UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, DC 20549

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended June 30, 2024

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from_____ to_____

Commission File Number: 001-35966

bluebird bio, Inc.

(Exact Name of Registrant as Specified in Its Charter)

Delaware	**13-3680878**
(State or Other Jurisdiction of Incorporation or Organization)	**(IRS Employer Identification No.)**
455 Grand Union Boulevard Somerville , Massachusetts	**02145**
(Address of Principal Executive Offices)	**(Zip Code)**

(339) 499-9300

(Registrant's Telephone Number, Including Area Code)

N/A

(Former Name, Former Address and Former Fiscal Year, if Changed Since Last Report)

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: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☐ No ☒

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☐ No ☒

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

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
		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. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of September 25, 2024, there were 193,913,585 shares of the registrant's Common Stock, par value $0.01 per share, outstanding.

FORWARD LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve risks and uncertainties, as well as assumptions that, if they never materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q 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," "would," or the negative of these words or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- our plans and expectations regarding our commercialization activities for SKYSONA, ZYNTEGLO, and LYFGENIA, as well as any future approved products and the timing or success thereof, including expectations regarding our network of qualified treatment centers;
- 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 obtain adequate financing to fund our operations and to execute on our strategy;
- our expectations and projections regarding the sufficiency of our cash and cash equivalents to fund our operations;
- our ability to establish and scale commercial viral vector and drug product manufacturing capabilities, and to ensure adequate supply of our viral vectors and drug products, and our plans and expectations regarding our manufacturing activities;
- the timing or likelihood of regulatory filings and marketing approvals for our product candidates and our plans and expectations relating thereto;
- our ability to obtain adequate pricing and reimbursement of any approved products;
- 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 and licenses;
- developments relating to our competitors and our industry;
- the impact of the general economic conditions and uncertainties;
- our ability to mitigate the commercial, reputational and regulatory risks to our business that may arise as a consequence of the restatement of our financial statements;
- the estimated charges and expenses related to, and anticipated benefits from, our restructuring action;
- our ability to comply with Nasdaq continued listing rules;
- our ability to comply with covenants in our loan agreement;
- our ability to remediate the material weakness in our internal control over financial reporting; and
- other risks and uncertainties, including those listed under Part II, Item 1A. Risk Factors.

Any forward-looking statements in this Quarterly Report on Form 10-Q 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 Part II, Item 1A. Risk Factors and elsewhere in this Quarterly Report on Form 10-Q. 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.

This Quarterly Report on Form 10-Q 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.

Summary of the Material and Other Risks Associated with Our Business

Below is a summary of the material risks to our business, operations and the investment in our common stock. This summary does not address all of the risks that we face. Risks and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations. Additional discussion of the risks summarized in this risk factor summary, and other risks that we face, can be found below under the heading "Risk Factors" and should be carefully considered, together with other information in this Quarterly Report on Form 10-Q in its entirety before making investment decisions regarding our common stock.

- We have incurred significant losses since our inception and we may not achieve our goal of becoming profitable in the timeframe we expect, or at all.
- There is substantial doubt regarding our ability to continue as a going concern. 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 commercial programs, product development efforts or other operations.
- Among other potential adverse events, insertional oncogenesis is a significant risk of gene therapies using viral vectors that can integrate into the genome. Any such adverse events may require us to halt or delay further clinical development of our products or any future product candidates or to suspend or cease commercialization, and the commercial potential of our products and any such future product candidates may be materially and negatively impacted.
- We rely on complex, single-source supply chains for SKYSONA, ZYNTEGLO, and LYFGENIA, respectively. The manufacture, testing and delivery of LVV and drug products present significant challenges for us, and we may not be able to produce our vector and drug products at the quality, quantities, or timing needed to support our clinical programs and commercialization.
- We have limited experience as a commercial company and the marketing and sale of ZYNTEGLO, SKYSONA and LYFGENIA may be unsuccessful or less successful than anticipated.
- The commercial success of ZYNTEGLO, SKYSONA and LYFGENIA will depend upon the degree of market acceptance by physicians, patients, payers and other stakeholders.
- If the market opportunities for our commercial products or any future product candidates are smaller than we believe they are, and if we are not able to successfully identify patients and achieve significant market share, our revenues may be adversely affected and our business may suffer.
- The insurance coverage and reimbursement status of newly-approved products in the United States is uncertain. Due to the novel nature of our technology and the potential for our products to offer lifetime therapeutic benefit in a single administration, we face unique and additional challenges in obtaining adequate coverage and reimbursement for our products. Failure to obtain or maintain adequate coverage and reimbursement for any new or current product, including to the extent that payers 'non-prefer' any or all of our therapies to our competitors, could limit our ability to market those products and decrease our ability to generate revenue.
- We face intense competition and rapid technological change and the possibility that our competitors may develop therapies that are more advanced, safer or more effective than ours, which may adversely affect our financial condition and our ability to successfully develop and commercialize ZYNTEGLO, SKYSONA and LYFGENIA.
- The restatement of our consolidated financial statements for the year ended December 31, 2022 and the quarterly periods in the years ended December 31, 2022 and 2023 has subjected us to a number of additional risks and uncertainties, including increased possibility of legal proceedings.
- Our existing and any future indebtedness could adversely affect our ability to operate our business.
- Our restructuring and reduction in force undertaken to optimize our cost structure may not achieve our intended outcome.

- We have identified a material weakness in our internal control over financial reporting and may identify additional material weaknesses in the future or otherwise fail to maintain an effective system of internal controls.

bluebird bio, Inc.

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PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

bluebird bio, Inc.

Condensed Consolidated Balance Sheets
(unaudited)
(in thousands, except par value amounts)

	As of June 30, 2024	As of December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 144,067	$ 221,755
Prepaid expenses	10,539	14,800
Inventory	33,330	22,919
Due from factor	1,302	560
Receivables and other current assets	14,278	21,651
Total current assets	203,516	281,685
Property, plant and equipment, net	78,756	65,936
Goodwill	5,646	5,646
Intangible assets, net	10,012	10,438
Operating lease right-of-use assets	189,206	201,113
Restricted cash and other non-current assets	58,057	54,343
Total assets	$ 545,193	$ 619,161
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 30,591	$ 18,498
Due to factor	5,220	2,520
Accrued expenses and other current liabilities	65,889	73,188
Operating lease liability, current portion	25,052	21,202
Financing lease liability, current portion	101,335	84,705
Term loan debt	69,843	—
Total current liabilities	297,930	200,113
Operating lease liability, net of current portion	174,535	186,687
Financing lease liability, net of current portion	19,655	37,732
Other non-current liabilities	92	92
Total liabilities	492,212	424,624
Commitments and contingencies *(Note 10)*		
Stockholders' equity:		
Preferred stock, $0.01 par value, 5,000 shares authorized; 0 shares issued and outstanding at June 30, 2024 and December 31, 2023	—	—
Common stock, $0.01 par value, 250,000 shares authorized; 193,856 and 192,772 shares issued and outstanding at June 30, 2024 and December 31, 2023, respectively	1,916	1,905
Additional paid-in capital	4,464,712	4,454,756
Accumulated other comprehensive loss	(2,122)	(1,796)
Accumulated deficit	(4,411,525)	(4,260,328)
Total stockholders' equity	52,981	194,537
Total liabilities and stockholders' equity	$ 545,193	$ 619,161

See accompanying notes to unaudited condensed consolidated financial statements.

bluebird bio, Inc.

Condensed Consolidated Statements of Operations and Comprehensive Loss
(unaudited)
(in thousands, except per share data)

	For the three months ended June 30,		For the six months ended June 30,	
	2024	2023	2024	2023
		(As Restated)		(As Restated)
Revenue:				
Product revenue, net	$ 16,101	$ 6,837	$ 34,662	$ 9,133
Other revenue	—	53	12	138
Total revenues	16,101	6,890	34,674	9,271
Cost of product revenue	28,946	6,697	54,810	12,209
Gross margin	(12,845)	193	(20,136)	(2,938)
Operating expenses:				
Selling, general and administrative	50,385	40,462	96,714	77,929
Research and development	25,162	31,448	50,236	73,035
Total operating expenses	75,547	71,910	146,950	150,964
Gain from sale of priority review voucher, net	—	—	—	92,930
Loss from operations	(88,392)	(71,717)	(167,086)	(60,972)
Interest income	2,837	2,679	5,416	5,507
Interest expense	(5,453)	(3,750)	(10,308)	(8,020)
Other income, net	9,636	9,919	20,802	19,546
Loss before income taxes	(81,372)	(62,869)	(151,176)	(43,939)
Income tax (expense) benefit	(21)	80	(21)	80
Net loss	$ (81,393)	$ (62,789)	$ (151,197)	$ (43,859)
Net loss per share - basic	$ (0.42)	$ (0.58)	$ (0.78)	$ (0.41)
Net loss per share - diluted	$ (0.42)	$ (0.58)	$ (0.78)	$ (0.41)
Weighted-average number of common shares used in computing net loss per share - basic:	193,716	108,685	193,433	105,819
Weighted-average number of common shares used in computing net loss per share - diluted:	193,716	108,685	193,433	105,819
Other comprehensive income (loss):				
Other comprehensive income (loss), net of tax benefit (expense) of $0.0 million for the three and six months ended June 30, 2024 and 2023	(14)	722	(327)	1,706
Total other comprehensive income (loss)	(14)	722	(327)	1,706
Comprehensive loss	$ (81,407)	$ (62,067)	$ (151,524)	$ (42,153)

See accompanying notes to unaudited condensed consolidated financial statements.

bluebird bio, Inc.

Condensed Consolidated Statements of Stockholders' Equity
(unaudited)
(in thousands)

	Common stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balances at December 31, 2023	192,772	$ 1,905	$ 4,454,756	$ (1,796)	$ (4,260,328)	$ 194,537
Vesting of restricted stock units	811	8	(8)	—	—	—
Issuance of warrants	—	—	2,571	—	—	2,571
Stock-based compensation expense	—	—	4,054	—	—	4,054
Other comprehensive loss	—	—	—	(312)	—	(312)
Net loss	—	—	—	—	(69,804)	(69,804)
Balances at March 31, 2024	193,583	$ 1,913	$ 4,461,373	$ (2,108)	$ (4,330,132)	$ 131,046
Vesting of restricted stock units	185	$ 2	$ (2)	$ —	$ —	$ —
Purchase of common stock under employee stock purchase plan (ESPP)	88	1	80	—	—	81
Stock-based compensation expense	—	—	3,261	—	—	3,261
Other comprehensive loss	—	—	—	(14)	—	(14)
Net loss	—	—	—	—	(81,393)	(81,393)
Balances at June 30, 2024	193,856	$ 1,916	$ 4,464,712	$ (2,122)	$ (4,411,525)	$ 52,981

	Common stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balances at December 31, 2022 (As Restated)	$ 82,923	$ 830	$ 4,185,988	$ (4,070)	$ (4,048,415)	$ 134,333
Vesting of restricted stock units	382	3	(198)	—	—	(195)
Exercise of stock options	3	—	7	—	—	7
Purchase of common stock under ESPP	62	1	226	—	—	227
Issuance of common stock	23,000	230	130,061	—	—	130,291
Stock-based compensation expense	—	—	5,843	—	—	5,843
Other comprehensive income	—	—	—	984	—	984
Net income	—	—	—	—	18,930	18,930
Balances at March 31, 2023 (As Restated)	106,370	$ 1,064	$ 4,321,927	$ (3,086)	$ (4,029,485)	$ 290,420
Vesting of restricted stock units	65	1	(1)	—	—	—
Exercise of stock options	19	—	77	—	—	77
Stock-based compensation expense	—	—	6,388	—	—	6,388
Other comprehensive income	—	—	—	722	—	722
Net loss	—	—	—	—	(62,789)	(62,789)
Balances at June 30, 2023 (As Restated)	106,454	$ 1,065	$ 4,328,391	$ (2,364)	$ (4,092,274)	$ 234,818

See accompanying notes to unaudited condensed consolidated financial statements.

bluebird bio, Inc.

Condensed Consolidated Statements of Cash Flows
(unaudited)
(in thousands)

	For the six months ended June 30,	
	2024	**2023**
		(As Restated)
Cash flows from operating activities:		
Net loss	$ (151,197)	$ (43,859)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	31,995	12,250
Stock-based compensation expense	6,968	11,145
Noncash research and development expense (finance lease)	—	434
Noncash operating lease expense	11,908	14,966
Gain from sale of priority review voucher	—	(92,930)
Excess inventory reserve	5,666	6,278
Noncash interest related to debt	1,004	—
Other non-cash items	(21)	343
Gain on foreign currency exchange rates	(581)	(281)
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(35,131)	(6,440)
Inventory	(15,729)	(17,599)
Accounts payable	12,517	(5,623)
Accrued expenses and other liabilities	(5,670)	3,052
Accrued interest payable under finance lease	5,646	819
Operating lease liabilities	(8,303)	(13,276)
Deferred revenue	—	(138)
Net cash used in operating activities	(140,928)	(130,859)
Cash flows from investing activities:		
Purchase of property, plant and equipment	(1,834)	(937)
Purchases of marketable securities	—	(34,418)
Proceeds from previously transferred invoices	2,800	—
Proceeds from maturities of marketable securities	—	26,521
Proceeds from sales of marketable securities	—	5,853
Purchase of intangible assets	—	(868)
Proceeds from sale of priority review voucher	—	92,930
Net cash provided by investing activities	966	89,081
Cash flows from financing activities:		
Proceeds from the issuance of debt, net of fees paid to lender	71,316	—
Proceeds allocated to detachable warrants issued in conjunction with debt	2,669	—
Payments of debt issuance costs	(2,576)	—
Proceeds from exercise of stock options and ESPP contributions	—	85
Proceeds from the transfer of invoices	35,820	—
Proceeds from vesting of restricted stock	—	(196)
Principal payments on finance leases	(48,645)	(28,504)
Proceeds from the secondary public offering, net of issuance costs	—	130,122
Net cash provided by financing activities	58,584	101,507
(Decrease) increase in cash, cash equivalents and restricted cash	(81,378)	59,729
Cash, cash equivalents and restricted cash at beginning of period	274,597	158,445
Cash, cash equivalents and restricted cash at end of period	$ 193,219	$ 218,174
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 144,067	$ 172,872
Restricted cash included in other current assets	1,154	1,364

Restricted cash included in restricted cash and other non-current assets	47,998	43,938
Total cash, cash equivalents and restricted cash	$ 193,219	$ 218,174
Supplemental cash flow disclosures from investing and financing activities:		
Purchases of property, plant and equipment included in accounts payable and accrued expenses	284	2,290
Right-of-use assets obtained in exchange for financial lease liabilities	43,055	3,436
Cash paid (refund received) during the period for income taxes	(10)	7
Beneficiary interest obtained in transferred invoices	3,680	—
Derecognition of due to factor liabilities	33,120	—

See accompanying notes to unaudited condensed consolidated financial statements.

bluebird bio, Inc.

Notes to Condensed Consolidated Financial Statements
(unaudited)

1. Description of the business

bluebird bio, Inc. (the “Company” or “bluebird”) was incorporated in Delaware on April 16, 1992, and is headquartered in Somerville, Massachusetts. The Company is a biotechnology company committed to researching, developing and commercializing potentially curative gene therapies for severe genetic diseases based on its proprietary lentiviral vector ("LVV") gene addition platform. Since its inception, the Company has devoted substantially all of its resources to its research and development efforts relating to its product candidates, and commercialization of its approved products, including activities to manufacture product candidates, conduct clinical studies of its product candidates, perform preclinical research to identify new product candidates, provide selling, general and administrative support for these operations and market and commercially manufacture and distribute its approved products.

The Company’s programs in severe genetic diseases include ZYNTEGLO (betibeglogene autotemcel, also known as beti-cel) as a treatment for β-thalassemia; LYFGENIA (lovotibeglogene autotemcel, also known as lovo-cel) as a treatment for sickle cell disease ("SCD"); and SKYSONA (elivaldogene autotemcel, also known as eli-cel) as a treatment for cerebral adrenoleukodystrophy ("CALD"). On August 17, 2022, ZYNTEGLO was approved by the U.S. Food and Drug Administration ("FDA") for the treatment of adult and pediatric patients with ß-thalassemia who require regular red blood cell transfusions. On September 16, 2022, the FDA granted Accelerated Approval of SKYSONA to slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active CALD. On December 8, 2023, LYFGENIA was approved by the FDA for the treatment of patients 12 years of age or older with sickle cell disease and with a history of vaso-occlusive-events.

In August 2023, the Company entered into an Open Market Sales Agreement (the "Sales Agreement") with Jefferies LLC ("Jefferies") to sell shares of the Company’s common stock up to $125.0 million, from time to time, through an “at the market” equity offering program under which Jefferies will act as sales agent. As of June 30, 2024, the Company has made no sales pursuant to the Sales Agreement.

In December 2023, the Company entered into an underwriting agreement (the “Underwriting Agreement”) with Goldman Sachs & Co. LLC ("Goldman") and J.P. Morgan Securities LLC, to sell 83.3 million shares of the Company's common stock. The Company received net proceeds of approximately $118.1 million.

In March 2024, the Company entered into a five-year term loan facility agreement with Hercules Capital, Inc. to secure debt financing for up to $175.0 million, available in four tranches. This is described in Note 8, *Term Loan Debt*, in the Notes to Condensed Consolidated Financial Statements appearing elsewhere in this Quarterly Report on Form 10-Q.

