disclosed to Celgene or Bluebird in accordance with this Exhibit L, including the information provided in accordance with Section 1.2 above, and the existence of a Call Option Triggering Event or the fact that discussions or negotiations are taking place concerning the Call Option, or any of the terms, conditions or other facts with respect thereto, or the outcome thereof, shall (i) be deemed to be the Confidential Information of the disclosing Party, (ii) be used by the recipient Party solely for purposes of evaluating and exercising its interest and obligations regarding the Call Option and the matters referred to in this Exhibit L and for no other purpose, and (iii) shall otherwise be subject to the restrictions on disclosure and exceptions thereto set forth in Section 10 of this Agreement.

1.8 Target Antigen License if Call Option Exercised Prior to Third Anniversary of the Effective Date . In the event that Celgene exercises the Call Option by delivering the Call Option Exercise Notice to Bluebird prior to the expiration of the applicable Call Option Exercise Period and prior to the third anniversary of the Effective Date of the Agreement, then upon the effectiveness of the Call Option Closing, if any, in addition to the consummation of the matters contemplated by Section 1.5 above in consideration of the payment required to be made as contemplated by Section 1.3 above, the Parties will also enter into a License Agreement in the form attached hereto as Exhibit A, modified as follows:

- a. Bluebird will grant Celgene a perpetual, non-terminable, worldwide, exclusive (even as to Bluebird and its Affiliates) license, with the right to sublicense as permitted by Section 3.4 of such License Agreement, under Licensed IP to Develop and Commercialize Licensed Products in the Field, provided that such license will not be non-terminable with respect to Bluebird In-Licensed IP if, as a result of a breach by Celgene of its obligations under Section 2.8(c), the applicable Third Party licensor has the right to terminate an Applicable Bluebird In-License pursuant to which such Bluebird In-Licensed IP is licensed to Bluebird;
  - i. “Licensed IP” will be defined to mean Patents, Materials and Know-How Controlled by Bluebird or any of its Affiliates (including any applicable Collaboration IP and Bluebird Technology), including Bluebird In-Licensed IP, that are necessary or useful to Develop and Commercialize Licensed Products;
  - ii. “Licensed Products” will be defined to mean any and all therapeutic candidates in the Field; and

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- iii. “Target Antigens” will be defined to mean any and all oncology associated antigens, provided that from and after the third anniversary of the Effective Date of the Agreement, “Target Antigens” will be limited to the oncology associated antigens designated by Celgene in a written notice to Bluebird as those oncology associated antigens for which Celgene reasonably intends to Develop Licensed Products, which written notice will be delivered to Bluebird within thirty (30) days after the third anniversary of the Effective Date of the Agreement, provided further that from and after the fifth anniversary of the Effective Date of the Agreement, “Target Antigens” will be limited to the oncology associated antigens “in development” and identified as such by Celgene in a written notice to Bluebird, which written notice will be delivered to Bluebird within thirty (30) days after the fifth anniversary of the Effective Date of the Agreement. For purposes of this Section 1.8(a)(iii), an oncology associated antigen will be deemed “in development” when at least one in vitro assay of a chimeric antigen receptor (CAR) construct targeting such oncology associated antigen, or of a genetically modified T cell targeting such oncology associated antigen, has been initiated;
- b. with respect to each Licensed Product, (i) Celgene will pay a royalty of [\*\*\*] percent ([\*\*\*]%) of worldwide Net Sales of such Licensed Product under Section 4.3(a) of the License Agreement (subject to the provisions of Sections 4.3(b)-(f)), and no other royalties will be payable under Section 4.3 of the License Agreement, (ii) Celgene will pay a milestone payment of [\*\*\*] (U.S.\$[\*\*\*]) under Section 4.2 of the License Agreement upon the earlier of (A) Regulatory Approval of a BLA in the United States by the FDA for such Licensed Product or (B) Regulatory Approval of an MAA by the EMA for such Licensed Product, and no other milestone payments will be payable under Section 4.2 of the License Agreement (including for any Licensed Product that is approved for multiple indications), provided that it is further agreed that no milestone payment will be payable for any improved or modified version of a Licensed Product for which the [\*\*\*] (U.S.\$[\*\*\*]) previously had been paid;
- c. with respect to any In-License Payment that becomes due from and after the Call Option Closing, Celgene will reimburse Bluebird for one hundred percent (100%) of such payments within thirty (30) days of receipt of Bluebird’s written invoice therefor;
- d. all recovery-sharing provisions in Section 5.2 and Section 7.2 shall terminate, other than with respect to the provisions of Section 7.2(e) regarding reimbursement of Bluebird, and any other Third Party licensees of Bluebird, on a pro rata basis for each of their out-of-pocket costs and expenses incurred as a result of cooperating with Celgene as reasonably requested by Celgene in connection with the action (provided that, for clarity, the rest of the provisions in Section 5.2 and Section 7.2 shall remain in full force and effect, including Bluebird’s right to indemnification and reimbursement of Patent Costs if joined as a party to any action controlled by Celgene under Section 7.2);



