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

# FORM 8-K

**CURRENT REPORT**
**Pursuant to Section 13 or 15(d)**
**of the Securities Exchange Act of 1934**

**Date of Report (Date of earliest event reported): January 9, 2023**

# bluebird bio, Inc.
**(Exact name of registrant as specified in its charter)**

| **Delaware** | **001-35966** | **13-3680878** |
|---|---|---|
| **(State or other jurisdiction of incorporation)** | **(Commission File Number)** | **(IRS Employer Identification Number)** |

**455 Grand Union Boulevard,**
**Somerville, MA**
**02145**
**(Address of principal executive offices) (Zip Code)**

**Registrant's telephone number, including area code: (339) 499-9300**

**Not Applicable**
**(Former name or former address, if changed since last report)**

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

| Title of each class | Trading Symbol(s) | Name of each exchange on which registered |
|---|---|---|
| Common Stock, $0.01 par value per share | BLUE | The Nasdaq Stock Market LLC |

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter). Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

**Item 2.02. Results of Operations and Financial Condition.**

On January 9, 2023, bluebird bio, Inc. (the "Company") announced that as of December 31, 2022, the Company's cash, cash equivalents and marketable securities were approximately $182 million, excluding restricted cash of approximately $45 million, which was not released in the fourth quarter of 2022.

The cash, cash equivalents and marketable securities information above is based on preliminary unaudited information and management estimates for the year ended December 31, 2022, is not a comprehensive statement of the Company's financial results as of and for the fiscal year ended December 31, 2022, and is subject to completion of the Company's financial closing procedures. The Company's independent registered public accounting firm has not conducted an audit or review of, and does not express an opinion or any other form of assurance with respect to, this preliminary estimate.

The information contained in this item is being furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended (the "Securities Act"), or the Exchange Act, except as expressly set forth by specific reference in such filing.

**Item 7.01. Regulation FD Disclosure.**

The Company plans to present a corporate update on January 12, 2023 at the 2023 J.P. Morgan Healthcare Conference. A copy of the presentation that will be used is being furnished as Exhibit 99.1, which is incorporated herein by reference.

The information contained in this item is being furnished and shall not be deemed "filed" for purposes of Section 18 of the Exchange Act or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act or the Exchange Act except as expressly set forth by specific reference in such filing.

**Item 9.01. Financial Statements and Exhibits.**

(d) Exhibits

| Exhibit No. | Description |
|---|---|
| 99.1 | Corporate Presentation by bluebird bio, Inc. |
| 104 | Cover Page Interactive Data File (embedded within the inline XBRL document). |

***Forward-Looking Statements***

*This Current Report on Form 8-K (the "Current Report") contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this Current Report that do not relate to matters of historical fact should be considered forward-looking statements, including without limitation statements regarding the Company's preliminary unaudited cash position as of December 31, 2022. Statements using words such as "expect", "anticipate", "believe", "may", "will" and similar terms are also forward-looking statements. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause the Company's actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, the important factors discussed under the caption "Risk Factors" in the Company's Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2022 and its other filings with the Securities and Exchange Commission. Except as required by law, the Company undertakes no obligations to make any revisions to the forward-looking statements contained in this Current Report or to update them to reflect events or circumstances occurring after the date of this Current Report, whether as a result of new information, future developments or otherwise.*

**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

**bluebird bio, Inc.**

Date: January 9, 2023

By: /s/ Andrew Obenshain
Andrew Obenshain
President and Chief Executive Officer

# bluebird bio J.P. Morgan Presentation

January 2023

**NASDAQ: BLUE**

# forward-looking statements

These slides and the accompanying oral presentation contain forward-looking statements and information. The use of words such as “may,” “might,” “will,” “should,” “expect,” “plan,” “anticipate,” “believe,” “estimate,” “project,” “intend,” “future,” “potential,” or “continue,” and other similar expressions are intended to identify forward-looking statements. For example, all statements we make regarding our expectations regarding our programs and therapies, including but not limited to the timing or likelihood of regulatory filings and approvals, our manufacturing and commercialization plans, and addressable market for approved products or product candidates, the timing of our first revenue, our preliminary unaudited cash position as of December 31, 2022, and our cash runway are forward looking. All forward-looking statements are based on estimates and assumptions by our management that, although we believe to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that we expected. These statements are also subject to a number of material risks and uncertainties that are described in our most recent quarterly report on Form 10-Q, as well as our subsequent filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. We undertake no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