Since its inception, the Company has incurred significant operating losses and negative operating cash flows. As of June 30, 2024, the Company had an accumulated deficit of $4.4 billion. During the six months ended June 30, 2024, the Company incurred a net loss of $151.2 million and used $140.9 million of cash in operations. As of June 30, 2024, the Company had cash and cash equivalents of $144.1 million.

In accordance with Accounting Standards Codification (“ASC”) 205-40, Going Concern, the Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company’s ability to continue as a going concern within one year after the date the financial statements are issued.

This evaluation initially does not take into consideration the potential mitigating effect of management’s plans that have not been fully implemented as of the date the financial statements are issued. When substantial doubt exists under this methodology, management evaluates whether the mitigating effect of its plans sufficiently alleviates substantial doubt about the Company’s ability to continue as a going concern. The mitigating effect of management’s plans, however, is only considered if both (1) it is probable that the plans will be effectively implemented within one year after the date that the financial statements are issued, and (2) it is probable that the plans, when implemented, will mitigate the relevant conditions or events that raise substantial doubt about the entity’s ability to continue as a going concern within one year after the date that these consolidated financial statements are issued.

The Company's history of recurring operating losses and negative operating cash flows, its expectation to generate operating losses and negative operating cash flows, and the need for additional funding to support its planned operations raise substantial doubt regarding the Company’s ability to continue as a going concern for a period of one year after the date that these consolidated financial statements are issued. Management's plans to alleviate the conditions that raise substantial doubt include controlling spending, executing on commercial launch plans, and exploring additional financing options. Management has concluded the likelihood that its plan to successfully obtain sufficient funding from one or more of these sources, or adequately reduce expenditures, while reasonably possible, is less than probable. Accordingly, the Company has concluded that

substantial doubt exists about the Company's ability to continue as a going concern for a period of at least 12 months from the date of issuance of these consolidated financial statements.

The Company has based the estimated cash needs on assumptions that may prove to be wrong, and its operating plan may change as a result of many factors currently unknown to it. As a result, the Company could deplete its capital resources sooner than it currently expects. Along with the Company's revenue from product sales, the Company expects to finance its future cash needs through the issuance of equity, or debt, or other alternative means. If the Company is unable to obtain funding on a timely basis, or if revenues from product sales are less than it has projected, the Company may be required to further revise its business plan and strategy, which may result in the Company significantly curtailing, delaying or discontinuing one or more of its research or development programs or the commercialization of any products or may result in the Company being unable to expand its operations or otherwise capitalize on its business opportunities. As a result, the Company's business, financial condition and results of operations could be materially affected.

The accompanying financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the ordinary course of business. The financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of the uncertainties described above.

2. Basis of presentation, principles of consolidation and significant accounting policies

Basis of presentation

The accompanying condensed consolidated financial statements are unaudited and have been prepared by the Company in accordance with accounting principles generally accepted in the United States ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative United States GAAP as included in the Accounting Standard Codification ("ASC") and Accounting Standards Updates ("ASUs") of the Financial Accounting Standards Board ("FASB"). Certain information and footnote disclosures normally included in the Company's annual financial statements have been condensed or omitted. These condensed consolidated financial statements, in the opinion of management, reflect all normal recurring adjustments necessary for a fair presentation of the Company's financial position and results of operations for the interim periods ended June 30, 2024 and 2023.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year. These condensed consolidated financial statements should be read in conjunction with the audited consolidated financial statements as of and for the year ended December 31, 2023, and the notes thereto, which are included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2023, filed with the Securities and Exchange Commission (the "SEC") on September 13, 2024 (the "2023 Annual Report on Form 10-K").

Amounts reported are computed based on thousands, except percentages, per share amounts, or as otherwise noted. As a result, certain totals may not sum due to rounding.

The accompanying condensed consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation. The Company views its operations and manages its business in one operating segment.

Restatement of Previously Issued Financial Statements

As previously disclosed in the Company's 2023 Annual Report Form 10-K filed with the SEC on September 13, 2024, the Company is restating its previously issued unaudited condensed consolidated financial information for the three and six month periods ending June 30, 2023 due to multiple prior period misstatements. The accompanying condensed consolidated financial information as of and for the quarter ended June 30, 2023 have been restated in this Quarterly Report on Form 10-Q (see Note 15: Restatement of Previously Issued Financial Statements). In addition, the Company has corrected the accompanying footnotes in connection with the restatement.

Significant accounting policies

The significant accounting policies used in preparation of these condensed consolidated financial statements for the three and six months ended June 30, 2024 are consistent with those discussed in Note 3 to the consolidated financial statements included in the Company's 2023 Annual Report on Form 10-K.

Reclassification of Prior Year Presentation

Certain prior year amounts have been reclassified to conform to the current year presentation. Specifically, interest expense has been reclassified from interest income to interest expense in the consolidated statement of operations and comprehensive loss. These reclassifications had no effect on the reported results of operations.

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 and judgments are used in the following areas, among others: the alternative future use of assets used in research and development activities, future undiscounted cash flows and subsequent fair value estimates used to assess potential and measure any impairment of long-lived assets, including goodwill and intangible assets, and the measurement of right-of-use assets and lease liabilities, gross-to-net revenue calculations, stock-based compensation expense, accrued expenses, income taxes, the assessment of the Company's ability to fund its operations for at least the next twelve months from the date of issuance of these financial statements, and the assessment of the likelihood and magnitude of losses that may be sustained upon resolution of contingencies.

Inventory

Inventories are stated at the lower of cost or net realizable value under the first-expired, first-out ("FEFO") methodology. Given human gene therapy products are a new and novel category of therapeutics and future economic benefit is not probable until regulatory approval for the product has been obtained, the Company has only considered inventory for capitalization upon regulatory approval. Manufacturing costs incurred prior to regulatory approval for pre-launch inventory that did not qualify for capitalization and clinical manufacturing costs are charged to research and development expense in the Company’s consolidated statements of operations and comprehensive loss as costs are incurred. Additionally, inventory that initially qualifies for capitalization but that may ultimately be used to produce clinical drug product is expensed as research and development expense when it has been designated for the manufacture of clinical drug product.

Inventory consists of cell banks, plasmids, LVV, other materials and compounds sourced from third party suppliers and utilized in the manufacturing process, and drug product which has been produced for the treatment of specific patients, that are owned by the Company until infusion.

Management periodically reviews inventories for excess or obsolescence, considering factors such as sales forecasts compared to quantities on-hand and firm purchase commitments as well as remaining shelf life of on-hand inventories. The Company writes down its inventory that is in excess, obsolete or otherwise unmarketable to its estimated net realizable value in the period in which the impairment is first identified. Any such adjustments are included as a component of cost of goods sold within cost of product revenue in the Company’s consolidated statements of operations and comprehensive loss.

Revenue recognition

Under ASC Topic 606, *Revenue from Contracts with Customers* (“Topic 606”), an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration that the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of Topic 606, the entity performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price, including variable consideration, if any; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect the consideration to which it is entitled in exchange for the goods or services it transfers to the customer.

Once a contract is determined to be within the scope of Topic 606, the Company assesses the goods or services promised within each contract and determines those that are performance obligations. Arrangements that include rights to additional goods or services that are exercisable at a customer's discretion are generally considered options. The Company assesses if these options provide a material right to the customer and if so, they are considered performance obligations.

The Company assesses whether each promised good or service is distinct to identify the performance obligations in the contract. This assessment involves subjective determinations and requires management to make judgments about the individual promised goods or services and whether such are separable from the other aspects of the contractual relationship. Promised goods and services are considered distinct provided that: (i) the customer can benefit from the good or service either on its own or together with other resources that are readily available to the customer (that is, the good or service is capable of being distinct) and (ii) the entity's promise to transfer the good or service to the customer is separately identifiable from other promises in the contract (that is, the promise to transfer the good or service is distinct within the context of the contract).

The transaction price is then determined and allocated to the identified performance obligations in proportion to their standalone selling prices ("SSP") on a relative SSP basis. SSP is determined at contract inception and is not updated to reflect changes between contract inception and when the performance obligations are satisfied.

If the consideration promised in a contract includes a variable amount, the Company estimates the amount of consideration to which it will be entitled in exchange for transferring the promised goods or services to a customer. The Company determines the amount of variable consideration by using the expected value method or the most likely amount method. The Company includes the unconstrained amount of estimated variable consideration in the transaction price. The amount included in the transaction price is constrained to the amount for which it is probable that a significant reversal of cumulative revenue recognized will not occur. At the end of each subsequent reporting period, the Company re-evaluates the estimated variable consideration included in the transaction price and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis in the adjustment period.

In determining the transaction price, the Company adjusts consideration for the effects of the time value of money if the timing of payments provides the Company with a significant benefit of financing. The Company does not assess whether a contract has a significant financing component if the expectation at contract inception is such that the period between payment by the licensees and the transfer of the promised goods or services to the licensees will be one year or less. The Company assessed each of its revenue generating arrangements to determine whether a significant financing component exists and concluded that a significant financing component does not exist in any of its arrangements.

The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) each performance obligation is satisfied, either at a point in time or over time, and if over time recognition is based on the use of an output or input method.

Product revenue

In 2022, the Company received approval of ZYNTEGLO and SKYSONA from the FDA. In 2023, the Company received approval of LYFGENIA from the FDA. The amount of revenue recognized by the Company is equal to the amount of consideration that is expected to be received from the sale of product to its customers. The Company uses Specialty Distributors ("SD") and Specialty Pharmacies ("SP") to deliver product to the Qualified Treatment Centers ("QTC"). Revenue is only recognized when the performance obligation is satisfied. The Company recognizes revenue upon infusion to the patient. To determine whether a significant reversal will occur in future periods, the Company will assess both the likelihood and magnitude of any such potential reversal of revenue. Gross product revenue is reduced by outcomes-based rebates, other rebates and distributor fees.

Rebates expense

Rebates are based on contractual arrangements or statutory requirements and include amounts due to Medicaid agencies and third-party payers. These amounts may vary by product and payer. Rebates are estimated primarily based on product sales, including product mix and pricing, historical and estimated payer mix and discount rates, among other inputs, which require significant estimates and judgment. The Company assesses and updates estimates each reporting period to reflect actual claims and other current information.

Rebates that are payable to Medicaid agencies and third-party payers are recorded in accrued expenses and other current liabilities on the Company's consolidated balance sheets.

Distributor fees

The Company pays distribution fees to SDs and SPs in connection with the sales of our product. These distributor fees are based on a contractually determined fixed percentage of sales.

Other revenue

In 2021, the Company entered into a grant agreement with the Bill and Melinda Gates Foundation. The Company recognizes grant revenue in accordance with ASC 958-605, *Revenue Recognition Not-for-Profit Entities*, when qualifying costs are incurred and barriers to restriction have been overcome. When grant funds are received after costs have been incurred, the Company records revenue and a corresponding grant receivable. Cash received from grants in advance of incurring qualifying costs is recorded as deferred revenue and recognized as revenue when qualifying costs are incurred. In 2023, the Company ceased further research work and is in the process of winding down such collaboration.

Recent accounting pronouncements

In November 2023, the FASB issued ASU 2023-07, Segment Reporting (Topic 280): *Improvements to Reportable Segment Disclosures* to update reportable segment disclosure requirements, primarily through enhanced disclosures about significant segment expenses and information used to assess segment performance and requires companies to disclose all annual disclosures about segments in interim periods. The ASU also requires companies with a single reportable segment to provide all disclosures required by Topic 280 – Segment Reporting. This update is effective beginning with the Company's 2024 fiscal year annual reporting period and interim periods beginning thereafter. The Company is currently evaluating the impact that the adoption of this standard will have on its consolidated financial statements and disclosures.

In December 2023, the FASB issued ASU 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures*. This ASU expands disclosures in an entity's income tax rate reconciliation table and disclosures regarding taxes paid both in the U.S. and foreign jurisdictions. This update is effective beginning with the Company's 2025 fiscal year annual reporting period. The Company is currently evaluating the impact to its income tax disclosures.

In March 2024, the FASB issued ASU 2024-01, *Compensation-Stock Compensation (Topic 718): Scope Application of Profits Interest and Similar Awards*. This update clarifies the scope of "Profit Interest" and similar awards and adds an illustrative example to the existing ASC 718 standard that includes four fact patterns to demonstrate how an entity should apply the scope guidance in paragraph 718-10-15-3 to determine whether a profits interest award should be accounted for in accordance with Topic 718. The amendments in this ASU are effective for annual periods beginning after December 15, 2024, and interim periods within those annual periods. Early adoption is permitted for interim and annual financial statements not yet issued or made available for issuance. The amendments in this ASU should be applied either (1) retrospectively to all prior periods presented in the financial statements or (2) prospectively to profits interest and similar awards granted or modified on or after the date at which the entity first applies the amendments. The Company is currently evaluating the impact that the adoption of this standard will have on its consolidated financial statements and disclosures.

In March 2024, the FASB issued ASU 2024-02 "*Codification Improvements—Amendments to Remove References to the Concepts Statements"*, which removes various references to concepts statements from the FASB Accounting Standards Codification. This ASU is effective for the Company beginning in the first quarter of fiscal year 2026, with early adoption permitted. The Company expects the new guidance will have an immaterial impact on its consolidated financial statements and intends to adopt the guidance when it becomes effective in the first quarter of fiscal year 2026.

3. Product revenue and reserves

For the three months ended June 30, 2024 and 2023, the Company recorded $16.1 million and $6.8 million, respectively, of product revenue. For the six months ended June 30, 2024 and 2023, the Company recorded $34.7 million and $9.1 million, respectively, of product revenue. Product revenue by therapy represents:

	For the three months ended June 30,		For the six months ended June 30,	
	2024	2023	2024	2023
ZYNTEGLO	$ 11,158	$ 4,180	$ 29,719	$ 4,180
SKYSONA	4,943	2,657	4,943	4,953
Total product revenue, net	$ 16,101	$ 6,837	$ 34,662	$ 9,133

Five individual customers accounted for 80% of product revenue for the six months ended June 30, 2024 and two individual customer accounted for 100% of product revenue for the six months ended June 30, 2023.

The Company considers there to be revenue concentration risks for customers that represent product revenues that exceed 10% of total product revenue. The concentration of the Company's product revenue within a particular customer may have a material adverse effect on the Company's revenue and results of operations if sales with the respective customer experience difficulties. All product revenue during the six months ended June 30, 2024 and 2023 were within the United States.

The following table summarizes an analysis of the change in reserves for gross to net deductions for the periods indicated:

	Total
Balance at December 31, 2023	$ 5,365
Provision for rebates	7,536
Payments/credits	—
Balance at June 30, 2024	$ 12,901

4. Fair value measurements

The following table sets forth the Company's assets and liabilities that are measured at fair value on a recurring basis as of June 30, 2024 and December 31, 2023 (in thousands):

Description	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
June 30, 2024				
Assets:				
Cash and cash equivalents	$ 144,067	$ 144,067	$ —	$ -
Marketable securities:				
U.S. government agency securities and treasuries	—	—	—	-
Due from factor:				
Beneficiary interest in factored invoices	1,440	—	—	1,44
Total	$ 145,507	$ 144,067	$ —	$ 1,44
December 31, 2023				
Assets:				
Cash and cash equivalents	$ 221,755	$ 221,755	$ —	$ -
Marketable securities:				
U.S. government agency securities and treasuries	—	—	—	-
Receivables and other current assets:				
Beneficiary interest in factored invoices	560	—	—	56
Total	$ 222,315	$ 221,755	$ —	$ 56

Cash and cash equivalents

The Company considers all highly liquid securities with original final maturities of 90 days or less from the date of purchase to be cash equivalents. As of June 30, 2024 and December 31, 2023, cash and cash equivalents comprise funds in cash and money market accounts held at multiple banking and asset management institutions.

Marketable securities

Marketable securities classified as Level 2 within the valuation hierarchy generally consist of U.S. government agency securities and treasuries, corporate bonds, and commercial paper. The Company estimates the fair values of these marketable securities by taking into consideration valuations obtained from third-party pricing sources. These pricing sources utilize industry standard valuation models, including both income and market-based approaches, for which all significant inputs are observable, either directly or indirectly, to estimate fair value. These inputs include market pricing based on real-time trade data for the same or similar securities, issuer credit spreads, benchmark yields, and other observable inputs. The Company validates the prices provided by its third-party pricing sources by understanding the models used, obtaining market values from other pricing sources and analyzing pricing data in certain instances.

The amortized cost of available-for-sale debt securities is adjusted for amortization of premiums and accretion of discounts to the earliest call date for premiums or to maturity for discounts. There were no and $0.1 million of realized losses recognized on the sale or maturity of available-for-sale debt securities during the six months ended June 30, 2024 and 2023, respectively.

Accrued interest receivable on the Company's available-for-sale debt securities totaled $0.2 million and $0.3 million as of June 30, 2024 and December 31, 2023, respectively. No accrued interest receivable was written off during the three and six months ended June 30, 2024 or 2023.

Factoring agreement

Due from factor classified as Level 3 within the valuation hierarchy consists of beneficiary interest in transferred invoices. The Company estimates the fair value of the beneficiary interest based on the estimated cash flows after applying counterparty and credit risk adjustments associated with the factoring agent and distributors, respectively. As of June 30, 2024, no adjustment to the beneficiary interest in invoices sold was deemed an unobservable input and was determined based from ongoing credit evaluations and historical experience with aging of such invoices, among other factors. A significant change to this input could result in a significantly lower or higher fair value measurement.