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- e. all obligations of Celgene to develop and/or commercialize any product will terminate, and Sections 2.6 (Annual Update Meetings), 2.7 (Reports by Celgene) and 2.8(b) (Maintenance of Celgene Licensed Product In-Licenses) will not apply;
- f. Bluebird and its Affiliates will be subject to the exclusivity provision contained in Section 3.4 with respect to all Target Antigens and products that target the Target Antigens (removing the reference to Elected Candidates), provided that if, after five (5) years from the Effective Date, Celgene or its Affiliates are no longer pursuing the Development (it being understood that if a Licensed Product is “in development” (as defined in Section 1.8(a)(iii)), then Celgene will be deemed to be pursuing the Development of such Licensed Product) or Commercialization of the applicable Licensed Product (including any reasonable periods of customary delay), then the exclusivity provision contained in Section 3.4 shall no longer apply to Bluebird and its Affiliates with respect to such applicable Licensed Product and the Target Antigen targeted by such applicable Licensed Product (but only if Celgene is not pursuing the Development or Commercialization of other Licensed Products targeting such Target Antigen) (and Celgene will provide notice of same to Bluebird upon Bluebird’s written request), and after such time Celgene will not have any enforcement rights under clause (g) below with respect to activities under clause (iii) of such “Competitive Infringement” definition, and neither Celgene nor its Affiliates shall be subject to the exclusivity provision contained in Section 3.4;
- g. Celgene will have the exclusive right to institute and prosecute all enforcement claims under Section 7.2 with respect to “Competitive Infringement” (as defined in Section 7.1) for which Celgene has the first right to institute such claims (provided that, for clarity, the rest of the provisions in Section 7.2 shall remain in full force and effect, including Bluebird’s right to participate and be involved in any action controlled by Celgene under Section 7.2, but not a secondary right for Bluebird to institute any such enforcement claim with respect to Competitive Infringement);
- h. the technology transfer obligations under Section 2.3 of the License Agreement will include, but not be limited to, the obligation to transfer to Celgene complete and fully annotated Vector maps and sequences, including details of Payloads and any and all nucleic acid constructs, plasmids, cell lines and associated methodologies and production protocols;
- i. such License Agreement will be appropriately tailored to reflect the above; and
- j. For clarity, and notwithstanding anything to the contrary herein, no license to Bluebird In-Licensed IP under any License Agreement described in this Section 1.8 shall be deemed to be fully-paid up, it being understood and agreed that in no event shall Celgene be relieved of any obligation to reimburse Bluebird for any In-License Payment within thirty (30) days of receipt of Bluebird’s written invoice therefor or any obligation to provide Bluebird with written reports pursuant to Section 4.4 in order to confirm any such required payments.

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For avoidance of doubt, the License Agreement contemplated by this Section 1.8 is separate from any amended License Agreement executed pursuant to Section 1.5(c) above or License Agreement entered into pursuant to Sections 1.5(b) or 1.5(d) above, and the terms of this Section 1.8 shall not apply to any License Agreement amended pursuant to Section 1.5(c) above or entered into pursuant to Sections 1.5(b) or 1.5(d) above.