bluebirdbio

*pursuing Curative gene Therapies ...*

TO GIVE PATIENTS AND THEIR FAMILIES MORE BLUEBIRD DAYS

# Demonstrating gene therapy expertise across clinical, regulatory and commercial

## Clinical Leadership

**180+ patients** treated with bluebird therapies across 8 clinical trials

**Over 10+ years** of gene therapy research

## Regulatory Success

Industry leader with **2 FDA approved gene therapies and seeking 3rd** in 2023

Established track record for LVV technology, **with 5 regulatory submissions**

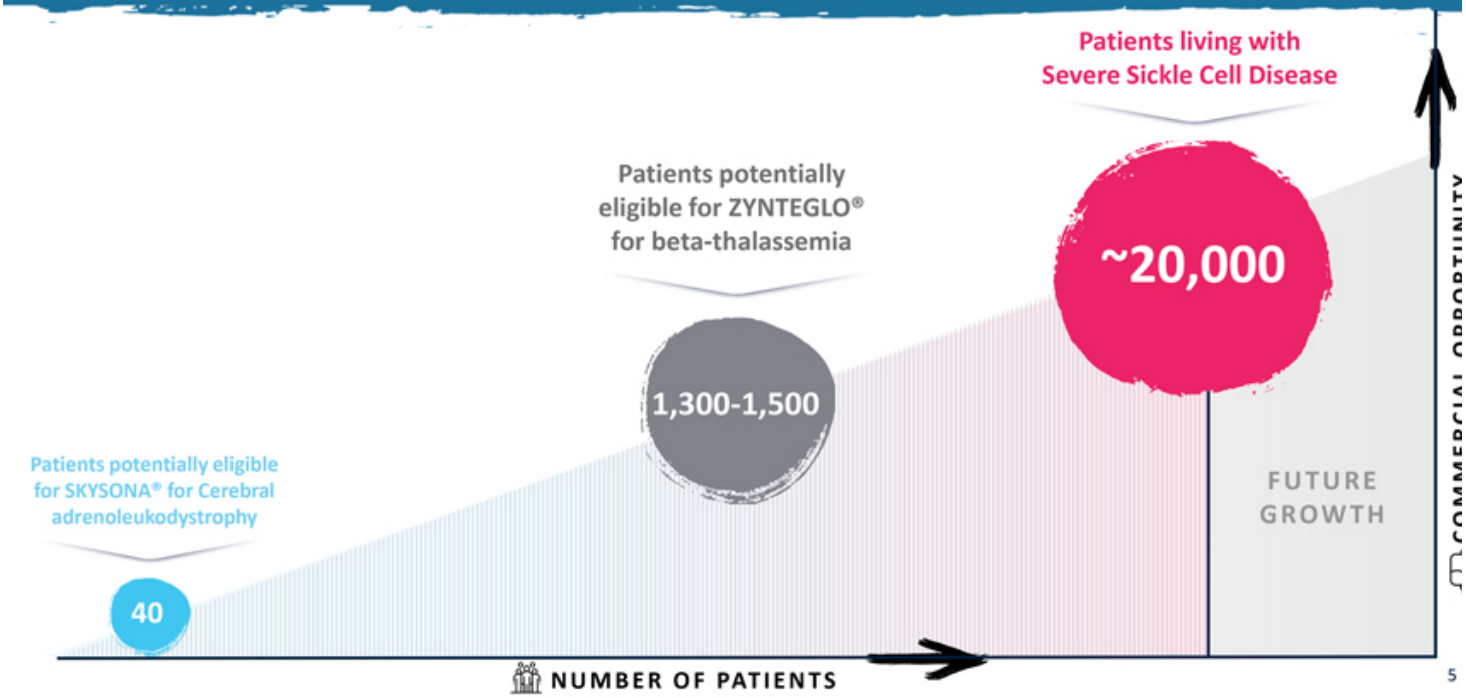
## Commercial Impact

**2 ongoing US launches,** revenue expected in Q1 2023, all with wholly-owned global rights

**~22,000 patients** potentially addressable with our 3 programs in the U.S.[1]

[1] Hassell KL. Population estimates of sickle cell disease in the U.S. Am J Prev Med. 2010;38(4 Suppl):S512 521; Jul '21 bbb analysis of Komodo patient-level claims data (Apr '20 – Mar '21), IQVIA patient-level claims data (Aug '18 – Jul '19); Hulihan, Mary M., et al. State-based surveillance for selected hemoglobinopathies. Genetics in Medicine 17.2 (2015): 125-130.; Bezman L, et al. Adrenoleukodystrophy: Incidence, new mutation rate, and results of extended family screening. Ann Neurol. 2001;49:512–517; Moser HW, Mahmood A, Raymond GV. X-linked adrenoleukodystrophy. Nature Clin Pract Neurol. 2007;3(3):140-51

# Momentum building with near-term commercial launches; opportunity to deliver significant value for patients and shareholders