The following table shows a reconciliation of the beginning and ending balances for Level 3 financial liabilities measured at fair value on a recurring basis for the six months ended June 30, 2024:

	For the six months ended June 30, 2024
Level 3 financial assets, beginning of period	$ 560
Beneficiary interest obtained in transferred invoices	3,680
Proceeds from previously transferred invoices	(2,800)
Level 3 financial assets, end of period	$ 1,440

5. Inventory

Inventory, net, consists of the following (in thousands):

	As of June 30, 2024	As of December 31, 2023
Raw materials	$ 3,092	$ 2,329
Work in progress	28,167	17,375
Finished goods	2,071	3,215
Inventory	$ 33,330	$ 22,919

Raw materials inventory consists of completed materials purchased directly from third party suppliers. Work in progress inventory consists of materials manufactured at contract manufacturing organizations ("CMOs") that are either partially completed, fully manufactured but are pending quality acceptance, or completed meeting quality acceptance standards to be used in the manufacture of drug product and drug products that are either partially completed or fully manufactured but are pending quality acceptance. Finished goods are completed and quality approved drug products that are either awaiting shipment, in-transit or delivered to a qualified treatment center, but have not yet been infused in a patient.

6. Factoring agreement

The company sells rights to future revenues associated with invoices issued upon delivery of drug product to QTCs. The upfront payments are treated as a short-term liability, presented as due to factor on the Company's consolidated balance sheets until the right to consideration from the customer is deemed unconditional. The remaining invoice amount payable to the Company at infusion is considered a beneficial interest in the factored invoice and represents the extent of our continued involvement in the sale of invoices.

Due to Factor

For the three and six months ending June 30, 2024, the Company collected $20.7 million and $35.8 million, respectively, in cash receipts prior to an unconditional right to consideration and derecognized $18.0 million and $33.1 million, respectively, of due to factor amounts as a result of patient drug product infusions. Amounts presented as due to factor on the consolidated balance sheets would be subject to payment based on the repurchase requirements that exist prior to the infusion date in the factoring agreement.

Due from Factor

For the three and six months ending June 30, 2024, the company obtained $2.1 million and $3.7 million, respectively, of beneficiary interest in invoices and collected $1.7 million and $2.8 million, respectively, in cash receipts. Uncollected amounts

presented as due from factor on the consolidated balance sheets are net of accrued fees of $0.1 million. The maximum loss exposure is $1.4 million at June 30, 2024.

The total loss from the sale of customer invoices is estimated on the patient infusion date of the drug product and was $0.3 million and $0.6 million, respectively, for the three and six months ending June 30, 2024. In addition to the loss from the sale of invoices the Company has incurred $0.4 million and $0.8 million, respectively, in servicing fees for the three and six months ending June 30, 2024.

7. Accrued expenses and other current liabilities

Accrued expenses and other current liabilities consist of the following (in thousands):

	As of June 30, 2024	As of December 31, 2023
Accrued CMO and CRO costs	$ 19,665	$ 24,824
Accrued employee compensation	16,669	19,972
Accrued rebates	12,942	5,365
Accrued goods and services	4,295	8,392
Accrued professional fees	3,733	2,531
Accrued refund liability	—	5,600
Other	8,585	6,504
Total accrued expenses and other current liabilities	$ 65,889	$ 73,188

8. Term loan debt

2024 Term Loan

On March 15, 2024 (the "Effective Date"), the Company entered into a Loan and Security Agreement (the "LSA") and a Warrant Agreement (the "Warrant Agreement") with Hercules Capital and funds managed by Hercules Capital (collectively, "Hercules" or "the lenders"). Under the terms of the LSA, the Company may borrow up to an aggregate principal amount of $175.0 million over the term of the agreement, in four separate tranches (the "Term Loans") dependent on the achievement of certain milestones. On the Effective Date, the Company drew down the initial $75.0 million of gross proceeds ("Initial Loan"), and paid initial debt issuance costs of $3.5 million, inclusive of legal fees of approximately $2.7 million and the original issuance discount ("OID") of $0.8 million, or 1% of the drawn amount, in accordance with the terms of the LSA. Additional amounts are available to borrow in two separate $25.0 million tranches based on the Company meeting certain pre-defined milestone achievements associated with cell collection from LYFGENIA patients (35 patients on or before September 30, 2024 or 55 patients on or before December 31, 2024) ("Tranche 2") and compliance with a gross profit metric (no later than the period ending June 30, 2025) ("Tranche 3"). Additionally, the Company may request up to an additional $50.0 million through December 15, 2026 from Hercules ("Tranche 4"). The lenders have no obligation to fund any amounts under Tranche 4 as funding is conditioned on approval by the lenders' investment committee.

The Term Loans bear both cash interest and paid-in-kind interest ("PIK"). The cash interest is due on the first business day of each month ("Payment Date") and will be equal to the WSJ prime rate ("prime rate") plus 1.45% (floored at 9.95%). The PIK interest is fixed at 2.45% and is to be capitalized and added to the outstanding principal on each Payment Date. Unless prepaid by the Company at their discretion, subject to certain prepayment penalties and conditions, the Term Loans are repayable in monthly interest-only payments until April 1, 2027, or April 1, 2028, if the Company has achieved, no later than December 31, 2026, certain financial metrics (the "Performance Milestone"). After the expiration of the interest-only payment period, the Term Loans are repayable in equal monthly payments of principal and accrued interest until maturity. The Term Loans will mature on April 1, 2029.

The 2024 Term Loan is collateralized by substantially all the Company's assets. In addition to other covenants, the 2024 Term Loan contains affirmative covenants as well as certain financial covenants, including a minimum cash coverage requirement of 40% of the outstanding principal of the term loan and a minimum net product revenue requirement that commences with the quarter ending September 30, 2024. The Company was in compliance with the minimum cash coverage covenant as of June 30, 2024, but the Company projects that it may not maintain its minimum cash coverage requirement within the next 12 months (see the Company's going concern assessment in Note 1, *Description of the business*). As such, the term loan is presented as a current liability as of June 30, 2024.

The Company also issued warrants under the Warrant Agreement for the right to purchase shares of the Company's common stock to Hercules, with the number of shares to be based on the amounts borrowed under the LSA (the "Warrants"). The Warrants are exercisable for a number of shares of common stock equal to the quotient of (i.) 5% times the aggregate original principal of amounts drawn under the LSA divided by (ii.) the exercise price of $1.45 per share. As of June 30, 2024, the Company had issued 2.6 million warrants associated with the Initial Loan ("Initial Warrants") to purchase common stock at an exercise price of $1.45 per share and a contractual life of 7 years from the Effective Date.

The Warrants have been recorded at their relative fair value using the Black-Scholes option-pricing model and the following assumptions: no dividend yield, expected volatility of 81.1%, risk free rate of 4.3%, and expected term of 7 years, equal to the life of the warrant. The relative fair value allocated to the initial warrants was $2.7 million, which was classified as additional-paid-in-capital and recorded as a debt discount which will be amortized, together with debt issuance costs, to interest expense using the effective interest method over the life of the loan at an effective interest rate of 15.7%. For the three and six months ended June 30, 2024, the Company recorded approximately $2.7 million and $3.2 million in interest expense associated with the Term Loans, respectively, inclusive of amounts related to the Warrants.

Refer to Note 16, *Subsequent Events*, for further discussion on the amendments to the LSA and the Warrant Agreement subsequent to the six months ended June 30, 2024.

9. Leases

The Company leases certain office and laboratory space, primarily located in Somerville, Massachusetts. Additionally, the Company has embedded leases through its agreements with CMOs and a contract testing organization ("CTO") in both the United States and internationally. Except as described below, there have been no material changes in lease obligations from those disclosed in Note 11 to the consolidated financial statements included in the Company's 2023 Annual Report on Form 10-K.

Embedded leases

Periodically, throughout the three and six months ended June 30, 2024, the Company amended several of its embedded contract manufacturing leases, some of which were accounted for as lease modifications under ASC 842. The lease modifications primarily relate to the execution of new statements of work with the vendors or the extension of contractual terms.

As it relates to embedded leases related to drug product manufacturing and quality testing, due to lease modification adjustments and amortization, the Company increased its finance lease right-of-use assets in the amount of $22.4 million during the six months ended June 30, 2024.

As it relates to embedded leases related to drug substance manufacturing, due to lease modification adjustments and amortization, the Company decreased its finance lease right-of-use assets in the amount of $8.9 million during the six months ended June 30, 2024.

The following paragraph describes a significant lease modification to the Company's embedded drug substance manufacturing lease that was executed during the three months ended June 30, 2024.

In June 2024, the Company extended its embedded lease for the manufacturing of vector and was obligated under the defined production schedule through July 1, 2024. Refer to Note 16, *Subsequent Events*, for further discussion on the extension of this agreement subsequent to June 30, 2024.

Summary of all lease costs recognized under ASC 842

The following table contains a summary of the lease costs recognized under ASC 842 and other information pertaining to the Company's operating and finance leases for the three months and six months ended June 30, 2024 and 2023 (in thousands):

	For the three months ended June 30,		For the six months ended June 30,	
	2024	2023	2024	2023
		(As Restated)		(As Restated)
Finance leases				
Interest expense	$ 2,710	$ 3,750	$ 7,057	$ 8,017
Amortization expense	15,375	5,228	29,703	10,444
Total fixed finance lease cost	$ 18,085	$ 8,978	$ 36,760	$ 18,461
Operating leases				
Fixed lease cost	$ 9,486	$ 11,470	$ 18,973	$ 22,941
Total fixed operating lease cost	$ 9,486	$ 11,470	$ 18,973	$ 22,941
Variable lease cost	6,863	5,091	12,999	11,953
Short-term lease cost	80	46	155	97
Total lease cost	$ 34,514	$ 25,585	$ 68,887	$ 53,452
Operating sublease income	$ 10,260	$ 10,373	$ 20,606	$ 20,481
Cash paid in the measurement of lease liabilities				
Operating cash flows used for operating leases			$ 8,303	$ 13,276
Operating cash flows used for finance leases			$ 1,411	$ 7,198
Financing cash flows for finance leases			$ 48,645	$ 28,504

Supplemental balance sheet information related to leases was as follows:

	As of June 30, 2024	As of June 30, 2023
Weighted average remaining lease term - finance leases	1.26 years	2.18 years
Weighted average discount rate - finance leases	11.18 %	14.02 %
Weighted average remaining lease term - operating leases	6.52 years	7.4 years
Weighted average discount rate - operating leases	6.99 %	7.03 %

As of June 30, 2024, future minimum commitments under ASC 842 under the Company's leases were as follows (in thousands):

	Operating Leases	Financing Leases
Remaining Lease Payments		
Remainder of 2024	$ 19,550	$ 78,746
2025	37,084	41,797
2026	35,719	3,408
2027	36,742	2,548
2028	37,795	1,043
2029 and thereafter	81,986	—
Total	$ 248,876	$ 127,542
Less: Imputed Interest	(49,289)	(6,552)
Present Value of Lease Liabilities	$ 199,587	$ 120,990

10. Commitments and contingencies

Lease commitments

The Company leases certain office and laboratory space and has embedded leases at CMOs and a CTO. As of June 30, 2024, the Company has commitments arising from a forward starting lease that had not yet commenced related to an embedded equipment lease with a CMO. This lease is expected to commence in 2025 with an initial lease term of three years. Fixed commitments under this contract approximate $56.6 million. The following table presents the non-cancelable contractual obligations arising from this arrangement as of June 30, 2024 (in thousands):

	Future commitment
Remainder of 2024	$ —
2025	13,563
2026	17,858
2027	17,858
2028	7,310
2029 and thereafter	—
Total purchase commitments	$ 56,589

Refer to Note 9, *Leases,* for further information on the terms of these lease agreements.

Litigation

From time to time, the Company is party to various claims and complaints arising in the ordinary course of business, including securities class action litigation and intellectual property litigation. 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. 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 generally unlimited. Accruals for loss contingencies are recognized when a loss is probable, and the amount of such loss can be reasonably estimated. The Company has not accrued for a loss for any matter as a loss is not probable and a loss, or a range of loss, is not reasonably estimable.

The Company also indemnifies each of its officers and directors for certain events or occurrences, subject to certain limits, while the officer or director is or was serving at the Company's request in such capacity, as permitted under Delaware law and in accordance with its certificate of incorporation and by-laws. The term of the indemnification period lasts as long as such officer or director may be subject to any proceeding arising out of acts or omissions of such officer or director in such capacity. The maximum amount of potential future indemnification is unlimited; however, the Company currently holds director and officer liability insurance. This insurance allows the transfer of risk associated with the Company's exposure and may enable it to recover a portion of any future amounts paid. The Company believes that the fair value of these indemnification obligations is minimal. Accordingly, it has not recognized any liabilities relating to these obligations.

There have been no material changes in claims and complaints from those disclosed in Note 12 to the consolidated financial statements included in the Company's 2023 Annual Report on Form 10-K.

11. Equity

On January 18, 2023, the Company entered into an underwriting agreement (the "January Underwriting Agreement") with Goldman and J.P. Morgan Securities LLC in connection with the public offering, issuance, and sale by the Company of 20.0 million shares of the Company's common stock at a public offering price of $6.00 per share, less underwriting discounts and commissions, pursuant to an effective shelf registration statement on Form S-3 and a related prospectus supplement filed with the Securities and Exchange Commission. Under the terms of the January Underwriting Agreement, the Company also granted the underwriters an option exercisable for 30 days to purchase up to an additional 3.0 million shares of common stock at the public offering price, less underwriting discounts and commissions, which the underwriters exercised in full. The offering closed on January 23, 2023. The Company received aggregate net proceeds of $130.5 million.

In August 2023, the Company entered into an Open Market Sales Agreement (the "Sales Agreement") with Jefferies LLC ("Jefferies") to sell shares of the Company's common stock up to $125.0 million, from time to time, through an "at the market" equity offering program under which Jefferies will act as sales agent. As of June 30, 2024, the Company has made no sales pursuant to the Sales Agreement.

On December 19, 2023, the Company entered into an underwriting agreement (the "December Underwriting Agreement") with Goldman and J.P. Morgan Securities LLC, in connection with the public offering, issuance, and sale by the Company of 83.3 million shares of the Company's common stock at a public offering price of $1.50 per share, less underwriting discounts and commissions, pursuant to an effective shelf registration statement on Form S-3 and a related prospectus supplement filed with the Securities and Exchange Commission. Under the terms of the December Underwriting Agreement, the Company also granted the underwriters an option exercisable for 30 days to purchase up to an additional 12.5 million shares of common stock at the public offering price, less underwriting discounts and commissions, which option was not exercised. The offering closed on December 22, 2023. The Company received aggregate net proceeds of $118.1 million.

12. Stock-based compensation

In June 2023, the Company's stockholders approved the bluebird bio, Inc. 2023 Incentive Award Plan (the "2023 Plan"), which replaced the 2013 Stock Option and Incentive Plan ("2013 Plan"). Following approval of the 2023 Plan, no further awards will be granted under the 2013 Plan. As of June 30, 2024, the total number of shares of common stock available for issuance under the 2023 Plan was approximately 2.7 million.

Stock-based compensation expense

The Company recognized stock-based compensation expense totaling $3.2 million and $5.8 million during the three months ended June 30, 2024 and 2023, respectively. The Company recognized stock-based compensation expense totaling $7.0 million and $11.1 million during the six months ended June 30, 2024 and 2023, respectively. Stock-based compensation expense recognized by award type is included within the condensed consolidated statements of operations and comprehensive loss was as follows (in thousands):

	For the three months ended June 30,		For the six months ended June 30,	
	2024	2023	2024	2023
Stock options	$ 1,192	$ 1,710	$ 2,422	$ 3,4
Restricted stock units	2,012	3,969	4,463	7,6
Employee stock purchase plan and other	2	75	83	
	$ 3,206	$ 5,754	$ 6,968	$ 11,1

Stock-based compensation expense by classification included within the condensed consolidated statements of operations and comprehensive loss was as follows (in thousands):

	For the three months ended June 30,		For the six months ended June 30,	
	2024	2023	2024	2023
Cost of product revenue	289	(10)	780	93
Selling, general and administrative	1,798	2,681	4,133	5,042
Research and development	1,119	3,083	2,055	6,010
	$ 3,206	$ 5,754	$ 6,968	$ 11,145

During the six months ended June 30, 2024 and 2023, the Company had $0.3 million and $1.1 million of stock compensation expense that was capitalized into inventory.

Stock options

The following table summarizes the stock option activity under the Company's equity award plans excluding awards held by employees of 2seventy bio:

	Shares (in thousands)	Weighted-average exercise price per share
Outstanding at December 31, 2023	4,227	$ 14.16
Granted	2,672	$ 1.54
Exercised	—	$ —
Canceled, forfeited, or expired	(600)	$ 10.86
Outstanding at June 30, 2024	6,299	$ 9.13
Exercisable at June 30, 2024	2,196	$ 19.60
Vested and expected to vest at June 30, 2024	6,299	$ 9.13

During the six months ended June 30, 2024, no stock options were exercised.