- 1.9 License Agreements. Further, with respect to any License Agreement amended pursuant to Section 1.5(c) above or entered into pursuant to Sections 1.5(b), 1.5(d) or 1.8(a) above, (i) Bluebird will have no obligation to continue the performance of any Phase 1 Study under Section 2.1 (a) of such License Agreement, (ii) Section 11.2(a) of such License Agreement will apply to the items listed therein based on the applicable Corporate Event, and (iii) the exclusivity restrictions in Section 3.4 of such License Agreement will continue to be subject to the exceptions set forth in such Section 3.4.
- 1.10 Expiration of Call Right Exercise. In the event that Celgene exercises the Call Option by delivery of written notice to Bluebird prior to the expiration of the Call Option Exercise Period and (1) Bluebird gives Celgene written notice that the applicable Call Option Triggering Event is no longer in effect or (2) a Corporate Event is not publicly announced within nine (9) months following the applicable Call Option Triggering Event Notice, then (a) the above-mentioned Call Option Exercise Notice shall be deemed rescinded, null and void, and (b) if during the remaining portion of the Call Option Period, a subsequent Call Option Triggering Event occurs, the procedures set forth in this Exhibit L will apply with respect to such subsequent Call Option Triggering Event.
- 1.11 Termination. Celgene’s right to exercise the Call Option as set forth in this Exhibit L will terminate upon the earlier of:
  - a. the consummation of a Corporate Event unless a Call Option Exercise Notice has been timely delivered by Celgene in accordance with Section 1.2 following a Call Option Triggering Event, prior to the expiration or termination of the Call Option Exercise Period and prior to consummation of such Corporate Event, in which case Celgene’s right to exercise the Call Option shall continue as contemplated by this Exhibit L (it being understood and agreed that, in the event of any failure by Bluebird to comply with the terms and conditions of Sections 1.1 and 1.2, Celgene’s right to exercise the Call Option shall not terminate);
  - b. the expiration or termination of the Call Option Period unless a Call Option Exercise Notice has been timely delivered by Celgene in accordance with Section 1.2 following a Call Option Triggering Event and prior to the expiration or termination of the Call Option Exercise Period, in which case Celgene’s right to exercise the Call Option shall continue as contemplated by this Exhibit L; and

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- c. the expiration or termination of this Agreement unless a Call Option Exercise Notice has been timely delivered by Celgene in accordance with Section 1.2 following a Call Option Triggering Event, prior to the expiration or termination of the Call Option Exercise Period and prior to the expiration or termination of this Agreement, in which case Celgene’s right to exercise the Call Option shall continue as contemplated by this Exhibit L.

1.12 Attorneys’ Fees. In any suit or proceeding between the Parties brought in accordance with Section 13.1 of the Agreement that relates to an allegation that a Party has breached its obligations under this Exhibit L in a manner that adversely affects the other Party’s rights hereunder with respect to the exercise and execution of the Call Option, the prevailing Party will have the right to recover from the other Party its documented costs and reasonable fees and expenses of attorneys, accountants, and other professionals incurred in connection with the suit or proceeding. For clarity, this Section 1.12 will apply solely with respect to suits or proceedings relating to the exercise and execution of the Call Option, and does not apply with respect to any other suit or proceeding related to or arising out of (i) this Agreement, (ii) any Development & Commercialization Agreement, or (iii) any License Agreement entered into pursuant to Section 1.8 above, amended License Agreement executed pursuant to Section 1.5(c) above or License Agreement entered into pursuant to Sections 1.5(b) or 1.5(d) above.

1.13 Definitions. For purposes of this Exhibit L:

- a. “Bluebird Call Option Triggering Event” means any determination by the board of directors of Bluebird to commence a process reasonably intended to result (or does then result) in a Corporate Event.
- b. “Call Option” means (i) Celgene’s right to acquire the licenses together with the other matters described in Section 1.5 in consideration of the payment by Celgene of the Call Option Exercise Price in accordance with the procedures described in this Exhibit L and (ii) if, and only if, the Call Option is exercised prior to the third anniversary of the Effective Date of the Agreement, Celgene’s right to acquire the Target Antigen License together with the other matters described in Section 1.8 in consideration of the payment obligations set forth in Section 1.8 in accordance with the procedures described in this Exhibit L.
- c. “Call Option Exercise Period” means the twelve (12) calendar day period following Celgene’s receipt of the Call Option Triggering Event Notice, provided that Bluebird may extend the Call Option Exercise Period as follows: (i) in ten (10) day increments following the end of the then-existing “Call Option Exercise Period” by delivery of written notice to Celgene at any time prior to the fifth (5<sup>th</sup>) day prior to the expiration of such Call Option Exercise Period (as it may be extended from time to time), and (ii) the extensions, when aggregated together, may not exceed 240 days in the aggregate. In the event the Call Option Exercise Period is extended as provided in the immediately

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preceding sentence, the Valuation Period will also be extended for a like number of calendar days. In the event that the Call Option Exercise Period is scheduled to expire on a calendar day that is not a business day, then the Call Option Exercise Period shall automatically be extended until the next business day.