# Inherited hemoglobin disorders

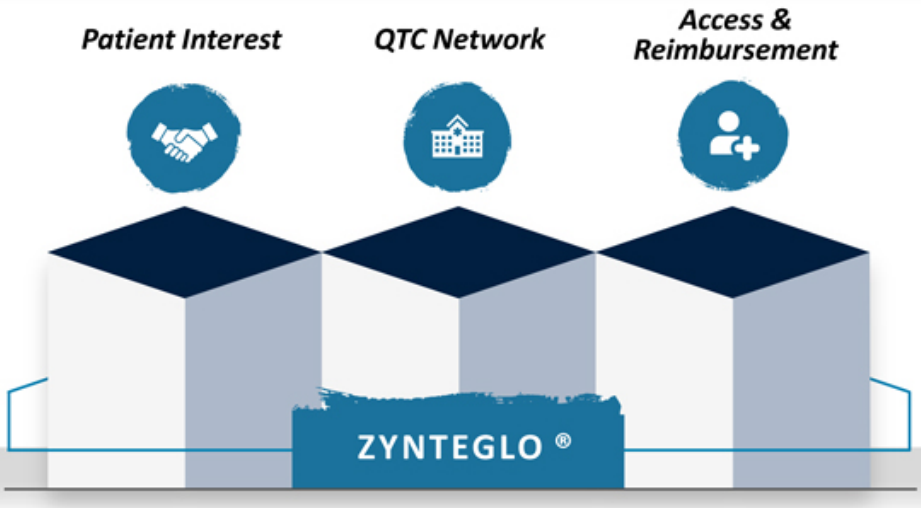
bluebirdbio

## Launching now

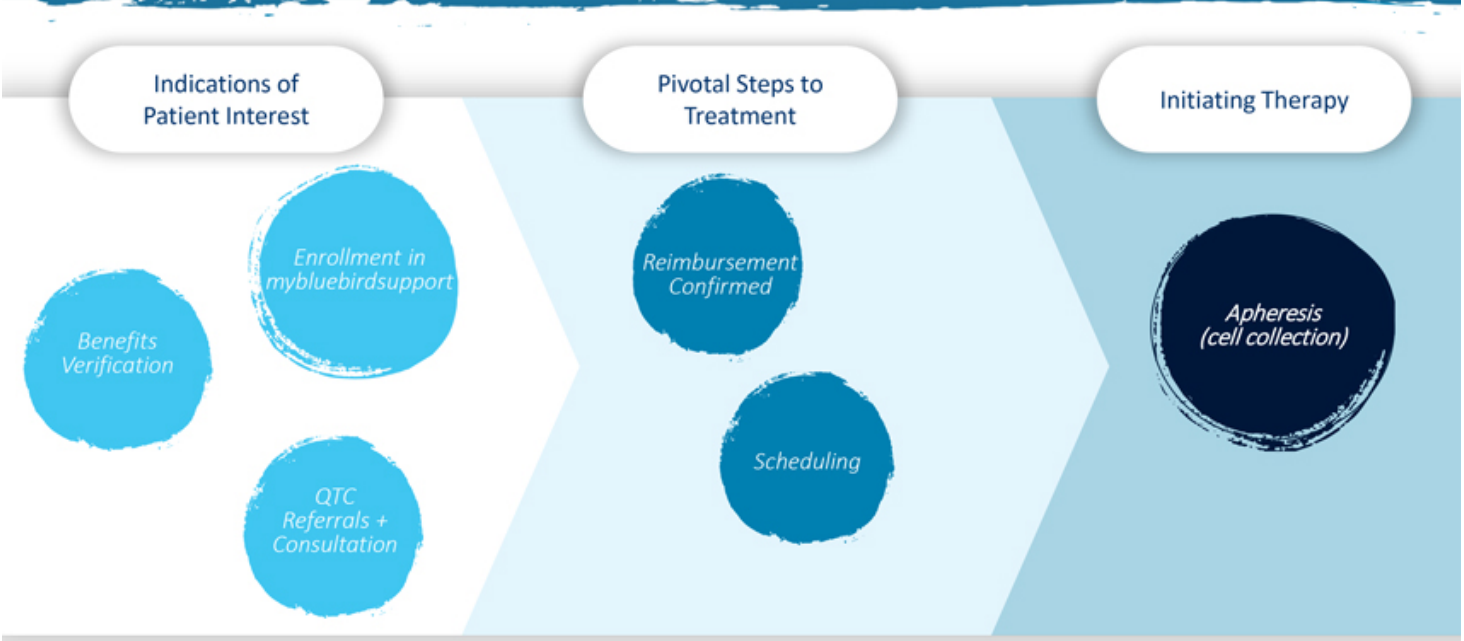
zynteglo®
(betibeglogene autotemcel)
suspension for IV infusion

# ZYNTEGLO commercial launch off to a strong start

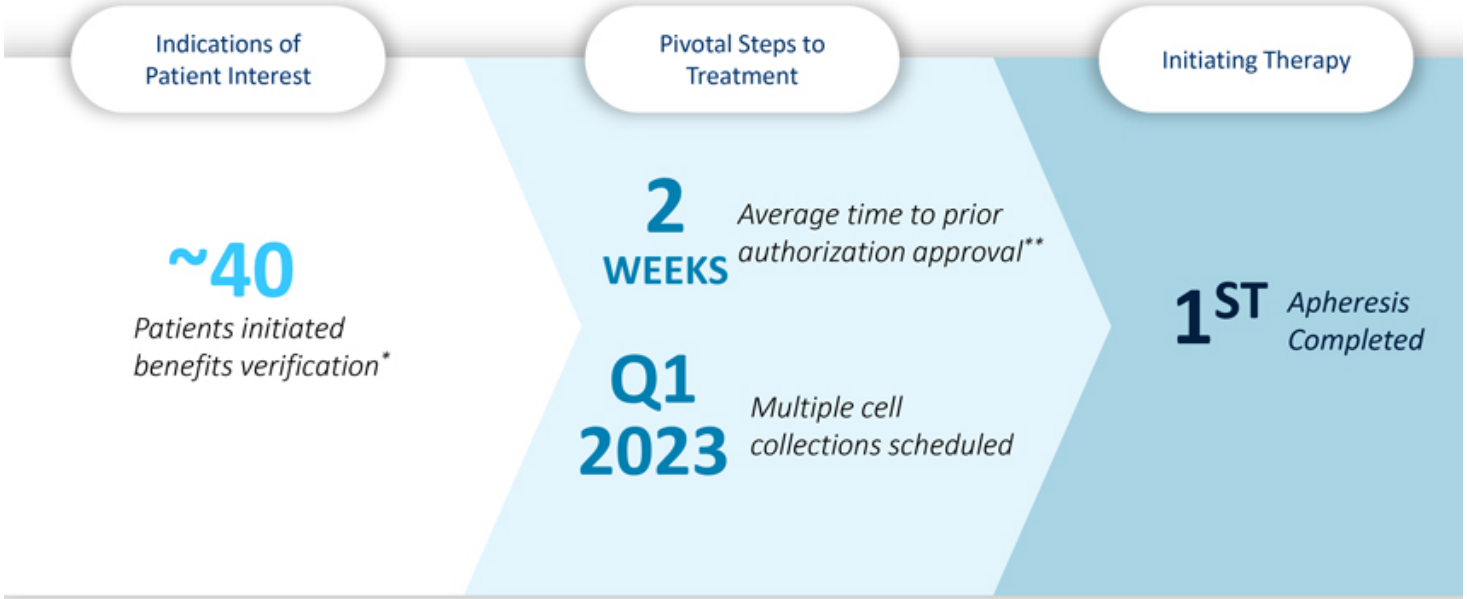
## Launch built on three key pillars



# Path to treatment is multi-faceted



# Clear signs of early patient uptake approximately four months into launch




*Estimate based on mybluebirdsupport data and metrics provided by QTCs
**Estimate based on QTC reports