Restricted stock units

The following table summarizes the restricted stock unit activity under the Company's equity award plans excluding awards held by employees of 2seventy bio:

	Shares (in thousands)	Weighted-average grant date fair value
Unvested at December 31, 2023	4,207	$ 6.08
Granted	2,337	$ 1.54
Vested	(970)	$ 6.76
Forfeited	(577)	$ 4.09
Unvested at June 30, 2024	4,997	$ 4.05

Employee stock purchase plan

In June 2013, the Company's board of directors adopted its 2013 Employee Stock Purchase Plan ("2013 ESPP"), which authorized the initial issuance of up to a total of 0.2 million shares of the Company's common stock to participating employees. In June 2021, the Company amended the 2013 ESPP to authorize an additional 1.4 million shares of the Company's common stock available to participating employees. During each of the six months ended June 30, 2024 and 2023, 0.1 million shares and 0.1 million shares, respectively, of common stock were issued under the 2013 ESPP.

13. Income taxes

Deferred tax assets and deferred tax liabilities are recognized based on temporary differences between the financial reporting and tax basis of assets and liabilities using statutory rates. A valuation allowance is recorded against deferred tax assets if it is more likely than not that some or all of the deferred tax assets will not be realized. Due to the uncertainty surrounding the realization of the favorable tax attributes in future tax returns, the Company has recorded a full valuation allowance against the Company's otherwise recognizable net deferred tax assets. The tax benefit recognized during the three and six months ended June 30, 2024 was $0.0 million due to the full valuation allowance.

14. Net loss per share

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 (in thousands):

	For the three and six months ended June 30,	
	2024	2023
Outstanding stock options[(1)]	7,495	6,109
Restricted stock units[(1)]	5,000	4,682
Warrants	2,586	—
ESPP shares and other	87	—
	15,168	10,791

[(1)] Outstanding stock options and restricted stock units include awards outstanding to employees of 2seventy bio.

Net loss per share for the three and six months ended June 30, 2024 was $0.42 and $0.78, respectively. Net loss per share for the three and six months ended June 30, 2023 was $0.58 and $0.41, respectively.

15. Restatement of Previously Issued Financial Statements

As further described below, as well as in Note 2 - Basis of Presentation, the Company identified several prior period misstatements that impacted its unaudited quarterly condensed consolidated financial statements for the three and six months ended June 30, 2023. Such restated and unaudited quarterly financial data and the related impacted amounts were presented in the Company's Annual Report on Form 10-K for the year ended December 31, 2023.

As part of the restatement, the Company recorded adjustments to correct the material misstatements related to accounting for embedded leases and other immaterial errors.

The following tables present the restated unaudited Condensed Consolidated Financial Information for the three and six months ended June 30, 2023.

Consolidated Statements of Operations and Comprehensive Loss

	For the three months ended June 30, 2023			
	As Previously Reported	Adjustments to Embedded Leases	Other Adjustments	As Restated
Revenue:				
Product revenue, net	$ 6,837	$ —	$ —	$ 6,837
Other revenue	53	—	—	53
Total revenues	6,890	—	—	6,890
Cost of product revenue	9,564	(2,867)	—	6,697
Gross margin	(2,674)	2,867	—	193
Operating expenses:				
Research and development	42,274	(10,189)	(637)	31,448
Selling, general and administrative	40,349	113	—	40,462
Total operating expenses	82,623	(10,076)	(637)	71,910
Loss from operations	(85,297)	12,943	637	(71,717)
Interest income	2,679	—	—	2,679
Interest expense	—	(3,750)	—	(3,750)
Other income, net	9,630	289	—	9,919
Loss before income taxes	(72,988)	9,482	637	(62,869)
Income tax (expense) benefit	80	—	—	80
Net loss	(72,908)	9,482	637	(62,789)
Net loss per share - basic (1)	$ (0.67)	$ 0.09	$ 0.01	$ (0.58)
Net loss per share - diluted (1)	$ (0.67)	$ 0.09	$ 0.01	$ (0.58)
Weighted-average number of common shares used in computing net loss per share - basic:	108,685	108,685	108,685	108,685
Weighted-average number of common shares used in computing net loss per share - diluted:	108,685	108,685	108,685	108,685
Other comprehensive income (loss):				
Other comprehensive income (loss), net of tax (benefit) expense of $0.0 million for the three months ended June 30, 2023	722	—	—	722
Total other comprehensive income (loss)	722	—	—	722
Comprehensive loss	$ (72,186)	$ 9,482	$ 637	$ (62,067)

(1) Due to differences in rounding to the nearest cent per basic or diluted share, totals may not equal the sum of the line items.

	For the six months ended June 30, 2023			
	As Previously Reported	Adjustments to Embedded Leases	Other Adjustments	As Restated
Revenue:				
Product revenue, net	$ 9,133	$ —	$ —	$ 9,133
Other revenue	138	—	—	138
Total revenues	9,271	—	—	9,271
Cost of product revenue	12,940	(731)	—	12,209
Gross margin	(3,669)	731	—	(2,938)
Operating expenses:				
Research and development	88,418	(14,705)	(678)	73,035
Selling, general and administrative	77,703	226	—	77,929
Total operating expenses	166,121	(14,479)	(678)	150,964
Gain from sale of priority review voucher, net	92,930	—	—	92,930
Loss from operations	(76,860)	15,210	678	(60,972)
Interest income	5,507	—	—	5,507
Interest expense	(3)	(8,017)	—	(8,020)
Other income, net	19,608	(62)	—	19,546
Loss before income taxes	(51,748)	7,131	678	(43,939)
Income tax (expense) benefit	80	—	—	80
Net loss	(51,668)	7,131	678	(43,859)
Net loss per share - basic	$ (0.49)	$ 0.07	$ 0.01	$ (0.41)
Net loss per share - diluted	$ (0.49)	$ 0.07	$ 0.01	$ (0.41)
Weighted-average number of common shares used in computing net loss per share - basic:	105,819	105,819	105,819	105,819
Weighted-average number of common shares used in computing net loss per share - diluted:	105,819	105,819	105,819	105,819
Other comprehensive income (loss):				
Other comprehensive income (loss), net of tax (benefit) expense of $0.0 million for the six months ended June 30, 2023	1,706	—	—	1,706
Total other comprehensive income (loss)	1,706	—	—	1,706
Comprehensive loss	$ (49,962)	$ 7,131	$ 678	$ (42,153)

Statements of Changes in Stockholders' Equity:

	Common stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
As Previously Reported						
Balances at December 31, 2022	82,923	$ 830	$ 4,186,086	$ (4,070)	$ (3,986,503)	$ 196,343
Vesting of restricted stock	382	3	(198)	—	—	(195)
Exercise of stock options	3	—	7	—	—	7
Purchase of shares under ESPP	62	1	226	—	—	227
Issuance of common stock for private equity placement	23,000	230	130,061	—	—	130,291
Stock-based compensation	—	—	5,843	—	—	5,843
Other comprehensive income (loss)	—	—	—	984	—	984
Net income (loss)	—	—	—	—	21,240	21,240
Balances at March 31, 2023	106,370	$ 1,064	$ 4,322,025	$ (3,086)	$ (3,965,263)	$ 354,740
Adjustments to Embedded Leases						
Balances at December 31, 2022	$ —	$ —	$ —	$ —	$ (59,700)	$ (59,700)
Vesting of restricted stock	—	—	—	—	—	—
Exercise of stock options	—	—	—	—	—	—
Purchase of shares under ESPP	—	—	—	—	—	—
Issuance of common stock for private equity placement	—	—	—	—	—	—
Stock-based compensation	—	—	—	—	—	—
Other comprehensive income (loss)	—	—	—	—	—	—
Net income (loss)	—	—	—	—	(2,351)	(2,351)
Balances at March 31, 2023	—	$ —	$ —	$ —	$ (62,051)	$ (62,051)
Other Adjustments						
Balances at December 31, 2022	—	—	(98)	—	(2,212)	(2,310)
Vesting of restricted stock	—	—	—	—	—	—
Exercise of stock options	—	—	—	—	—	—
Purchase of shares under ESPP	—	—	—	—	—	—
Issuance of common stock for private equity placement	—	—	—	—	—	—
Stock-based compensation	—	—	—	—	—	—
Other comprehensive income (loss)	—	—	—	—	—	—
Net income (loss)	—	—	—	—	41	41
Balances at March 31, 2023	—	$ —	$ (98)	$ —	$ (2,171)	$ (2,269)
As Restated						
Balances at December 31, 2022	82,923	830	4,185,988	(4,070)	(4,048,415)	134,333
Vesting of restricted stock	382	3	(198)	—	—	(195)
Exercise of stock options	3	—	7	—	—	7
Purchase of shares under ESPP	62	1	226	—	—	227
Issuance of common stock for private equity placement	23,000	230	130,061	—	—	130,291
Stock-based compensation	—	—	5,843	—	—	5,843
Other comprehensive income (loss)	—	—	—	984	—	984
Net income (loss)	—	—	—	—	18,930	18,930
Balances at March 31, 2023	106,370	$ 1,064	$ 4,321,927	$ (3,086)	$ (4,029,485)	$ 290,420

	Common stock		Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
As Previously Reported						
Balances at March 31, 2023	106,370	$ 1,064	$ 4,322,025	$ (3,086)	$ (3,965,263)	$ 354,740
Vesting of restricted stock	65	1	(1)	—	—	—
Exercise of stock options	19	—	77	—	—	77
Purchase of shares under ESPP	—	—	—	—	—	—
Issuance of common stock for private equity placement	—	—	—	—	—	—
Stock-based compensation	—	—	6,388	—	—	6,388
Other comprehensive income (loss)	—	—	—	722	—	722
Net income (loss)	—	—	—	—	(72,908)	(72,908)
Balances at June 30, 2023	106,454	$ 1,065	$ 4,328,489	$ (2,364)	$ (4,038,171)	$ 289,019
Adjustments to Embedded Leases						
Balances at March 31, 2023	$ —	$ —	$ —	$ —	$ (62,051)	$ (62,051)
Vesting of restricted stock	—	—	—	—	—	—
Exercise of stock options	—	—	—	—	—	—
Purchase of shares under ESPP	—	—	—	—	—	—
Issuance of common stock for private equity placement	—	—	—	—	—	—
Stock-based compensation	—	—	—	—	—	—
Other comprehensive income (loss)	—	—	—	—	—	—
Net income (loss)	—	—	—	—	9,482	9,482
Balances at June 30, 2023	—	$ —	$ —	$ —	$ (52,569)	$ (52,569)
Other Adjustments						
Balances at March 31, 2023	—	—	(98)	—	(2,171)	(2,269)
Vesting of restricted stock	—	—	—	—	—	—
Exercise of stock options	—	—	—	—	—	—
Purchase of shares under ESPP	—	—	—	—	—	—
Issuance of common stock for private equity placement	—	—	—	—	—	—
Stock-based compensation	—	—	—	—	—	—
Other comprehensive income (loss)	—	—	—	—	—	—
Net income (loss)	—	—	—	—	637	637
Balances at June 30, 2023	—	$ —	$ (98)	$ —	$ (1,534)	$ (1,632)
As Restated						
Balances at March 31, 2023	106,370	1,064	4,321,927	(3,086)	(4,029,485)	290,420
Vesting of restricted stock	65	1	(1)	—	—	—
Exercise of stock options	19	—	77	—	—	77
Purchase of shares under ESPP	—	—	—	—	—	—
Issuance of common stock for private equity placement	—	—	—	—	—	—
Stock-based compensation	—	—	6,388	—	—	6,388
Other comprehensive income (loss)	—	—	—	722	—	722
Net income (loss)	—	—	—	—	(62,789)	(62,789)
Balances at June 30, 2023	106,454	$ 1,065	$ 4,328,391	$ (2,364)	$ (4,092,274)	$ 234,818

Consolidated Statement of Cash Flows

	For the six months ended June 30, 2023			
	As Previously Reported	Adjustments to Embedded Leases	Other Adjustments	As Restated
Cash flows from operating activities:				
Net loss	$ (51,668)	$ 7,131	$ 678	$ (43,859)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation and amortization	2,052	10,274	(76)	12,250
Stock-based compensation expense	11,145	—	—	11,145
Noncash research and development expense (finance lease)	—	434	—	434
Noncash operating lease expense	—	14,966	—	14,966
Gain from sale of priority review voucher	(92,930)	—	—	(92,930)
Excess inventory reserve	3,939	—	2,339	6,278
Other non-cash items	343	—	—	343
Gain on foreign currency exchange rates	—	(281)	—	(281)
Changes in operating assets and liabilities:				
Prepaid expenses and other assets	(9,082)	2,642	—	(6,440)
Inventory	(16,496)	1,838	(2,941)	(17,599)
Operating right of use assets	24,102	(24,102)	—	—
Accounts payable	(14,767)	9,144	—	(5,623)
Accrued expenses and other liabilities	3,625	(573)	—	3,052
Accrued interest payable under finance lease	—	819	—	819
Operating lease liabilities	(19,488)	6,212	—	(13,276)
Deferred revenue	(138)	—	—	(138)
Net cash (used in) provided by operating activities	(159,363)	28,504	—	(130,859)
Cash flows from investing activities:				
Purchase of property, plant and equipment	(937)	—	—	(937)
Purchases of marketable securities	(34,418)	—	—	(34,418)
Proceeds from maturities of marketable securities	26,521	—	—	26,521
Proceeds from sales of marketable securities	5,853	—	—	5,853
Purchase of intangible assets	(868)	—	—	(868)
Proceeds from sale of priority review voucher	92,930	—	—	92,930
Net cash provided by investing activities	89,081	—	—	89,081
Cash flows from financing activities:				
Proceeds from exercise of stock options and ESPP contributions	85	—	—	85
Proceeds from vesting of restricted stock	(196)	—	—	(196)
Principal payments on finance lease	—	(28,504)	—	(28,504)
Proceeds from the secondary public offering, net of issuance costs	130,122	—	—	130,122
Net cash (used in) provided by financing activities	130,011	(28,504)	—	101,507
Increase in cash, cash equivalents and restricted cash	59,729	—	—	59,729
Cash, cash equivalents and restricted cash at beginning of year	158,445	—	—	158,445
Cash, cash equivalents and restricted cash at end of year	$ 218,174	$ —	$ —	$ 218,174
Reconciliation of cash, cash equivalents and restricted cash:				
Cash and cash equivalents	$ 172,872	$ —	$ —	$ 172,872
Restricted cash included in receivables and other current assets	1,364	—	—	1,364
Restricted cash included in restricted cash and other non-current assets	43,938	—	—	43,938
Total cash, cash equivalents and restricted cash	$ 218,174	$ —	$ —	$ 218,174
Supplemental cash flow disclosures:				
Right-of-use assets obtained in exchange for operating lease liabilities	44,968	(44,968)	—	—
Increase (Reduction) of right of use asset and associated operating lease liability due to lease reassessment	(14)	14	—	—
Purchases of property, plant and equipment included in accounts payable and accrued expenses	2,290	—	—	2,290
Right-of-use assets obtained in exchange for finance lease liabilities	—	3,436	—	3,436
Cash paid during the period for income taxes	7	—	—	7

16. Subsequent events

Term Loan Debt

As previously disclosed in Note 8, *Term loan debt*, on March 15, 2024, the Company entered into a five-year term loan facility agreement with Hercules Capital, Inc. ("Hercules") to secure debt financing for up to $175 million, available in four tranches. The first tranche in an amount equal to $75 million was drawn at the time of closing (the "Initial Loan"). The Company may draw upon two additional tranches of $25 million each, subject to satisfaction of certain conditions, including achievement of commercial milestones. The facility also provides a fourth tranche of $50 million, available at the sole discretion of Hercules, until December 15, 2026.

The Loan and Security Agreement ("LSA") requires the Company to comply with customary affirmative and negative covenants, including, among other things, a requirement to deliver annual financial statements within 90 days of each fiscal year and quarterly financial statements within 45 days of each fiscal quarter. A failure to comply with these covenants, or failure to obtain a waiver for any non-compliance, would result in an event of default under the LSA and would allow Hercules to accelerate repayment of the debt, which could materially and adversely affect the business, results of operations and financial condition of the Company. On April 30, 2024, July 9, 2024, August 13, 2024, and August 29, 2024 the Company and Hercules entered into amendments to the LSA providing for revised monthly financial reporting metrics for each month through September 30, 2024 and extension of the deadlines by which the Company must provide certain annual and quarterly financial statements.

On August 13, 2024, the Company and Hercules entered into a third amendment to the LSA (the “Third Amendment”), pursuant to which the parties agreed to, among other things, revised terms for the availability of the second and third tranches of funding under the LSA. In accordance with the Third Amendment, the Company may draw the second tranche of $25.0 million during the period commencing on the date the Company has (x) received at least $75.0 million in gross cash proceeds from qualified financing transactions by December 20, 2024 and (y) completed patient starts (cell collections) for at least 50 LYFGENIA patients by March 31, 2025 or 70 LYFGENIA patients by June 30, 2025 (collectively, the “Tranche 2 Milestone”) and ending on the earlier of (i) the date that is 30 days immediately following achievement of the Tranche 2 Milestone and (ii) July 31, 2025. The Company may draw the third tranche of $25.0 million during the period commencing on the date the Company has (x) received at least $100.0 million in gross cash proceeds from qualified financing transactions by December 20, 2024 or at least $125.0 million by June 30, 2025 and (y) completed 70 drug product deliveries within a given six-month period ending no later than December 31, 2025, at least 40 of which are for LYFGENIA (collectively, the “Tranche 3 Milestone”) and ending on the earlier of (i) the date that is 30 days immediately following the date the Company achieves the Tranche 3 Milestone and (ii) December 31, 2025. Additionally, the Company and Hercules agreed to increase the minimum cash coverage requirement from 40% to 45% of the outstanding principal of the term loan.