- d. “Call Option HSR Clearance Date” means the earliest date on which the Parties have actual knowledge that all applicable waiting periods under the HSR Act with respect to the transactions contemplated by the Call Option have expired or have been terminated.
- e. “Call Option Triggering Event” means (i) a Bluebird Call Option Triggering Event or (ii) a Third Party Call Option Triggering Event.
- f. “Third Party Call Option Triggering Event” means (i) the receipt by Bluebird of a *bona fide* written offer by a Third Party with respect to a Corporate Event subsequent to which the board of directors of Bluebird determines, subject to compliance with this Agreement, to enter into negotiations with such Third Party with respect to such Corporate Event or (ii) the public announcement by a Third Party of an intention to effect a Corporate Event.
- g. “Valuation Period” means the fifteen (15) calendar day period following Celgene’s receipt of the Call Option Triggering Event Notice, subject to extension as set forth in Section 1.13(c) above, provided that Bluebird may further extend the Valuation Period by delivery of written notice to Celgene at any time prior to the expiration of the Valuation Period (as it may be extended from time to time) as and to the extent permitted by Section 1.13(c) above. In the event that the Valuation Period is scheduled to expire on a calendar day that is not a business day, then the Valuation Period shall automatically be extended until the next business day.

## BLUEBIRD BIO, INC.

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**EXECUTIVE CASH INCENTIVE BONUS PLAN**1. Purpose

This Executive Cash Incentive Bonus Plan (the “*Incentive Plan*”) is intended to provide an incentive for superior work and to motivate eligible executives of bluebird bio, Inc. (the “*Company*”) and its subsidiaries toward even higher achievement and business results, to tie their goals and interests to those of the Company and its stockholders and to enable the Company to attract and retain highly qualified executives. The Incentive Plan is for the benefit of Covered Executives (as defined below).

2. Covered Executives

From time to time, the Compensation Committee of the Board of Directors of the Company (the “*Compensation Committee*”) may select certain key executives (the “*Covered Executives*”) to be eligible to receive bonuses hereunder. Participation in this Plan does not change the “at will” nature of a Covered Executive’s employment with the Company.

3. Administration

The Compensation Committee shall have the sole discretion and authority to administer and interpret the Incentive Plan.

4. Bonus Determinations

(a) Corporate Performance Goals. A Covered Executive may receive a bonus payment under the Incentive Plan based upon the attainment of one or more performance objectives that are established by the Compensation Committee and relate to financial and/or operational metrics with respect to the Company or any of its subsidiaries (the “*Corporate Performance Goals*”), including the following: achievement of specified research and development, publication, clinical and/or regulatory milestones, cash flow (including, but not limited to, operating cash flow and free cash flow); sales or revenue; corporate revenue; earnings before interest, taxes, depreciation and amortization; net income (loss) (either before or after interest, taxes, depreciation and/or amortization); changes in the market price of the Company’s common stock; economic value-added; funds from operations or similar measure; acquisitions or strategic transactions; operating income (loss); return on capital, assets, equity, or investment; stockholder returns; return on sales; gross or net profit levels; productivity; expense efficiency; margins; operating efficiency; customer satisfaction; working capital; earnings (loss) per share of the Company’s common stock; bookings, new bookings or renewals; sales or market shares; number of customers, number of new customers or customer references; operating income and/or net annual recurring revenue, any of which may be (A) measured in absolute terms or compared to any incremental increase, (B) measured in terms of growth, (C) compared to another company or companies or to results of a peer group, (D) measured against the market as a whole and/or as

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compared to applicable market indices and/or (E) measured on a pre-tax or post-tax basis (if applicable). Further, any Corporate Performance Goals may be used to measure the performance of the Company as a whole or a business unit or other segment of the Company, or one or more product lines or specific markets. The Corporate Performance Goals may differ from Covered Executive to Covered Executive.