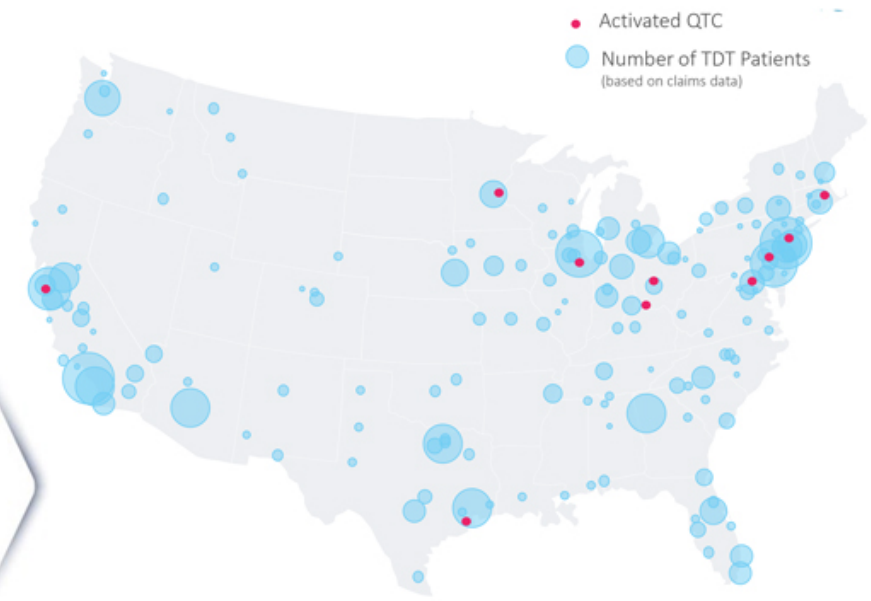
# Fit-for-purpose Qualified Treatment Center (QTC) network being activated in waves

## Targeted QTC selection

- Focused on high prevalence states
- Centers actively treating beta-thalassemia today
- Deep experience with commercial cell and gene therapies

## QTC growth aligned with demand

- 10 QTCs activated
- >15 QTCs in on-boarding or MSA negotiation stage
- Anticipated expansion to ~40-50 QTCs by YE 2023 to maximize opportunity for ZYNTEGLO and in anticipation of lovo-cel launch




*Graphic is illustrative and subject to change as final QTC network is determined; Activated QTC defined as Qualified Treatment Center with a signed MSA



QTC: Qualified Treatment Center

# Confident in timely, quality access and reimbursement with upfront payment at $2.8M price

## PRICE TIED TO RECOGNIZED VALUE

**Beta-thalassemia requiring regular RBC transfusions is associated with:**

- $6.4 million average lifetime medical care cost per patient[1]
- 23X higher average total health care cost per patient per year vs. general population[2]
- Blood transfusions every 2-5 weeks for life[3]

## SIMPLE AND INNOVATIVE PAYMENT STRATEGY

**bluebird is offering payers:**

- One-time upfront payment
- Outcomes-based agreement with up to 80% rebate if patient does not reach transfusion independence within 2 years
- Clinically-relevant outcome, easily tracked in claims data

## ENCOURAGING PAYER INTERACTIONS

**All target payers have responded favorably to approach:**

- Estimated 70-75% of patients with beta-thalassemia have commercial insurance
- Engaging with state Medicaid agencies representing ~80% of publicly-insured beta-thalassemia patients



[1] Date on file [2] Weiss et al. 2019 [3] TIF Guidelines

# Early indications show value of ZYNTEGLO is recognized; patients are achieving access

## ~4 months since FDA approval:

**~190M**

lives covered by a favorable coverage policy

**THREE**

of the largest PBMs have signed outcomes-based agreements

**ZERO**

ultimate denials

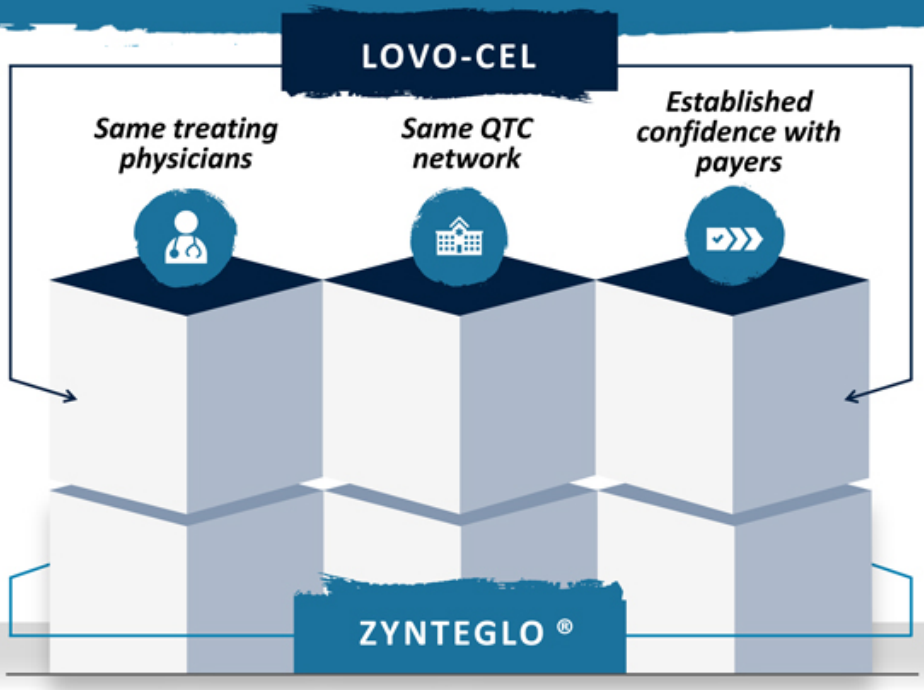
# ZYNTEGLO® manufacturing allows for flexible scheduling and is designed to deliver high quality drug product