Additionally, in connection with entry into the LSA, the Company issued to the lenders warrants to purchase that number of shares of the Company’s common stock equal to five percent of the Initial Loan, or $3.75 million, divided by the volume-weighted average price (“VWAP”) of the Company’s common stock for the ten-day period preceding March 15, 2024 (the “Initial Warrants”). The Company agreed to issue additional common stock warrants to the lenders at the closings of future tranches of funding under the LSA, if any, to purchase that number of shares of common stock equal to five percent of the applicable loan divided by the VWAP of the Company’s common stock for the ten-day period preceding March 15, 2024 (together with the Initial Warrants, the “Warrants”). On August 13, 2024, in connection with the Third Amendment, the Company agreed to amend the exercise price of the Warrants to purchase shares of the Company’s common stock from $1.45 per share to the lesser of the VWAP of the Company’s common stock for the ten-day period preceding August 13, 2024, and the price per share of the Company’s first equity financing event within six months of August 13, 2024. The amendment does not impact the number of shares the lenders may purchase pursuant to the Warrants.

Reduction in force

The Company’s board of directors approved a restructuring action (the "Restructuring") on September 23, 2024, following a comprehensive review of the Company’s operations. The Restructuring includes a reduction of the Company’s workforce by 94 employees, or approximately 25% of employees.

As a result of the Restructuring, the Company estimates that it will incur aggregate charges of approximately $3.3 million to $3.7 million in cash expenditures for severance and employee termination-related costs to be paid out over multiple weeks through the end of the fiscal year ending December 31, 2024, as well as approximately $0.3 million to $0.5 million in non-cash stock-based compensation expense associated with accelerated vesting of RSUs. The Company expects to record a significant portion of these charges in the third quarter of 2024.

Extended agreement for manufacturing of vector

On September 20, 2024, the Company extended its embedded lease for the manufacturing of vector effective September 15, 2024, which previously had an expiration date of July 1, 2024, and was subsequently extended through September 15, 2024.

This extended agreement provides that it will remain in effect until September 25, 2029, and will automatically renew for additional two-year periods unless either party provides advance notice of non-renewal. Either party may terminate the agreement in the event of the other party's material uncured breach or bankruptcy proceedings, or due to certain force majeure events.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following information should be read in conjunction with the unaudited financial information and the notes thereto included in this Quarterly Report on Form 10-Q and the audited financial information and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2023, which was filed with the Securities and Exchange Commission, or the SEC, on September 13, 2024 (the "2023 Annual Report on Form 10-K").

The Company restated the consolidated financial statements for the year ended December 31, 2022 presented in its 2023 Annual Report on Form 10-K. In addition, the Company restated its unaudited quarterly financial data for the period ended March 31, 2023. Such restated unaudited quarterly financial data and related impacted amounts were presented in the Company's 2023 Annual Report on Form 10-K. The following discussion gives effect to the restatement of our unaudited interim consolidated financial statements for the three and six months ended June 30, 2023. See the related discussion in Note 2, "Basis of presentation, principles of consolidation and significant accounting policies" of the Notes to Unaudited Consolidated Financial Statements included in Part I, Item 1 of this Quarterly Report on Form 10-Q.

Except for the historical information contained herein, the matters discussed in this Quarterly Report on Form 10-Q may be deemed to be forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. In this Quarterly Report on Form 10-Q, words such as "may," "expect," "anticipate," "estimate," "intend," "plan," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances) are intended to identify forward-looking statements.

Our actual results and the timing of certain events may differ materially from the results discussed, projected, anticipated, or indicated in any forward-looking statements. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this Quarterly Report on Form 10-Q. In addition, even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this Quarterly Report on Form 10-Q, they may not be predictive of results or developments in future periods.

The following information and any forward-looking statements should be considered in light of factors discussed elsewhere in this Quarterly Report on Form 10-Q, including those risks identified under Part II, Item 1A. Risk Factors.

We caution readers not to place undue reliance on any forward-looking statements made by us, which speak only as of the date they are made. We disclaim any obligation, except as specifically required by law and the rules of the SEC, to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.

Overview

We are a biotechnology company committed to researching, developing, and commercializing potentially curative gene therapies for severe genetic diseases based on our proprietary lentiviral vector ("LVV") gene addition platform. We currently market three gene therapies in the U.S.: ZYNTEGLO™ (betibeglogene autotemcel, also known as beti-cel), SKYSONA™ (elivaldogene autotemcel, also known as eli-cel), which were approved by the U.S. Food and Drug Administration (the "FDA") in 2022, and LYFGENIA™ (lovotibeglogene autotemcel, also known as lovo-cel), which received approval from the FDA in December 2023.

The FDA approved ZYNTEGLO for the treatment of adult and pediatric patients with ß-thalassemia who require regular red blood cell transfusions on August 17, 2022. The FDA granted accelerated approval of SKYSONA to slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active cerebral adrenoleukodystrophy ("CALD") on September 16, 2022. On December 8, 2023, LYFGENIA, was approved by the FDA for the treatment of patients 12 years of age or older with sickle cell disease ("SCD") and a history of vaso-occlusive-events.

We are focusing our development and commercialization efforts in the U.S. market. We have obtained the withdrawal of the marketing authorization for beti-cel and eli-cel in the European Union, which became effective in 2022 and 2021, respectively. We are continuing the long-term follow-up of patients previously enrolled within the clinical trial programs in Europe as planned but do not intend to initiate any new clinical trials in Europe for β-thalassemia, CALD or SCD.

Since our inception in 1992, we have devoted substantially all of our resources to our development and commercialization efforts relating to our products and product candidates, including activities to manufacture products and product candidates in compliance with good manufacturing practices (“GMP”) to conduct clinical studies of our product candidates, to provide selling, general and administrative support for these operations, to market, commercially manufacture and distribute our approved products and to protect our intellectual property. We have funded our operations primarily through the sale of common stock in our public offerings, issuance of warrants, the sale of two Rare Pediatric Disease Priority Review Vouchers ("PRVs"), debt financing agreements and through collaborations.

In August 2022 and September 2022 we received the two PRVs under an FDA program intended to encourage the development of treatments for rare pediatric diseases. In the fourth quarter of 2022, we sold our first PRV for aggregate net proceeds of $102.0 million. In the first quarter of 2023, we sold our second PRV for aggregate net proceeds of $92.9 million, inclusive of additional legal costs incurred.

In the first quarter of 2023, we sold 23.0 million shares of common stock (inclusive of shares sold pursuant to an option to the underwriters in connection with the offering) through an underwritten public offering at a price of $6.00 per share for aggregate net proceeds of $130.5 million, inclusive of additional offering costs incurred. In the fourth quarter of 2023, we sold 83.3 million shares of common stock through an underwritten public offering at a price of $1.50 per share for aggregate net proceeds of $118.1 million, after deducting for offering costs.

In March 2024, we entered into a five-year term loan facility agreement with Hercules Capital, Inc. to secure debt financing for up to $175.0 million, available in four tranches, based on amendments executed through August 2024.

In September 2024, our board of directors approved a restructuring action (the "Restructuring"), following a comprehensive review of our operations. The Restructuring includes a reduction of our workforce by 94 employees, or approximately 25% of employees.

As a result of the Restructuring, we estimate that we will incur aggregate charges of approximately $3.3 million to $3.7 million in cash expenditures for severance and employee termination-related costs to be paid out over multiple weeks through the end of the fiscal year ending December 31, 2024, as well as approximately $0.3 million to $0.5 million in non-cash stock-based compensation expense associated with accelerated vesting of RSUs. We expect to record a significant portion of these charges in the third quarter of 2024.

As of June 30, 2024, we had cash and cash equivalents of approximately $144.1 million. Absent the sale of our PRVs, we have never been profitable and have incurred net losses in each year since inception. Our net loss was $81.4 million and $151.2 million for the three and six months ended June 30, 2024, respectively, and our accumulated deficit was $4.4 billion as of June 30, 2024. Substantially all of our net losses resulted from costs incurred in connection with our research and development programs and from selling, general and administrative costs associated with our operations, and cost of product revenue. We expect to continue to incur significant expenses and operating losses for the foreseeable future, if and as we:

- fund activities related to the commercialization of ZYNTEGLO, SKYSONA, and LYFGENIA in the United States;
- scale our manufacturing capabilities in support of the commercialization of ZYNTEGLO, SKYSONA, and LYFGENIA;
- conduct clinical studies; and
- continue research and development-related activities for severe genetic diseases.

Because of the numerous risks and uncertainties associated with product development and commercialization, 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. We may not be able to generate substantial revenue from the sale of our products, and 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. Until we reach profitability, if ever, we expect to continue to seek to fund our operations through public or private equity or debt financings, strategic collaborations, 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 business.

Business update

We had cash and cash equivalents of approximately $144.1 million as of June 30, 2024. We will continue to generate operating losses and negative operating cash flows for the foreseeable future as we continue to commercialize ZYNTEGLO, SKYSONA and LYFGENIA and we will require the need for additional funding to support our planned operations before becoming profitable.

Based on our current forecasts, and assuming we implement planned cost-saving initiatives, we expect our existing cash and cash equivalents will enable us to fund our operations and maintain compliance with the minimum cash requirements of the Loan Agreement with Hercules Capital, Inc. into the first quarter of 2025. Not accounting for the minimum cash requirements of the Loan Agreement, we expect our existing cash and cash equivalents will enable us to fund our operations into the second quarter of 2025.

We have based this estimate on assumptions of revenues and operating costs that may prove to be wrong. Our cash runway estimate does not include use of our restricted cash, which as of June 30, 2024 was $49.2 million. The restricted cash was unavailable for use as of June 30, 2024, and we believe at least $48.0 million of this restricted cash is unlikely to be released in the near term. In addition, our future net product revenues will depend upon the demand for our products, the size of the markets, our ability to timely scale our manufacturing capabilities to meet market demand, our ability to achieve sufficient market acceptance, reimbursement from third-party payers, adequate market share in those markets and the performance of drug product subject to outcome-based programs. As a result, we could deplete our capital resources sooner than we currently expect. If, for any reason, our revenues or our expenses differ materially from our assumptions or we utilize our cash more quickly than anticipated, or if we are unable to obtain funding on a timely basis, we may be required to revise our business plan and strategy, which may result in bluebird failing to achieve profitability, significantly curtailing, delaying or discontinuing the commercialization of any products or may result in bluebird being unable to expand our operations or otherwise capitalize on our business opportunities. As a result, our business, financial condition, and results of operations could be materially affected.

The accompanying financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the ordinary course of business. The financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of the uncertainties described above.

Financial operations overview

Product revenue

Our revenues were derived from product revenues associated with the sale of SKYSONA and ZYNTEGLO in the United States.

Other revenue

We have recognized an immaterial amount of revenue associated with grants.

Cost of product revenue

Cost of product revenue includes costs associated with the sale of SKYSONA and ZYNTEGLO in the United States.

Selling, general and administrative expenses

Selling, 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, legal, business development, commercial, information technology, and human resource functions. Other selling, general and administrative expenses include facility-related costs, professional fees for accounting, tax, legal and consulting services, directors' fees and expenses associated with obtaining and maintaining patents. These expenses include lease expense related to 50 Binney Street and 100 Binney Street; however, sublease income is presented in other income, net.

We anticipate that our selling, general and administrative expenses, including payroll and sales and marketing expenses, may continue to increase in the future relative to current levels as we continue commercialization activities for ZYNTEGLO, SKYSONA, and LYFGENIA in the United States.

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 contract research organizations ("CROs") and clinical sites that conduct our clinical studies;
- expenses, including amortization of right-of-use assets when used in research and development, incurred under agreements with contract manufacturing organizations ("CMOs") related to pre-commercial manufacturing activities;
- facilities, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, information technology, insurance, and other supplies in support of research and development activities;
- costs associated with our research platform and preclinical activities;
- milestones and upfront license payments; and
- costs associated with our regulatory, quality assurance and quality control operations.

Research and development costs including those under executory contracts and variable costs related to arrangements that contain a lease are expensed as incurred. Right-of-use assets related to arrangements with CMOs and contract testing organizations ("CTOs") that contain a lease under ASC 842 but have no alternative future use under ASC 730 are immediately expensed to research and development expense at commencement or upon a modification until commercialization is achieved. 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 products or to what extent we will generate revenues from the commercialization and sale of our approved products. The duration, costs, and timing of clinical studies and development of our products will depend on a variety of factors, any of which could affect our research and development expenses, including:

- the scope, rate of progress, and expense of our ongoing as well as any additional clinical studies and other research and development activities we undertake;
- future clinical study results;
- uncertainties in clinical study enrollment rates;
- new manufacturing processes or protocols that we may choose to or be required to implement in the manufacture of our LVV or drug product;
- regulatory feedback on requirements for regulatory approval, as well as changing standards for regulatory approval; and
- the timing and receipt of any regulatory approvals.

We plan to continue to incur research and development expenses for the foreseeable future as we continue to conduct research activities for our platform technology. We expect our research and development expenses to decrease in conjunction with an increase in commercial activities and selling, general and administrative expense due to the approvals of ZYNTEGLO, SKYSONA, and LYFGENIA. Our research and development expenses include expenses associated with the following activities:

- the long-term follow-up protocol associated with the clinical studies of ZYNTEGLO, and a postmarketing study for the same;
- the long-term follow-up protocol associated with the clinical studies of SKYSONA, and a postmarketing study for the same;
- HGB-210, the long-term follow-up protocol associated with the clinical studies of LYFGENIA, and a postmarketing study for the same;
- research and development activities for our platform technology; and
- the manufacture of clinical study materials in support of our clinical studies.

Our direct research and development expenses consist principally of external costs, such as 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 allocate salary and benefit costs that are directly related to specific programs. We do not allocate personnel-related discretionary bonus or stock-based compensation costs, laboratory and related expenses, certain license and other collaboration costs, depreciation or other indirect costs that are deployed across multiple projects under development and, as such, the costs are separately classified as other research and development expenses in the table below:

	For the three months ended June 30,		For the six months ended June 30,	
	2024	2023	2024	2023
	(in thousands)		(in thousands)	
		(As Restated)		(As Restated)
LYFGENIA (lovo-cel)	$ 5,302	$ 8,540	$ 12,381	$ 26,142
SKYSONA (eli-cel)	2,986	89	3,453	3,782
ZYNTEGLO (beti-cel)	857	5,227	2,740	7,904
Preclinical programs	194	165	227	473
Total direct research and development expense	9,339	14,021	18,801	38,301
Employee-and contractor-related expenses	7,398	6,774	15,246	13,728
Stock-based compensation expense	1,119	3,083	2,055	6,010
License and other related expenses	—	24	—	111
Laboratory and other expenses	1,442	902	3,225	1,634
Facility expenses	5,864	6,644	10,909	13,251
Total other research and development expenses	15,823	17,427	31,435	34,734
Total research and development expense	$ 25,162	$ 31,448	$ 50,236	$ 73,035

Gain from sale of priority review voucher, net

Gain from sale of priority review voucher, net consists of gain from the sale of our priority review vouchers. In the first quarter of 2023, we sold our PRV for aggregate net proceeds of $92.9 million. We received the PRV in September 2022 under an FDA program intended to encourage the development of treatments for rare pediatric diseases.

Interest income

Interest income consists primarily of interest income earned on investments.

Interest expense

Interest expense consists primarily of interest expense associated with finance lease arrangements and long-term debt.

Other income, net

Other income, net consists primarily of sublease income, gains and losses on disposal of fixed assets, and gains and losses on foreign currency transactions.

Critical accounting policies and significant judgements 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 generally accepted accounting principles in the U.S. 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 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. 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. In making estimates and judgments, management employs critical accounting policies. During the six months ended June 30, 2024, there were no material changes to our critical accounting policies as reported in our

2023 Annual Report on Form 10-K, except as otherwise described in Note 2, *Basis of presentation, principles of consolidation and significant accounting policies,* in the Notes to Condensed Consolidated Financial Statements appearing elsewhere in this Quarterly Report on Form 10-Q.

Results of Operations

Comparison of the three months ended June 30, 2024 and 2023:

	For the three months ended June 30,		
	2024	2023	Change
	(in thousands)		
		(As Restated)	
Revenue:			
Product revenue, net	$ 16,101	$ 6,837	$ 9,264
Other revenue	—	53	(53)
Total revenues	16,101	6,890	9,211
Cost of product revenue	28,946	6,697	22,249
Gross margin	(12,845)	193	(13,038)
Operating expenses:			
Selling, general and administrative	50,385	40,462	9,923
Research and development	25,162	31,448	(6,286)
Total operating expenses	75,547	71,910	3,637
Income (loss) from operations	(88,392)	(71,717)	(16,675)
Interest income	2,837	2,679	158
Interest expense	(5,453)	(3,750)	(1,703)
Other income, net	9,636	9,919	(283)
Income (loss) before income taxes	(81,372)	(62,869)	(18,503)
Income tax (expense) benefit	(21)	80	(101)
Net income (loss)	$ (81,393)	$ (62,789)	$ (18,604)

Revenues. Total revenue was $16.1 million for the three months ended June 30, 2024, compared to $6.9 million for the three months ended June 30, 2023. The increase of $9.2 million is primarily attributable to increased product sales during 2024.