(b) Calculation of Corporate Performance Goals. At the beginning of each applicable performance period, the Compensation Committee will determine whether any significant element(s) will be included in or excluded from the calculation of any Corporate Performance Goal with respect to any Covered Executive. In all other respects, Corporate Performance Goals will be calculated in accordance with the Company's financial statements, generally accepted accounting principles, or under a methodology established by the Compensation Committee at the beginning of the performance period and which is consistently applied with respect to a Corporate Performance Goal in the relevant performance period.

(c) Target; Minimum; Maximum. Each Corporate Performance Goal shall have a "target" (e.g., 100 percent attainment of the Corporate Performance Goal) and may also have a "minimum" hurdle and/or a "maximum" amount.

(d) Bonus Requirements; Individual Goals. Except as otherwise set forth in this Section 4(d), to the extent practicable under the circumstances: (i) any bonuses paid to Covered Executives under the Incentive Plan shall be based upon objectively determinable bonus formulas that tie such bonuses to one or more performance targets relating to the Corporate Performance Goals, (ii) bonus formulas for Covered Executives shall be adopted in each performance period by the Compensation Committee and communicated to each Covered Executive at the beginning of each performance period and (iii) no bonuses shall be paid to Covered Executives unless and until the Compensation Committee makes a determination with respect to the attainment of the performance targets relating to the Corporate Performance Goals. Notwithstanding the foregoing, the Compensation Committee may adjust bonuses payable under the Incentive Plan based on achievement of one or more individual performance objectives or pay bonuses (including, without limitation, discretionary bonuses) to Covered Executives under the Incentive Plan based on individual performance goals and/or upon such other terms and conditions as the Compensation Committee may in its discretion determine.

(e) Individual Target Bonuses. The Compensation Committee shall establish a target bonus opportunity for each Covered Executive for each performance period. For each Covered Executive, the Compensation Committee shall have the authority to apportion the target award so that a portion of the target award shall be tied to attainment of Corporate Performance Goals and a portion of the target award shall be tied to attainment of individual performance objectives.

(f) Employment Requirement. Subject to any additional terms contained in a written agreement between the Covered Executive and the Company, the payment of a bonus to a Covered Executive with respect to a performance period shall be conditioned upon the Covered Executive's employment by the Company on the bonus payment date. If a Covered Executive was not employed for an entire performance period, the Compensation Committee may pro rate the bonus based on the number of days employed during such period.

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5. Timing of Payment

(a) The Corporate Performance Goals will be measured at the end of each performance period after the Company's financial reports with respect to such period(s) have been published. If the Corporate Performance Goals and/or individual goals for such period are met, payments will be made as soon as practicable following the end of such period, but not later 74 days after the end of the fiscal year in which such performance period ends.

(b) With respect to Corporate Performance Goals established and measured on an annual or multi-year basis, Corporate Performance Goals will be measured as of the end of each such performance period (e.g., the end of each fiscal year) after the Company's financial reports with respect to such period(s) have been published. If the Corporate Performance Goals and/or individual goals for any such period are met, bonus payments will be made as soon as practicable, but not later than 74 days after the end of the relevant fiscal year.

(c) For the avoidance of doubt, bonuses earned at any time in a fiscal year must be paid no later than 74 days after the last day of such fiscal year.

6. Amendment and Termination

The Company reserves the right to amend or terminate the Incentive Plan at any time in its sole discretion.

**ADOPTED BY THE BOARD OF DIRECTORS: May 5, 2013**

**Subsidiaries of Registrant**

<u>Name</u>	<u>Jurisdiction of Incorporation</u>
bluebird bio France – SARL	France
bluebird bio Securities Corporation	Massachusetts



**Consent of Independent Registered Public Accounting Firm**

We consent to the reference to our firm under the caption "Experts" and to the use of our report dated March 21, 2013, in the Registration Statement (Form S-1) and related Prospectus of bluebird bio, Inc. dated May 14, 2013.

/s/ Ernst & Young LLP

Boston, Massachusetts  
May 14, 2013

**Consent of Independent Registered Public Accounting Firm**

We consent to the use in this Registration Statement on Form S-1 of bluebird bio, Inc. of our report dated March 21, 2013, relating to our audit of the consolidated financial statements, appearing in the Prospectus, which is part of this Registration Statement.

We also consent to the reference to our firm under the captions "Experts" in such Prospectus.

/s/ McGladrey LLP

Boston, Massachusetts

May 14, 2013