*Bulk of time spent on release testing to deliver high quality drug product*

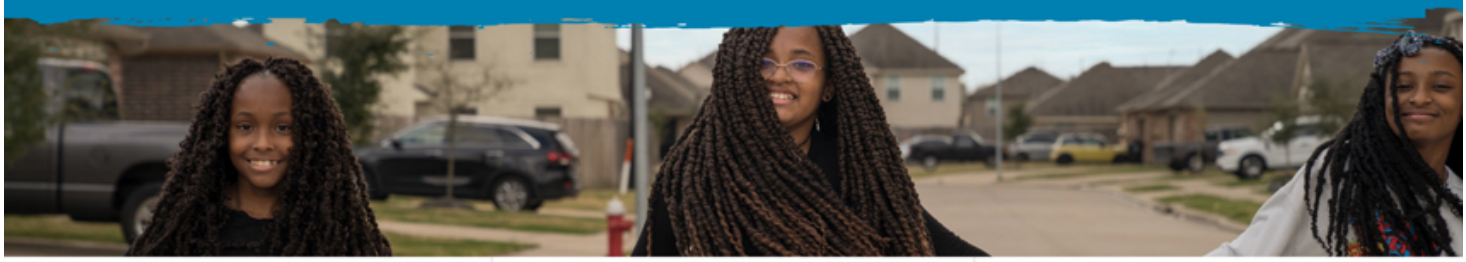


QTC: Qualified Treatment Center; CMO: Contract Manufacturing Organization

# ZYNTEGLO expected to enable seamless transition to commercializing lovo-cel for sickle cell disease



# Opportunity to address a critical unmet need for >20,000 individuals living with severe sickle cell disease in the US



## LARGE PATIENT POPULATION

- 1 in 365 Black or African American babies is born with sickle cell disease[1]
- **>20,000 SCD patients** in the US may be addressed by gene therapy[2]

## SIGNIFICANT UNMET NEED

- VOEs are the hallmark of SCD, but the disease is more than just pain
- 1 in 4 patients have a stroke by age 45[3]
- Widespread risk of organ damage or organ failure[3]
- 75% report difficulty completing daily tasks[4]

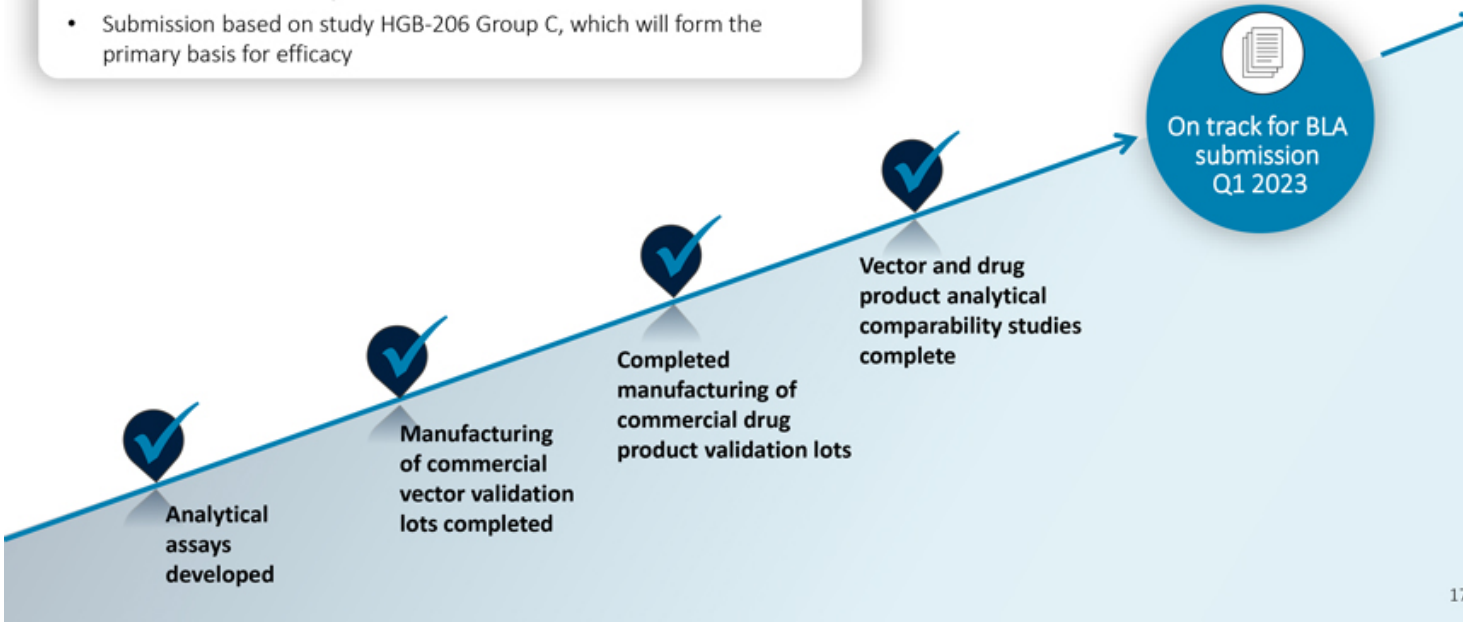
## MEANINGFUL OPPORTUNITY

- Patients average $4.0 million in direct medical costs, despite a median age of death of only 45[5]
- Approximately 65% report giving up a job due to SCD[4]
- Estimates of foregone income over a lifetime up to $1.3 million[6]
- Nearly 1/3 report experiencing discrimination in a healthcare setting[7]

[1] CDC [2] Data on file [3] Mortality Rates and Age at Death from Sickle Cell Disease: U.S., 1979–2005 [3] Kato GJ, Piel FB, Reid CD, et al. Sickle cell disease. Nat Rev Dis Primers. 2018;4:18010. [4] Holdford et al 2021 [5] Gallagher ME et al, J Med Econ. 2022 Jan-Dec [6] Graf 2022 [7] Harvard Chan, RWJF Poll 2017