Cost of product revenue. Cost of product revenue was $28.9 million for the three months ended June 30, 2024, compared to $6.7 million for the three months ended June 30, 2023. The increase is primarily attributable to increased product sales during 2024 and increased inventoriable expenses associated with contract manufacturing costs.

Selling, general and administrative expenses. Selling, general and administrative expenses were $50.4 million for the three months ended June 30, 2024, compared to $40.5 million for the three months ended June 30, 2023. The net increase of $9.9 million was primarily attributable to the following:

- $8.2 million of increased professional fees driven by increased legal and accounting advisory costs; and
- $3.0 million of increased net employee compensation, benefit, and other headcount-related expenses, driven by increased headcount in selling, general and administrative costs in 2024 with an offset of $0.9 million decrease in stock-based compensation expense.

Research and development expenses. Research and development expenses were $25.2 million for the three months ended June 30, 2024, compared to $31.4 million for the three months ended June 30, 2023. The net decrease of $6.3 million was primarily attributable to the following:

- $3.8 million of decreased net employee compensation, benefit, and other headcount related expenses, including a decrease of $2.0 million in stock-based compensation expense, driven by related expenses being included in inventory and cost of product revenue for our commercial products;
- $2.0 million of decreased consulting fees; and

- $1.2 million of decreased information technology and facility-related costs primarily driven by information technology and facility-related expenses now being included in inventory and cost of product revenue.

These decreased costs were partially offset by the following:

- $1.3 million of increased costs related to manufacturing expense.

Interest income. The increase in interest income is primarily related to higher interest income earned related to our restricted cash and higher interest rates on cash and cash equivalents in operating accounts, offset by an overall decrease in total cash balances in 2024 compared to 2023.

Interest expense. The increase in interest expense is primarily due to interest expense associated with finance lease arrangements and our term loan debt with Hercules that we entered into in March 2024.

Other income, net. The decrease in other income, net is primarily related to gains and losses on foreign currency transactions.

Comparison of the six months ended June 30, 2024 and 2023:

	For the six months ended June 30,		
	2024	2023	Change
	(in thousands)		
		(As Restated)	
Revenue:			
Product revenue, net	$ 34,662	$ 9,133	$ 25,529
Other revenue	12	138	(126)
Total revenues	34,674	9,271	25,403
Cost of product revenue	54,810	12,209	42,601
Gross margin	(20,136)	(2,938)	(17,198)
Operating expenses:			
Selling, general and administrative	96,714	77,929	18,785
Research and development	50,236	73,035	(22,799)
Total operating expenses	146,950	150,964	(4,014)
Gain from sale of priority review voucher, net	—	92,930	(92,930)
Income (loss) from operations	(167,086)	(60,972)	(106,114)
Interest income	5,416	5,507	(91)
Interest expense	(10,308)	(8,020)	(2,288)
Other income, net	20,802	19,546	1,256
Income (loss) before income taxes	(151,176)	(43,939)	(107,237)
Income tax (expense) benefit	(21)	80	(101)
Net income (loss)	$ (151,197)	$ (43,859)	$ (107,338)

Revenues. Total revenue was $34.7 million for the six months ended June 30, 2024, compared to $9.3 million for the six months ended June 30, 2023. The increase of $25.4 million is primarily attributable to increased product sales during 2024.

Cost of product revenue. Cost of product revenue was $54.8 million for the six months ended June 30, 2024, compared to $12.2 million for the six months ended June 30, 2023. The increase is primarily attributable to increased product sales during 2024 and increased inventoriable expenses associated with contract manufacturing costs.

Selling, general and administrative expenses. Selling, general and administrative expenses were $96.7 million for the six months ended June 30, 2024, compared to $77.9 million for the six months ended June 30, 2023. The net increase of $18.8 million was primarily attributable to the following:

- $12.0 million of increased professional fees driven by increased legal and accounting advisory costs; and

- $8.1 million of increased net employee compensation, benefit, and other headcount-related expenses, driven by increased headcount in selling, general and administrative costs in 2024 with an offset of $0.9 million decrease in stock-based compensation expense.

Research and development expenses. Research and development expenses were $50.2 million for the six months ended June 30, 2024, compared to $73.0 million for the six months ended June 30, 2023. The net decrease of $22.8 million was primarily attributable to the following:

- $8.2 million of decreased net employee compensation, benefit, and other headcount related expenses, including a decrease of $4.0 million in stock-based compensation expense, driven by related expenses being included in inventory and cost of product revenue for our commercial products;
- $4.6 million of decreased consulting fees;
- $3.7 million of decreased manufacturing costs primarily driven by material production for all commercial products now being included in inventory and cost of product revenue;
- $3.5 million of decreased information technology and facility-related costs primarily driven by information technology and facility-related expenses now being included in inventory and cost of product revenue; and
- $3.4 million of decreased clinical subject treatment costs.

These decreased costs were partially offset by the following:

- $2.2 million of increased costs related to lab expenses driven by an increase in lab consumables

Gain from sale of priority review voucher, net. The decrease in gain from sale of priority review voucher, net was related to the sale of a priority review voucher in the first quarter of 2023.

Interest income. The decrease in interest income was primarily related to an overall decrease in total cash balances in the first two quarters of 2024 compared to the first two quarters of 2023.

Interest expense. The increase in interest expense is primarily due to interest expense associated with finance lease arrangements and term loan debt.

Other income, net. The increase in other income, net is primarily related to gains and losses on foreign currency transactions.

Liquidity and Capital Resources

As of June 30, 2024, we had cash and cash equivalents of approximately $144.1 million. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation. As of June 30, 2024, our funds are primarily held in U.S. government agency securities and treasuries, and money market accounts with maturities at date of purchase of 90 days or less.

We have incurred losses and cumulative negative cash flows from operations since our inception in April 1992, and as of June 30, 2024, we had an accumulated deficit of $4.4 billion. We expect our research and development expenses to decrease in conjunction with an increase in commercial activities and selling, general and administrative expense due to the commercialization of ZYNTEGLO, SKYSONA, and LYFGENIA.

In September 2024, we implemented the Restructuring designed to support our commercial focus and reduce our cash operating expenses. See also "Management's Discussion and Analysis - Overview".

Based on our current forecasts, and assuming we implement planned cost-saving initiatives, we expect our existing cash and cash equivalents will enable us to fund our operations and maintain compliance with the minimum cash requirements of the Loan Agreement with Hercules Capital, Inc. into the first quarter of 2025. Not accounting for the minimum cash requirements of the Loan Agreement, we expect our existing cash and cash equivalents will enable us to fund our operations into the second quarter of 2025.

We have based this estimate on assumptions of revenues and operating costs that may prove to be wrong. Our cash runway estimate does not include use of our restricted cash of $49.2 million, which was unavailable for use as of June 30, 2024, and we

believe at least $48.0 million of this restricted cash is unlikely to be released in the near term. In addition, our future net product revenues will depend upon the demand for our products, the size of the markets, our ability to timely scale our manufacturing capabilities to meet market demand, our ability to achieve sufficient market acceptance, reimbursement from third-party payers, adequate market share in those markets and the performance of drug product subject to outcome-based programs. As a result, we could deplete our capital resources sooner than we currently expect. If, for any reason, our revenues or our expenses differ materially from our assumptions or we utilize our cash more quickly than anticipated, or if we are unable to obtain funding on a timely basis we may be required to revise our business plan and strategy, which may result in bluebird failing to achieve profitability, significantly curtailing, delaying or discontinuing one or more of our research or development programs or the commercialization of any products or may result in bluebird being unable to expand our operations or otherwise capitalize on our business opportunities. As a result, our business, financial condition, and results of operations could be materially affected.

We have funded our operations principally from the sale of common stock in public offerings, the Loan Agreement, and the sale of the two PRVs. The following is a summary of recent financing transactions:

- In the first quarter of 2023, we sold our second PRV for aggregate net proceeds of $92.9 million.
- In the first quarter of 2023, we sold 23.0 million shares of common stock (inclusive of shares sold pursuant to an option to the underwriters in connection with the offering) in an underwritten public offering at a price of $6.00 per share for aggregate net proceeds of $130.5 million.
- In August 2023, we entered into an Open Market Sales Agreement (the "Sales Agreement") with Jefferies LLC ("Jefferies") to sell shares of our common stock up to $125.0 million, from time to time, through an “at the market” equity offering program under which Jefferies will act as sales agent. As of June 30, 2024, we have made no sales pursuant to the Sales Agreement.
- In December 2023, we sold 83.3 million shares of common stock in an underwritten public offering at a price of $1.50 per share for aggregate net proceeds of $118.1 million.
- In March 2024, we entered into the Loan Agreement for up to $175.0 million in debt financing.

Sources of Liquidity

Cash Flows

The following table summarizes our cash flow activity:

	For the six months ended June 30,	
	2024	**2023**
	(in thousands)	
		(As Restated)
Net cash (used in) operating activities	$ (140,928)	$ (130,859)
Net cash provided by investing activities	966	89,081
Net cash provided by financing activities	58,584	101,507
Net (decrease) increase in cash, cash equivalents and restricted cash	$ (81,378)	$ 59,729

Operating Activities. The $10.1 million increase in cash used in operating activities for the six months ended June 30, 2024 compared to the six months ended June 30, 2023 was primarily due to the increase in net loss of $107.3 million, the increase in net working capital of $7.5 million, a change in non-cash items including excess inventory reserves of $0.6 million, offset by no adjustments for non-cash items relating to the gain from the sale of priority review voucher of $92.9 million, which was sold in 2023, and by non-cash items including change in depreciation and amortization expense of $19.7 million.

Investing Activities. The $88.1 million decrease in cash provided by investing activities for the six months ended June 30, 2024 compared to the six months ended June 30, 2023 was primarily due to no proceeds from sale of priority review voucher, compared to $92.9 million in proceeds from sale of priority review voucher during the six months ended June 30, 2023.

Financing Activities. The $42.9 million decrease in cash provided by financing activities for the six months ended June 30, 2024 compared to the six months ended June 30, 2023 was primarily due to no proceeds from the secondary public offering, compared to $130.1 million in proceeds from the secondary public offering, net of paid offering costs, issued during the six months ended June 30, 2023, the increase in principal payments on finance lease of $20.1 million, offset by the proceeds from

the issuance of debt of $71.3 million and the proceeds from factoring arrangement of $35.8 million during the six months ended June 30, 2024.

Contractual Obligations and Commitments

Except as discussed in Note 9, *Leases*, and Note 10, *Commitments and contingencies*, in the Notes to Condensed Consolidated Financial Statements appearing elsewhere in this Quarterly Report on Form 10-Q, there have been no material changes to our contractual obligations and commitments as included in our 2023 Annual Report on Form 10-K.

Item 3. Quantitative and Qualitative Disclosures About Market Risks

We are exposed to market risk related to changes in interest rates. As of June 30, 2024 and December 31, 2023, we had cash and cash equivalents of $144.1 million and $221.8 million, respectively, primarily invested in U.S. government agency securities and treasuries, corporate bonds, commercial paper, equity securities, and money market accounts. 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.

Item 4. Controls and Procedures

Limitations on Effectiveness of Controls and Procedures

In designing and evaluating our disclosure controls and procedures, our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives. In addition, the design of disclosure controls and procedures must reflect the fact that there are resource constraints and that management is required to apply judgment in evaluating the benefits of possible controls and procedures relative to their costs.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and principal financial officer, has evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13(a)- 15(e) and 15(d)- 15(e) under the Securities Exchange Act of 1934, as amended (the “Exchange Act”)), as of June 30, 2024, the end of the period covered by this Quarterly Report on Form 10-Q. Based on such evaluation, our principal executive officer and principal financial officer have concluded that as of such date, our disclosure controls and procedures were not effective at the reasonable assurance level due to the material weakness in our internal control over financial reporting described below.

In connection with the Company’s preparation of its financial statements and the restatement (as further described within Note 2, Restatement of Previously Issued Financial Statements to the consolidated financial statements appearing in the Annual Report on Form 10-K filed September 13, 2024), the Company identified a material weakness in its internal controls over financial reporting, which failed to prevent or detect the identified misstatements requiring restatement.

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the Company’s annual or interim financial statements will not be prevented or detected on a timely basis.

Our management concluded that a material weakness existed as of December 31, 2023, and in prior periods, with respect to the design and operating effectiveness of the Company’s controls over the accounting for arrangements that contain a lease. Specifically, the Company did not: (i) design controls to properly apply the Company's accounting policy to combine lease and non-lease components in lease arrangements, including embedded leases, (ii) operate controls to review the identification of leases and lease elements and accounting for lease arrangements, including embedded leases and lease modifications, by individuals with appropriate knowledge and competency, and (iii) operate controls to review the accounting for embedded leases with contract manufacturing organizations and contract testing organizations by individuals with appropriate knowledge and competency to determine the appropriate lease classification, presentation and commencement date.

This material weakness resulted in the restatement of the Company’s consolidated financial statements as of and for the year ended December 31, 2022, and the unaudited condensed consolidated financial information for each of the first three quarters of 2023 and 2022. Additionally, the material weakness could result in misstatements to the Company’s accounts and

disclosures that would result in a material misstatement of the annual or interim consolidated financial statements that would not be prevented or detected.

Remediation Plan

Our management is committed to maintaining a strong internal control environment. In response to the identified material weakness above, management intends to take comprehensive actions to remediate the material weakness in internal control over financial reporting, including:

- reassess and enhance the design of existing internal controls over lease accounting and design and implement new or modified internal controls to ensure that financial statement assertion level risks (e.g. valuation, completeness, accuracy, presentation and disclosure) related to leases are addressed;

- strengthen the lease accounting technical knowledge and experience within the Company's accounting function to enhance the oversight of the processes related to accounting for leases and arrangements that could contain embedded leases or lease modifications;

- conduct training for individuals responsible for performing and reviewing the accounting and presentation for leases and arrangements with contract manufacturing and contract testing organizations which could contain embedded leases or modifications.

The remediation plan, when finalized, is expected to include a number of enhanced activities that reflect a continuation of activities the Company has started to undertake during the 2023 financial close process. We believe that the actions outlined above, when fully implemented, will remediate the material weakness. The material weakness will not be considered remediated, however, until the applicable controls operate for a sufficient period of time and management has concluded, through testing, that these controls are operating effectively. We may also conclude that additional measures may be required to remediate the material weakness in our internal control over financial reporting, which may necessitate additional implementation and evaluation time. We will continue to assess the effectiveness of our internal control over financial reporting and take steps to remediate the material weakness expeditiously.

Changes in Internal Control over Financial Reporting

Other than the changes associated with the material weakness and corresponding remediation procedures described above, there have been no changes in our internal control over financial reporting, as such term is defined in Rules 13(a)-15(f) and 15(d)-15(f) promulgated under the Securities Exchange Act of 1934, during the second quarter of 2024 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

In the ordinary course of business, we are from time to time involved in lawsuits, claims, investigations, proceedings, and threats of litigation relating to intellectual property, commercial arrangements, employment and other matters. The outcome of these proceedings and claims cannot be predicted with certainty. We believe no governmental proceedings are pending or, to our knowledge, contemplated against us. We are not a party to any material proceedings in which any director, member of senior management or affiliate of ours is either a party adverse to us or our subsidiaries or has a material interest adverse to us or our subsidiaries.

On October 21, 2021, San Rocco Therapeutics, LLC, formerly known as Errant Gene Therapeutics, LLC, filed a complaint against us in the United States District Court for the District of Delaware for alleged infringement of U.S. Patent Nos. 7,541,179 and 8,058,061. The term of U.S. Patent No. 8,058,061 already expired on November 25, 2022, and U.S. Patent No. 7,541,179 will expire on May 13, 2024. The allegations relate to our use of the BB305 lentiviral vector, including in connection with the beti-cel program and seeks injunctive relief and money damages. On February 21, 2022, the parties stipulated to amend the case caption, in light of the plaintiff's name change, from Errant Gene Therapeutics, LLC to San Rocco Therapeutics, LLC ("SRT"). The Court granted this stipulation and, accordingly, the case is now captioned, San Rocco Therapeutics, LLC v. bluebird bio, Inc. and Third Rock Ventures, LLC, C.A. No. 21-1478-RGA. On April 6, 2022, we—along with Third Rock Ventures, LLC—filed a motion seeking various relief including to stay the proceedings and compel arbitration on two threshold issues, which we argued warranted complete dismissal of the action as a matter of law, regardless of the merits of SRT's underlying infringement claims. On July 26, 2022, the Court granted our request to stay the proceedings and issued an Order compelling the parties to arbitrate the threshold issues we raised. On February 7, 2023, the Arbitrator issued a final award finding in favor of SRT on both threshold issues, thereby enabling SRT to pursue its claims for alleged infringement. On March 1, 2023, the parties jointly stipulated, subject to the approval of the United States District Court for the District of Delaware, to lift the stay. The Court lifted the stay on March 2, 2023, and on March 31, 2023, we filed our answer to SRT's complaint with counterclaims asserting that we do not infringe the patents-in-suit and that the patents-in-suit are invalid. Also, on April 22, 2024, the Patent Trial & Appeal Board of the U.S. PTO found that our two petitions for inter partes review did not show by a preponderance of the evidence that the challenged claims of the patents-in-suit are unpatentable, and we filed notices of appeal with the U.S. Court of Appeals for the Federal Circuit on June 21, 2024. Our opening brief is currently due on December 6, 2024, SRT's responsive brief is due January 15, 2025, and our reply brief is due February 5, 2025. On June 17, 2024, the Court entered a claim construction order in bluebird's favor. On July 17, 2024, the Court granted our request for leave to file a case-dispositive motion for summary judgment of noninfringement, and on July 25, 2024, the Court ordered a stay of discovery pending a decision on the summary judgment motion. On August 1, 2024, we filed our motion for summary judgment of noninfringement, SRT filed an opposition on September 3, 2024 and we filed our reply on September 17, 2024. Briefing is now complete, and we have requested oral argument. We plan to vigorously defend against SRT's claims in this action.