# lovo-cel BLA submission on track for Q1 2023; comparability studies complete

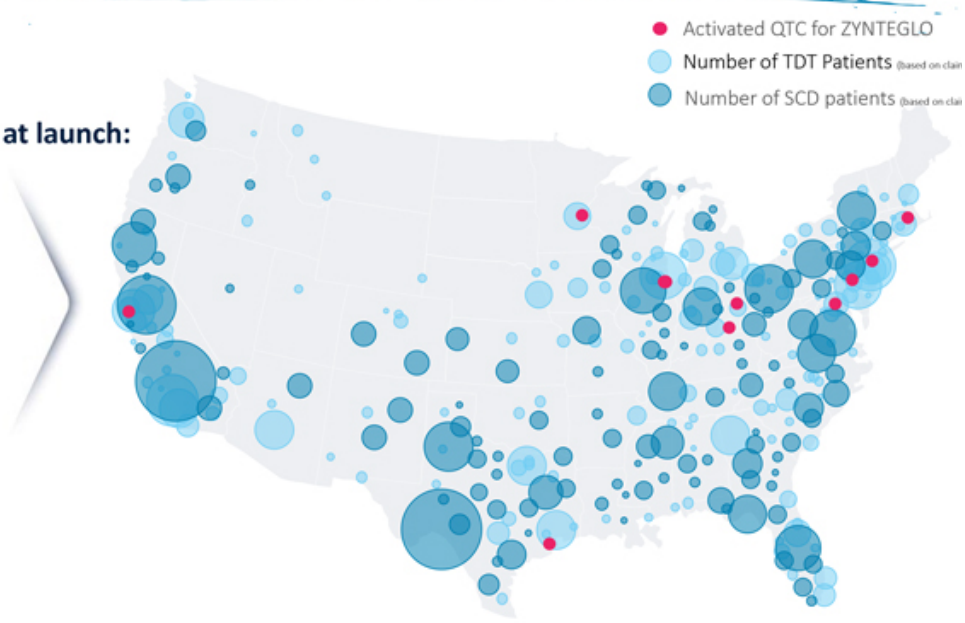
- Plan to submit BLA for patients 12 and older
- Submission based on study HGB-206 Group C, which will form the primary basis for efficacy



# Planned 2023 network expansion ensures QTCs are in place and ready to treat appropriate SCD patients upon FDA approval of lovo-cel

## Significant synergies in QTC network at launch:

- Expansion to ~40-50 QTCs by YE 2023 maximizes opportunity to rapidly reach patients
- Established contract allows for simplified activation process
- Estimated 65% of SCD patients within 50 miles of a planned QTC; (95% within 200 miles); anticipate continued expansion in 2024






*Graphic is illustrative and subject to change as final QTC network is determined; Activated QTC defined as Qualified Treatment Center with a signed MSA

# SKYSONA

bluebirdbio

# SKYSONA®: FDA Approved

skysona™
(elivaldogene autotemcel)

- First apheresis scheduled for January 2023
- Two activated QTCs; three additional planned
- Zero ultimate denials; payers recognize value and urgency to treat
- Anticipate 5-10 patient starts in 2023

Early, active cerebral adrenoleukodystrophy refers to asymptomatic or mildly symptomatic (neurologic function score, NFS ≤ 1) boys who have gadolinium enhancement on brain magnetic resonance imaging (MRI) and Loes scores of 0.5-9. SKYSONA was granted accelerated approval based on 24-month Major Functional Disability (MFD)-free survival observed in clinical studies. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s). *Real patients pictured, but they have not used our therapies. QTC: Qualified Treatment Center

# Closing

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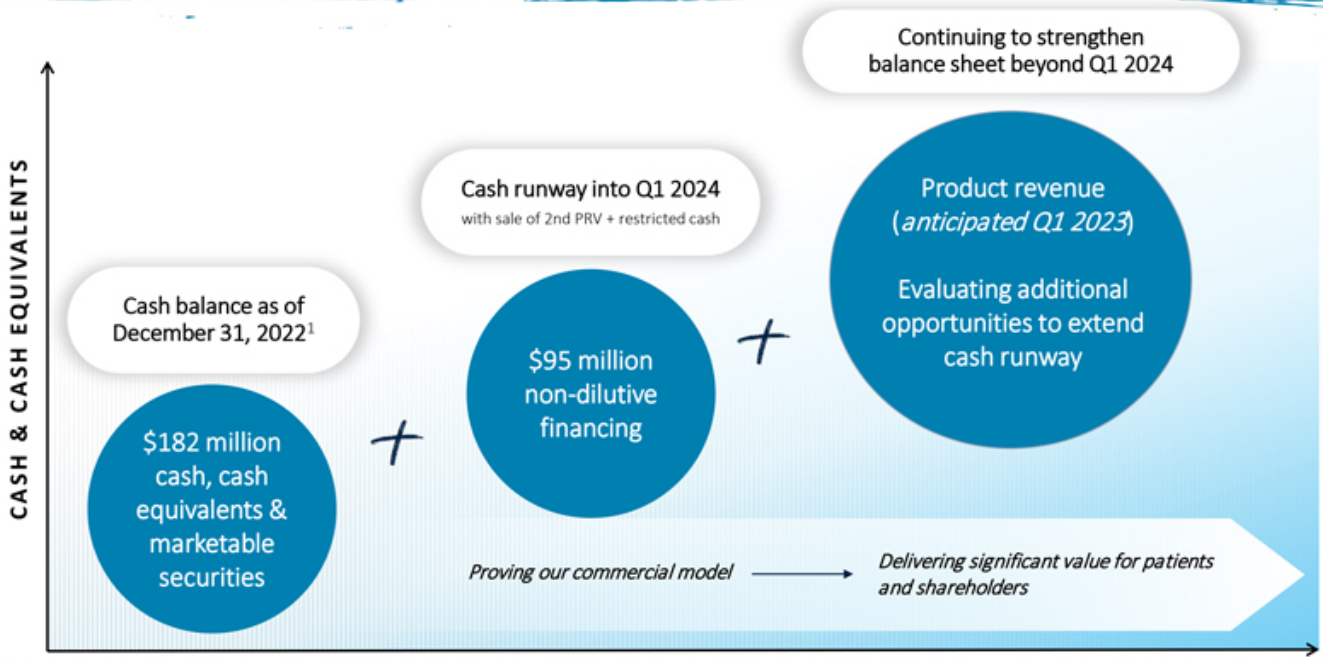
# Strong financial position – cash burn and runway horizon




1. Excludes $45m in restricted cash. The cash, cash equivalents and marketable securities information above is based on preliminary unaudited information and management estimates for the year ended December 31, 2022, is not a comprehensive statement of the Company's financial results as of and for the fiscal year ended December 31, 2022 and is subject to completion of the Company's financial closing procedures. The Company's independent registered public accounting firm has not conducted an audit or review of and does not express an opinion or any other form of assurance with respect to, this preliminary estimate.; 2. Cash Runway is calculated using the current cash balance / net burn rate (cash from revenue less cash paid for expenses)

# Upcoming milestones

*First to market gene therapy for inherited hemoglobin disorders in the U.S.*

**SKYSONA® for cerebral adrenoleukodystrophy**

- First cell collection scheduled for January 2023
- Continued launch expansion throughout 2023

**ZYNTEGLO® for beta-thalassemia**

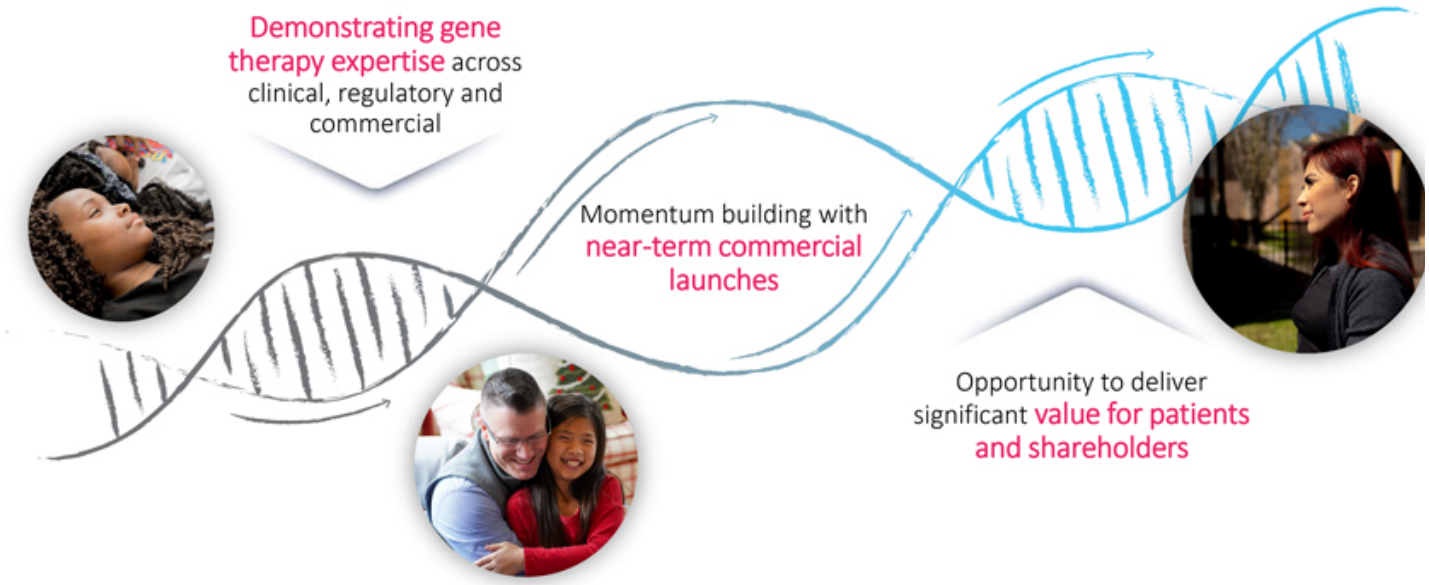
- First commercial revenue expected in Q1 2023
- Continued launch expansion throughout 2023
- 40-50 QTCs by end of 2023

**lovo-cel for sickle cell disease**

- BLA submission planned for Q1 2023
- Potential FDA approval expected by end of 2023
- Commercial launch expected early 2024

*Proving our commercial model* → *Significant value driver* →

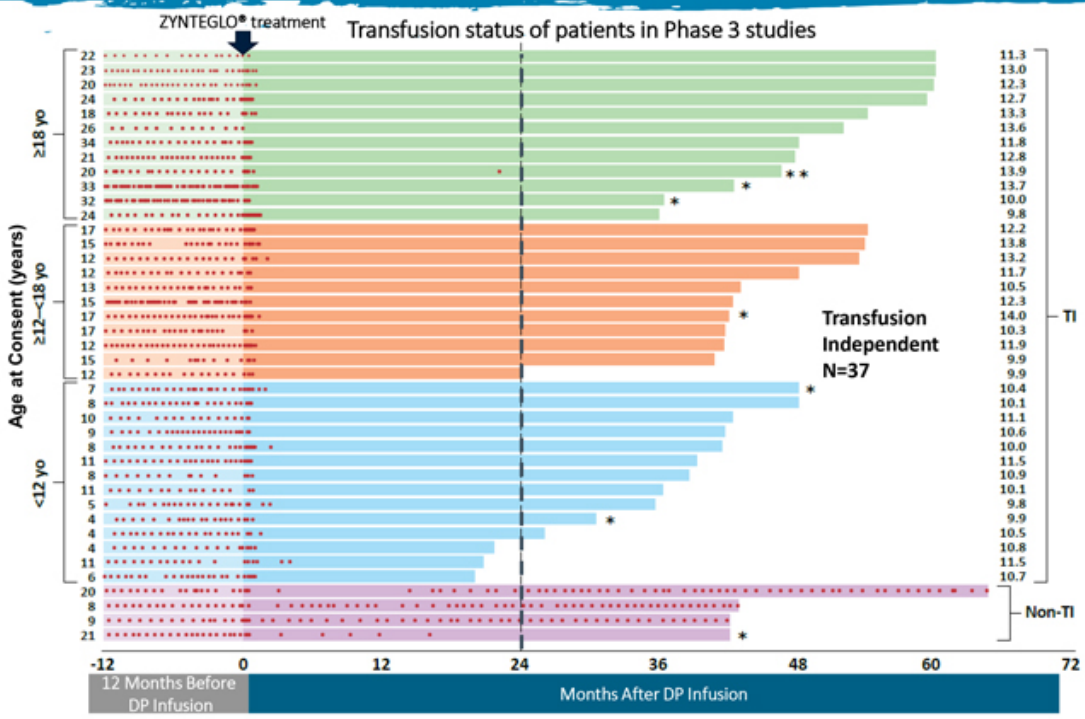
# bluebird bio: Setting the standard and proving the gene therapy commercial model