On April 27, 2023, SRT filed another complaint against us (as well as against Mr. Nick Leschly, Mr. Mitchell Finer, Mr. Philip Reilly, Third Rock Ventures LLC, and 2Seventy Bio, Inc.) in the United States District Court for the District of Massachusetts. This complaint alleges civil violations of the Federal Racketeer Influenced and Corrupt Organizations Act, violations of Mass. Gen. Laws ch. 93A, § 11, and fraudulent inducement of SRT into a release provision in a November 2020 confidential settlement agreement we executed with, inter alia, SRT. The allegations relate to our use of the BB305 lentiviral vector, including in connection with the beti-cel program, and SRT seeks declaratory relief and money damages. On July 3, 2023, we (in conjunction with the other defendants) moved to dismiss all claims with prejudice brought by SRT in its Complaint for failure to state a claim upon which relief may be granted. On August 7, 2023, SRT filed an amended complaint, adding Craig Thompson as a defendant, and adding additional claims of alleged antitrust violations under federal and state law. The case is now captioned San Rocco Therapeutics, LLC v. Nick Leschly, Mitchell Finer, Philip Reilly, Craig Thompson, Third Rock Ventures LLC, bluebird bio, Inc. and 2Seventy Bio, Inc., C.A. No. 1:23-cv-10919-ADB. On September 18, 2023, we (in conjunction with the non-Thompson defendants), moved to dismiss with prejudice once again. SRT filed an opposition to that motion on October 12, 2023. On October 24, 2023, we filed a motion for leave to file a reply brief, which was granted on October 30, 2023. SRT filed a sur-reply brief on November 2, 2023. The motion to dismiss remains pending and we plan to vigorously defend against SRT's claims in this action.

On April 15, 2024, SRT filed a Demand for Arbitration with the American Arbitration Association, accusing us of breaching a November 2020 confidential settlement agreement by initiating a proceeding before the Patent Trial & Appeal Board (PTAB) of the United States Patent & Trademark Office in October 2022, asserting invalidity of two patents licensed to SRT. SRT seeks reimbursement of its costs and fees, including attorney's fees, incurred in the PTAB proceeding, totaling approximately $1.5 million. On August 26, 2024, the parties submitted their respective opening dispositive briefs. We filed our

response on September 24, 2024, when SRT also filed its opposition to our dispositive motion. The parties' respective replies are due on October 8, 2024. A hearing on the parties' respective dispositive motions is scheduled for October 22, 2024, to be conducted virtually. A final hearing on the merits, if needed, is scheduled for March 25 - 26, 2025, in New York City. We believe SRT's breach claim has no merit and intend to vigorously defend against it.

On March 28, 2024, a class action lawsuit captioned Garry Gill v. bluebird bio, Inc. et al., Case No. 1:24-cv-10803-PBS, was filed against us in the United States District Court for the District of Massachusetts. An amended complaint was filed on August 15, 2024. The amended complaint purports to assert claims against us and certain of our current and former officers pursuant to Sections 10(b) and 20(a) of the Securities Exchange Act of 1934, as amended, and Rule 10b-5 promulgated thereunder, on behalf of a putative class of investors who purchased or otherwise acquired the Company's shares between April 24, 2023 and December 8, 2023 (the "class period"). Plaintiff seeks to recover damages allegedly caused by purported misstatements and omissions regarding (i) whether the Company could obtain FDA approval for the lovo-cel BLA without a black box warning for hematologic malignancies; and (ii) whether the Company would be granted a priority review voucher by the FDA in connection with the BLA, which it could sell in order to strengthen its financial position. The amended complaint claims these alleged statements and omissions operated to artificially inflate the price paid for our common stock during the class period. On September 2, 2024, the Court entered the parties' stipulated schedule for briefing a motion to dismiss the amended complaint: the opening brief in support of a motion to dismiss is due October 11, 2024; the opposition brief is due December 5, 2024; and a reply brief in further support of a motion to dismiss is due December 20, 2024. We intend to vigorously defend against the claims in this action.

On June 27, 2024, a shareholder derivative lawsuit captioned *Šimaitis v. Obenshain et al.*, Case No. 1:24-cv-11674-PBS, was filed nominally on our behalf against certain current and former members of Company management and the Board of Directors in the United States District Court for the District of Massachusetts. The complaint purports to assert derivative claims pursuant to Sections 10(b), 14(a), and 21D of the Securities Exchange Act of 1934, as well as for breach of fiduciary duties, unjust enrichment, waste of corporate assets, gross mismanagement, and abuse of control. Plaintiff seeks to recover damages on our behalf allegedly caused by purported materially false and misleading public statements and omissions, including in the April 28, 2023 proxy statement, regarding (i) whether the Company could obtain FDA approval for the lovo-cel BLA without a black box warning for hematologic malignancies; and (ii) whether the Company would be granted a priority review voucher by the FDA in connection with the BLA, which it could sell in order to strengthen its financial position. The complaint claims these allegedly misleading statements and omissions operated to artificially inflate the Company's common stock price during the relevant time period. To support its derivative claims, the complaint alleges that a legally required pre-suit demand on the Board would be futile and should be excused. On July 25, 2024, the case was consolidated with *Syracuse v. Obenshain et al.*, Case No. 1:24-cv-11752 (D. Mass. July 8, 2024). The consolidated actions were recaptioned *In re bluebird bio, Inc. Stockholder Derivative Litigation*, Case No. 1:24-cv-11674-PBS.

On July 8, 2024, a shareholder derivative lawsuit captioned *Syracuse v. Obenshain et al.*, Case No. 1:24-cv-11752-PBS, was filed nominally on our behalf against certain current and former members of Company management and the Board of Directors in the United States District Court for the District of Massachusetts. The complaint purports to assert derivative claims against pursuant to Section 14(a) of the Securities Exchange Act of 1934, as well as for breach of fiduciary duties, gross mismanagement, waste of corporate assets, and unjust enrichment. Plaintiff seeks to recover damages on our behalf allegedly caused by purported materially false and misleading public statements and omissions, including in the April 28, 2023 proxy statement, regarding (i) whether the Company could obtain FDA approval for the lovo-cel BLA without a black box warning for hematologic malignancies; and (ii) whether the Company would be granted a priority review voucher by the FDA in connection with the BLA, which it could sell in order to strengthen its financial position. The complaint claims these allegedly misleading statements and omissions operated to artificially inflate the Company's common stock price during the relevant time period. To support its derivative claims, the complaint alleges that a legally required pre-suit demand on the Board would be futile and should be excused. On July 25, 2024, the case was consolidated with *Šimaitis v. Obenshain et al.*, Case No. 24-cv-11674 (D. Mass. July 27, 2024). The consolidated actions were recaptioned *In re bluebird bio, Inc. Stockholder Derivative Litigation*, Case No. 1:24-cv-11674-PBS.

Item 1A. 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 Quarterly Report on Form 10-Q, including our financial statements and related notes hereto, 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.

We have incurred significant losses since our inception and we may not achieve our goal of becoming profitable in the timeframe we expect, or at all.

We have incurred significant net losses since our inception in 1992, including net losses from continuing operations of $211.9 million for the year ended December 31, 2023. As of December 31, 2023, we had an accumulated deficit of $4.3 billion. To date, we have devoted significant financial resources to building our commercial infrastructure and research and development, including our clinical and preclinical development activities. We will continue to incur net losses for the foreseeable future and we may not become profitable on the timeline we anticipate, or at all. To date, we have financed our operations primarily through our loan agreement with Hercules Capital, Inc., the sale of equity securities and priority review vouchers, and, to a lesser extent, through collaboration agreements and grants from governmental agencies and charitable foundations. We did not generate material revenues from the sale of ZYNTEGLO in the European Union and are just beginning to recognize revenue from our approved products in the U.S. given the treatment cycle time, in which revenue is recognized upon infusion. Our future revenues will depend upon the size of any markets in which our products have received approval, and our ability to achieve sufficient market acceptance, reimbursement from third-party payers and adequate market share for our products in those markets.

We anticipate that our expenses may increase substantially, we may continue to incur operating losses, and we may not generate profit if and as we:

- grow our capabilities to support our commercialization efforts for ZYNTEGLO, SKYSONA and LYFGENIA, including continuing to establish a sales, marketing and distribution infrastructure in the United States;
- obtain, build and expand manufacturing capacity, including capacity at third-party manufacturers;
- attract and retain skilled personnel;
- initiate additional research, preclinical, clinical or other programs as we seek to identify and validate additional product candidates;
- continue our ongoing and planned clinical development of ZYNTEGLO, SKYSONA and LYFGENIA, including completion of the HGB-210 clinical trial and long-term follow-up studies;
- acquire or in-license other product candidates and technologies;
- maintain, protect and expand our intellectual property portfolio;
- incur expenses for legal, accounting, and other professional services in connection with the restatement of our consolidated financial statements;
- defend against lawsuits, including patent or stockholder litigation; 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. Further, there is no assurance that we will ever achieve profitability. In addition, 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.

There is substantial doubt regarding our ability to continue as a going concern. 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 commercial programs, product development efforts or other operations.

Based on our current business plan as of the date hereof, management has concluded that there is substantial doubt regarding our ability to continue as a going concern. See Part I, Item 2 “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Liquidity and Capital Resources” of this Quarterly Report on Form 10-Q for a discussion

of our expected cash runway. Accordingly, we will need to raise additional funding in order to execute on our current business plans and strategy, including prior to becoming profitable or generating free cash flow.

We cannot guarantee that 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, traditional debt or other debt-like arrangements, 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 ability to conduct our business. See also "Risk Factors – *"Our existing and any future indebtedness could adversely affect our ability to operate our business"*. We could also be required to seek funds through arrangements with collaborative partners or otherwise, which may require us to relinquish rights to some of our technologies or products or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects. In addition, our efforts to raise additional funding may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our products.

Furthermore, as a result of the restatement of our consolidated financial statements for the year ended December 31, 2022 and the quarterly periods in the years ended December 31, 2022 and 2023, we were delayed in filing our Annual Report on Form 10-K and this Quarterly Report on Form 10-Q and we have not yet filed our Quarterly Report on Form 10-Q for the period ended June 30, 2024. As a result, we will not be eligible to sell securities under our existing shelf registration statement on Form S-3 or file a new Form S-3 until we have filed in a timely manner all required reports in accordance with the requirements of Form S-3. See "Risk Factor — *The restatement of our consolidated financial statements for the year ended December 31, 2022 and the quarterly periods in the years ended December 31, 2022 and 2023 has subjected us to a number of additional risks and uncertainties, including increased possibility of legal proceedings*". Our inability to use Form S-3 could make it more difficult and costly for us to obtain funding through a sale of securities.

Moreover, as a result of recent volatile market conditions, the cost and availability of capital has been and may continue to be adversely affected. Lenders and institutional investors may reduce, and in some cases, cease to provide credit to businesses and consumers. Continued turbulence in the U.S. market and economy may adversely affect our liquidity and financial condition, including our ability to access the capital markets to meet liquidity needs. In addition, we maintain the majority of our cash and cash equivalents in accounts with major financial institutions, and our deposits at these institutions exceed insured limits. Market conditions can impact the viability of these institutions. In the event of failure of any of the financial institutions where we maintain our cash and cash equivalents, there can be no assurance that we would be able to access uninsured funds in a timely manner or at all. Any inability to access or delay in accessing these funds could adversely affect our business and financial position.

If we are unable to obtain funding on a timely basis, or if revenues from collaboration arrangements or product sales are less than we have projected, we may be required to further revise our business plan and strategy, which may result in us significantly curtailing, delaying or discontinuing the commercialization of any current or future products or may result in our being unable to expand our operations or otherwise capitalize on our business opportunities. As a result, our business, financial condition and results of operations could be materially affected.

Among other potential adverse events, insertional oncogenesis is a significant risk of gene therapies using viral vectors that can integrate into the genome. Any such adverse events may require us to halt or delay further clinical development of our products or any future product candidates or to suspend or cease commercialization, and the commercial potential of our products and any such future product candidates may be materially and negatively impacted.

Adverse events or other undesirable side effects caused by our products or any future product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay, denial or withdrawal of regulatory approval by the FDA or other comparable foreign regulatory authorities. A potentially significant risk in any gene therapy product using viral vectors that can integrate into the genome is that the vector will insert in or near cancer-causing genes, leading to the proliferation of certain cellular clones that could cause cancer in the patient, known as insertional oncogenesis. For instance, multiple patients with CALD treated with eli-cel (now SKYSONA) in our clinical studies have been diagnosed with myelodysplastic syndrome ("MDS") or acute myeloid leukemia ("AML"), likely mediated by Lenti-D LVV insertion. SKYSONA's label includes a boxed warning for the known risk of hematologic malignancy and, accordingly, we expect additional cases to arise over time. In April 2024, the boxed warning was revised to include updated information on hematologic malignancies diagnosed in our clinical study patients, as well as other updates to monitoring procedures and alternative treatment options. We continue to closely monitor potential cases of hematologic malignancy in patients treated with

SKYSONA and we are communicating regularly with treating physicians and regulatory authorities. We cannot make assurances that additional patients treated with SKYSONA, ZYNTEGLO or LYFGENIA in the clinical or commercial setting will not be diagnosed with hematologic malignancy.

Moreover, in December 2021, the FDA placed the lovo-cel clinical development program under a partial clinical hold for patients under the age of 18. The hold related to a case of persistent anemia in an adolescent patient with two α-globin gene deletions (−α3.7/−α3.7), also known as alpha-thalassemia trait, who was treated with lovo-cel. In December 2022, the FDA lifted its partial clinical hold for patients under the age of 18 in studies evaluating lovo-cel for SCD. Notwithstanding the lifting of this partial clinical hold, additional adverse events or new data or analyses regarding previously reported events may indicate significant safety issues, and the FDA could potentially impose or reimpose a clinical hold in the future on studies evaluating lovo-cel. Moreover, laboratory results following gene therapy can be difficult to interpret, resulting in different or changing diagnoses by treating physicians. For instance, on January 31, 2023, we received a physician diagnosis of MDS in a patient treated with lovo-cel, in response to lab results obtained through routine monitoring of the same adolescent patient with two α-globin gene deletions subject to the partial clinical hold noted above. Consistent with established safety protocols, the information was reviewed by an independent Data Monitoring Committee which concluded that available evidence did not support a diagnosis of MDS and additional data would be needed to confirm such diagnosis, and that lovo-cel clinical studies should continue. Test results received since the investigator's initial report (including integration site analysis) demonstrated no evidence of insertional oncogenesis and as of August 27, 2024, the patient remained clinically stable with stable laboratory results and was not undergoing treatment for an MDS diagnosis. Study investigators and the FDA were informed and we will continue to monitor additional analyses as further test results are received.

Furthermore, treatment with our products and any future product candidates involves or may involve chemotherapy or myeloablative treatments, which can cause side effects or adverse events that may impact the perception of the potential benefits of our products and any future product candidates. For instance, MDS leading to AML is a known risk of certain myeloablative regimens. Accordingly, it is possible that the events of MDS and AML previously reported in our HGB-206 clinical study of lovo-cel in SCD were caused by underlying SCD, transplant procedure, and stress on the bone marrow following drug product infusion in connection with the lovo-cel treatment. The product label for LYFGENIA includes a boxed warning for the known risk of hematologic malignancy. Additionally, the procedures associated with the administration or collection of cells for ZYNTEGLO, SKYSONA, or LYFGENIA, could potentially cause other adverse events that have not yet been predicted. The inclusion of patients with significant underlying medical problems 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, or the progression of their disease.

Moreover, patients treated with our therapies, including lovo-cel, have exhibited persistent oligoclonality, which we define as two consecutive instances of (i) any LVV insertion site observed at >=10% relative frequency, or (ii) two or more insertion sites observed at >= to 5% relative frequency, as measured by integration site analysis. Based on our clinical protocols, we increase monitoring of patients who exhibit persistent oligoclonality. It is not clear at this time whether persistent oligoclonality represents an increased risk of developing hematologic malignancy in the future, but it is a criterion used by the FDA to evaluate the safety of gene therapies over time.

Additionally, there is the potential risk of other 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 LVVs possess characteristics that may pose high risks of delayed adverse events.

If any such adverse events occur, including insertional oncogenesis, further advancement of our ongoing and future clinical studies and other development efforts could be halted or delayed, and we may be unable to commercialize our approved products in the manner we expect, or at all. It is possible that upon occurrence or recurrence of any of these events, the FDA may place one or more of our programs on hold, impose requirements that result in delays for regulatory approvals for our products or any future product candidates, require the implementation of risk evaluation or mitigation strategies, or may cause us to cease commercialization of our approved products. If any of these were to occur, the commercial potential of our programs may be materially and negatively impacted.