thank you


Q + A

# ZYNTEGLO® approval is underscored by impressive clinical study data




**In Phase 3 studies presented at ASH 2022:**

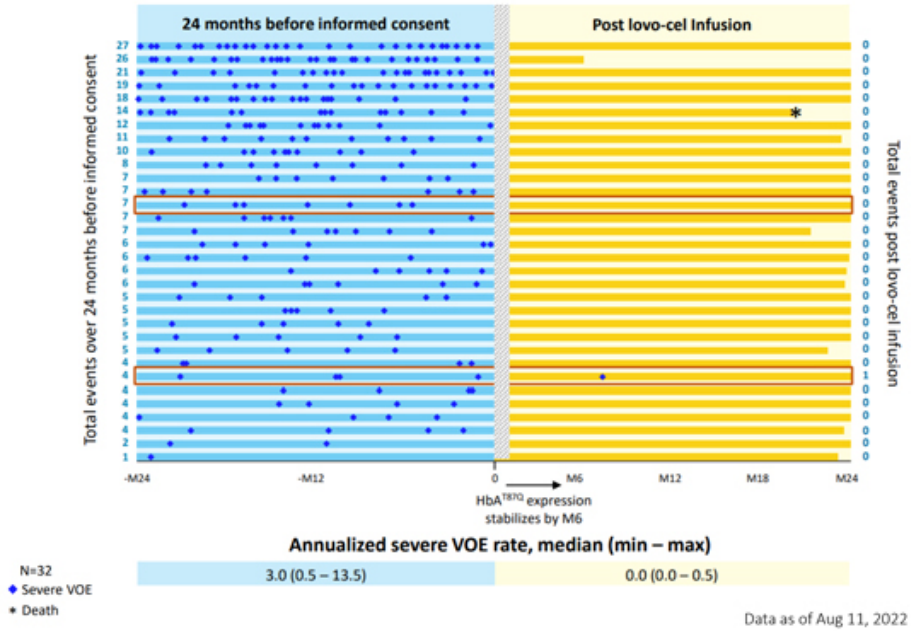
- 90% of patients achieved transfusion independence (TI) and normal or near-normal hemoglobin levels
- All patients who achieved TI remained transfusion free as of last follow-up
- Durable results with longest follow-up out to 5 years
- Results were consistent across ages and genotypes
- Majority of AEs and SAEs were consistent with myeloablative conditioning

**After a planned orthopedic surgery, the patient ha blood loss, which required 1 packed red blood cell transfusion



Data as of July 202

# lovo-cel: most advanced sickle cell disease gene therapy development program in the industry

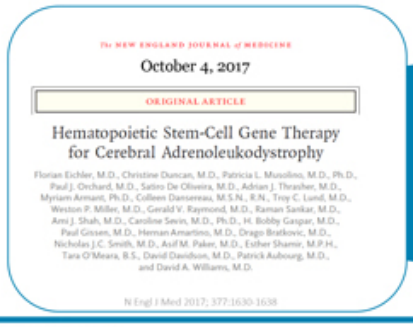


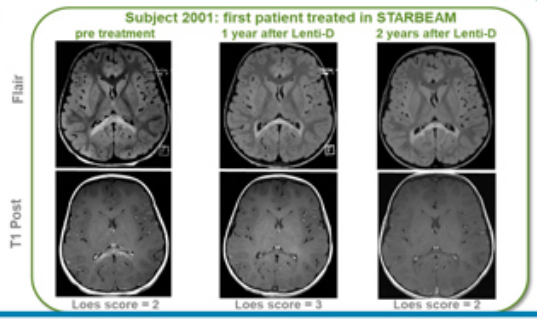

Data as of Aug 11, 2022

## Update on Pivotal Cohort (HGB 206 Group C) Presented at ASH 2022

- 96% experienced complete resolution of severe VOEs through 24 months of follow-up (ASH 2022)
- As of August 2022, 50 patients had been treated with lovo-cel, with up to 7 years of follow-up (median: 37.7 months)
- Safety data remained consistent with the known side effects of autologous hematopoietic stem cell collection, myeloablative single-agent busulfan conditioning and underlying SCD
- As previously reported, patient with significant baseline SCD-related cardiopulmonary disease died >18 months post-infusion (considered unlikely to be related to lovo-cel).
- Updated data cut, including long-term follow-up, being prepared for BLA submission anticipated in Q1 2023

# The approval of SKYSONA® was based on data from bluebird bio's Phase 2/3 study ALD-102 and Phase 3 ALD-104 study




## EFFICACY

Accelerated approval was based on a post hoc analysis of 24-month improvement in major functional disability (MFD) free survival

SKYSONA treated patients (n = 11) had an estimated 72% likelihood of MFD-free survival at 24 months compared to untreated patients in a natural history study (n = 7) who had only an estimated 43% likelihood of MFD-free survival

A total of 67 patients were treated in clinical trials

## SAFETY

The label includes a Boxed Warning on SKYSONA for hematologic malignancy; as previously reported, 3 boys treated in our clinical trials developed MDS which is believed to be caused by insertion of the Lenti-D vector

Other risks include serious infections, prolonged cytopenias, delayed platelet engraftment, risk of neutrophil engraftment failure, and hypersensitivity reactions.

Under accelerated approval, bluebird has agreed to provide confirmatory data to the FDA

Early, active cerebral adrenoleukodystrophy refers to asymptomatic or mildly symptomatic (neurologic function score, NFS ≤ 1) boys who have gadolinium enhancement on brain magnetic resonance imaging (MRI) and Loes scores of 0.5-9. SKYSONA was granted accelerated approval based on 24-month Major Functional Disability (MFD)- free survival observed in clinical studies. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s). MDS: myelodysplastic syndrome