Although ZYNTEGLO, SKYSONA and LYFGENIA have been approved by the FDA, serious safety events may result in an approved product being removed from the market or its market opportunity being significantly reduced. For instance, it is possible that as we commercialize our products, conduct long-term follow-up, or test any future product candidates in larger, longer and more extensive clinical trials, or as use of these products or any future products becomes more widespread, illnesses, injuries, discomforts and other adverse events that were observed in previous trials, as well as conditions that did not occur or went undetected in previous trials, will be reported by patients. Many times, side effects (that may or may not be related to our

products or any future product candidate) are only detectable after investigational products are tested in large-scale clinical trials or, in some cases, after they are made available to patients on a commercial scale following approval. Other patients receiving our products may develop hematologic malignancies in the future, which may negatively impact the commercial prospects of our products and any future product candidates. We or others may later identify undesirable side effects or adverse events caused by such products, or side effects or adverse events could accumulate over time, and a number of potentially significant negative consequences could result, including but not limited to:

- regulatory authorities may suspend, limit or withdraw approvals of such product, or seek an injunction against its manufacture or distribution;
- regulatory authorities may require additional warnings on the label, including “boxed” warnings, or issue safety alerts, "Dear Healthcare Provider" or "Dear Doctor" letters, press releases or other communications containing warnings or other safety information about the product;
- we may be required to change the way the product is administered or conduct additional clinical trials or post-marketing studies;
- we may be required to create a risk evaluation and mitigation strategy, or REMS which could include elements to assure safe use, or a medication guide outlining the risks of such side effects for distribution to patients;
- we may be subject to fines, injunctions or the imposition of criminal penalties;
- patients and/or treating physicians could perceive the risk of undesirable side effects or adverse events caused by the product to exceed its potential benefit and choose not to use the product;
- we could choose to remove such product from the market;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could impair our ability to develop or commercialize our products or any future product candidates, and their commercial potential may be materially and negatively impacted.

We rely on complex, single-source supply chains for SKYSONA, ZYNTEGLO, and LYFGENIA, respectively. The manufacture, testing and delivery of LVV and drug products present significant challenges for us, and we may not be able to produce our vector and drug products at the quality, quantities, or timing needed to support our clinical programs and commercialization.

We rely on third parties to manufacture the LVV and the drug product for ZYNTEGLO, SKYSONA and LYFGENIA. The manufacture of LVV and drug products is complex and requires significant expertise. Even with the relevant experience and expertise, manufacturers of cell therapy products often encounter difficulties in production, particularly in scaling out and validating initial production, managing the transition from clinical manufacturing to manufacturing in the commercial setting, and ensuring that the product meets required specifications. These problems include difficulties with production costs and yields, quality control, quality assurance testing, operator error, scarcity of qualified manufacturing and quality control testing personnel, shortages of any production raw materials as well as compliance with strictly enforced federal, state and foreign regulations. Further, the transition from clinical to commercial manufacturing is complex and has resulted in, and may continue to result in, lower operational success rates due to, among other things, tighter specifications and higher regulatory standards associated with commercial products. We cannot make any assurances that these problems will not occur in the future, or that we will be able to resolve or address in a timely manner or with available funds problems that occur. Because of this complexity, transitioning production of either LVV or drug products to backup or second source manufacturing requires a lengthy technology transfer process and regulatory review and approval, which often takes significant time and may require additional significant financial expenditures.

We currently have only one manufacturer of final drug product and one manufacturer of LVV for both ZYNTEGLO and SKYSONA and, separately, one manufacturer of final drug product and one manufacturer of LVV for LYFGENIA;

accordingly, any significant disruption or change in our supplier relationships could harm our business. For instance, we have recently provided notice to our manufacturer of LVV for ZYNTEGLO and SKYSONA that we intend to wind down production as we explore alternative manufacturing methods and plans for LVV used in these products. Further, we have experienced challenges in manufacturing adherent LVV, which is currently used in ZYNTEGLO and SKYSONA. As a result of these events, or other difficulties related to our manufacturing relationships and processes, we may be unable to meet our manufacturing forecasts. Any inability to meet our manufacturing forecasts could impact the ongoing commercialization of these drug products, and hinder our ability to meet our financial goals. Further, 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 SKYSONA, ZYNTEGLO, and LYFGENIA. Such suppliers may not sell these key materials to us or to our manufacturers at the times we need them or on commercially reasonable terms. We do not control the process for acquisition of all key materials and shortages may occur for reasons beyond our control.

We continue to advance plans to make additional investment in manufacturing to expand capacity and, to date, we have secured adequate commercial-scale drug product manufacturing capacity in order to meet our near-term sales forecasts for ZYNTEGLO, SKYSONA and LYFGENIA, including recent approval to double our manufacturing capacity for ZYNTEGLO and SKYSONA; however, any plans to further expand our manufacturing capacity are subject to FDA approval, which we may not receive in connection with any planned expansions. If we fail to secure adequate capacity to manufacture our drug products or LVV used in the manufacture of our drug products in accordance with our forecasts we may be unable to execute on our commercialization plans on the timing that we expect, or at all.

The actual cost to manufacture our LVV and drug products could be greater than we expect and could materially and adversely affect the commercial viability of SKYSONA, ZYNTEGLO, or LYFGENIA. If we or our third-party manufacturers are unable to produce the necessary quantities of LVV and drug product, or in compliance with GMP or other pertinent regulatory requirements, and within our planned time frame and cost parameters, including due to reduced operational success rates as a result of the transition to commercial manufacturing, the development and commercialization of our products and future product candidates may be materially harmed, result in delays in our plans or increased capital expenditures.

Additionally, since the hematopoietic stem cells ("HSCs") used as starting material for our products have a limited window of stability following procurement from a patient, we have initially established transduction facilities in areas that we believe can adequately service patients from regions where we are commercializing SKYSONA, ZYNTEGLO, and LYFGENIA. However, we cannot ensure that such facilities will enable us to produce and deliver drug product in a timely manner; any issues with production and delivery of drug product could have a material adverse effect on our successful commercialization or further development of our products or any future product candidates. Moreover, establishing additional facilities in appropriate regions may be financially impractical or 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.

Changes in our manufacturing processes may cause delays in our clinical development and commercialization plans.

The manufacturing processes for our LVV and our drug products are complex. We explore improvements to our manufacturing processes on a continual basis, as we evaluate clinical and manufacturing data and based on discussions with regulatory authorities. In some circumstances, changes in the manufacturing process may require us to perform additional comparability studies, collect additional data from patients, submit additional regulatory filings, or comply with additional requirements, which may lead to delays in our clinical development and commercialization plans. Such changes may require regulatory review and approval including reaching agreement with the FDA on an acceptable comparability data package. The FDA may require us to conduct additional clinical studies, collect additional data, develop additional assays, or modify product specifications relating to such comparability analysis and, therefore, the proposed change may not be approved in a timely manner, if at all. Any such requests or delays may impact our commercialization plans and may require substantial additional funds.

Risks related to commercialization

We have limited experience as a commercial company and the marketing and sale of ZYNTEGLO, SKYSONA and LYFGENIA may be unsuccessful or less successful than anticipated.

We have limited experience as a commercial company as we recently launched our three FDA-approved products, ZYNTEGLO, SKYSONA and LYFGENIA. Consequently, there is limited information about our ability to overcome many of

the risks and uncertainties encountered by companies commercializing products in the biopharmaceutical industry in the U.S. To execute our business plan, we will need to successfully:

- sustain adequate pricing and reimbursement for ZYNTEGLO, SKYSONA and LYFGENIA across all U.S. payer segments;
- establish and maintain, in the regions where we hope to treat patients, relationships with qualified treatment centers who will be treating the patients who receive ZYNTEGLO, SKYSONA, and LYFGENIA;
- manage our manufacturing capabilities and supply chain operations in the coordination and delivery of drug product to patients at qualified treatment centers;
- manage our spending as we engage in commercialization efforts;
- manage the patient uptake process for each of our products, including with respect to overall timing and potential barriers such as clinical assessment periods and payer approval processes; and
- initiate, develop and maintain successful strategic alliances.

If we are not successful in accomplishing these objectives, we may not be able to effectively commercialize ZYNTEGLO, SKYSONA or LYFGENIA, raise capital, expand our business, or continue our operations. For instance, the phasing of LYFGENIA patient starts has affected the timing of our revenue expectations. If we are unable to meet our forecasts, our business may suffer.

The commercial success of ZYNTEGLO, SKYSONA and LYFGENIA will depend upon the degree of market acceptance by physicians, patients, payers and other stakeholders.

The commercial success of ZYNTEGLO, SKYSONA, and LYFGENIA will depend in part on the medical community, patients, and third-party or governmental payers accepting gene therapy products in general, and ZYNTEGLO, SKYSONA, and LYFGENIA, in particular, as medically useful, cost effective, and safe. ZYNTEGLO, SKYSONA, and LYFGENIA may not gain market acceptance by physicians, patients, payers and other stakeholders. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable and our future business prospects will be adversely impacted. The degree of market acceptance of ZYNTEGLO, SKYSONA, and LYFGENIA will depend on a number of factors, including:

- our ability to compete with alternative treatments, including other approved gene therapies for similar indications, including with respect to potential and perceived efficacy and other potential advantages;
- the prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling; for instance, each of the LYFGENIA and SKYSONA product labels includes a boxed warning for the risk of hematologic malignancy;
- the prevalence and severity of any side effects resulting from the chemotherapy and myeloablative treatments associated with the procedure by which our products are administered, including the possible prejudicial effects that chemotherapy can have on fertility;
- relative convenience and ease of administration, including patients' willingness and ability to travel to qualified treatment centers within our network;
- given the complexity of manufacturing product and the reduced operational success rates in connection with the transition to commercial manufacturing, the perception or possibility that issues may continue to arise in the supply of product which could delay treatment;
- 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;
- the pricing of our products, including in comparison to competitors;
- publicity concerning our products, or competing products and treatments;
- sufficient insurance coverage or reimbursement;
- the possible occurrence of adverse clinical findings or decreased effectiveness of a product or product candidate over time identified during continued monitoring and evaluation of patients; and
- the mix of private and governmental payer coverage, which can impact both the total reimbursement for the drug and the time-to-reimbursement, and the conditions to coverage imposed by the various payers, including non-preferred or exclusion decisions in favor of our competitor.

Even if a product displays a favorable efficacy and safety profile in clinical studies, market acceptance of the product will not be known until some period after it is launched. Our efforts to educate the medical community and payers on the benefits of our products may require significant resources and may never be successful. Our efforts to educate the marketplace may require more resources than are required by the conventional technologies marketed by our competitors. Any of these factors may cause ZYNTEGLO, SKYSONA, or LYFGENIA to be unsuccessful or less successful than anticipated.

If the market opportunities for our commercial products or any future product candidates are smaller than we believe they are, and if we are not able to successfully identify patients and achieve significant market share, our revenues may be adversely affected and our business may suffer.

Our platform focuses on treatments for severe genetic 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 products or any future product candidates we may develop, are based on estimates. These estimates have been derived from a variety of sources, including scientific literature, surveys of clinics, patient foundations, or market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower or more difficult to identify than expected. Additionally, the potentially addressable patient populations for our products or any future product candidates may be limited or may not be amenable to treatment with such products or product candidates. For instance, each of the SKYSONA and LYFGENIA product labels includes a boxed warning for the risk of hematologic malignancy, which may impact market opportunity.

Any of these factors may negatively affect our ability to generate revenues from sales of our products as forecasted and our ability to achieve and maintain profitability and, as a consequence, our business may suffer.

We have limited sales and distribution experience and limited capabilities for marketing and market access. Although we have invested and expect to continue to invest significant financial and management resources, if we are unable to establish and maintain these commercial capabilities and infrastructure, or to enter into agreements with third parties to market and sell our products, we may be unable to generate sufficient revenue to sustain our business.

We have limited prior sales or distribution experience and limited capabilities for marketing and market access, and we did not generate meaningful product sales following the commercial launch of ZYNTEGLO following marketing approval in Europe. To successfully commercialize ZYNTEGLO, SKYSONA, and LYFGENIA, we will need to further develop these capabilities. We may need to expand our infrastructure to further support commercial operations in the United States, either on our own or with others. Commercializing an autologous gene therapy is resource-intensive and has required, and will continue to require, substantial investment in commercial capabilities. We are competing with companies that currently have extensive and well-funded marketing and sales operations. Without significant commercial experience as a company or the support of a third party to perform these functions, including marketing and sales functions, we may be unable to compete successfully against these more established companies.

Furthermore, a significant proportion of the patient populations for ZYNTEGLO, SKYSONA, and LYFGENIA lies outside of the United States. We currently expect to focus our operations and efforts on markets in the United States and will need to rely heavily on third parties for commercializing any products in geographies outside of the United States, if at all. We may enter into collaborations with third parties to utilize their mature marketing and distribution capabilities, but we may be unable

to enter into agreements on favorable terms, if at all. If we do not enter into collaboration arrangements with third parties to pursue regulatory authorization or commercialization of our programs for markets outside of the United States, or if our future collaborative partners do not commit sufficient resources to such efforts, we may be unable to generate sufficient revenue to sustain our business.

We may encounter challenges with engaging or coordinating with qualified treatment centers needed for the ongoing commercialization of ZYNTEGLO, SKYSONA and LYFGENIA.

Our commercial strategy is to engage apheresis and transplant centers as qualified treatment centers for the collection of patient HSCs and infusion of the drug product once manufactured. To ensure that the qualified treatment centers are prepared to collect patient HSCs and to ship them to our transduction facilities in accordance with our specifications and regulatory requirements, we train and conduct quality assessments of each center as part of engagement. These qualified treatment centers are the first and last points on our complex supply chain to reach patients in the commercial setting. We may encounter challenges or delays in engaging and interacting with our qualified treatment centers, and such challenges could impact a qualified treatment center's willingness and ability to administer our products.

Furthermore, we may fail to manage the logistics of collecting and shipping patient material to the manufacturing site and shipping the drug product back to the patient. Logistical and shipment delays and problems caused by us, our third-party vendors, and other factors not in our control, such as weather, could prevent or delay the manufacture of or delivery of drug product to patients. If our qualified treatment centers fail to perform satisfactorily, we may suffer reputational, operational, and business harm. Additionally, delays with infusion at the qualified treatment centers, due to, for instance, the patient's schedule or health condition or such center's capacity or the availability of manufacturing slots at our CMOs, or due to the need for multiple cell collections, could result in a patient becoming medically ineligible for our treatment or selecting an alternative treatment, the drug product becoming unusable and loss of medical coverage, which would have a material adverse effect on commercial sales. These delays may also impact our relationship with our qualified treatment center network. Any failure in our engagement or interaction with our qualified treatment centers due to delays in treatment or complications related to manufacturing, among other things, may limit patient access to our therapies and, accordingly, have a material adverse effect on our commercial forecasts and business.

We are required to maintain a complex chain of identity and chain of custody with respect to patient material as it moves through the manufacturing process, from the qualified treatment center to the transduction facility, and back to the patient. Failure to maintain chain of identity and chain of custody could result in adverse patient outcomes, loss of product or regulatory action.

The insurance coverage and reimbursement status of newly-approved products in the United States is uncertain. Due to the novel nature of our technology and the potential for our products to offer lifetime therapeutic benefit in a single administration, we face unique and additional challenges in obtaining adequate coverage and reimbursement for our products. Failure to obtain or maintain adequate coverage and reimbursement for any new or current product, including to the extent that payers 'non-prefer' any or all of our therapies to our competitors, 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 payers is essential for most patients to be able to afford healthcare, and especially expensive medicines, such as gene therapy products. Sales of our products depend substantially on the extent to which our products are covered by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or are reimbursed by government health administration authorities, private health coverage insurers and other payers. There is no assurance that payers will be willing to, or continue to, reimburse providers at the company-established list price or that reimbursement levels that payers will be willing to pay will be sufficient. Moreover, given that our therapies are generally administered in the inpatient care setting, it is important that our products are either reimbursed as a separate item from the underlying services incurred during the patient's hospitalization or that, if reimbursement for our therapies is "bundled" with reimbursement for the hospital stay, the bundled payment rate adequately reflects the price of our therapy. We cannot assure you that payers will agree to either "separate reimbursement" or an appropriate bundled payment rate. Accordingly, the estimation of potential revenues is complex and it is difficult to predict what payers 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.

In the U.S., regional Medicare Administrative Contractors ("MACs") are responsible for making a determination with regard to whether a new therapy meets the federal standard of "reasonable and necessary" such that it is covered and reimbursed by Medicare. For the Medicaid program, each State Medicaid Agency is responsible for establishing coverage

criteria, billing policies, and reimbursement rates for FDA-approved drugs. Reimbursement methodologies in Medicare and Medicaid can vary based on the type of therapeutic agent and setting of care, and for Medicaid, the reimbursement methodologies also vary by state. There is uncertainty with this process both in terms of the timing of the decision-making process and the coverage decision itself. We anticipate that Medicaid coverage will be significant for the potential patient population for our products. On the other hand, we anticipate that Medicare coverage will be less significant, given that only a small percentage of our patient population may be Medicare eligible. We expect these patients may be dually eligible for Medicare and Medicaid based on meeting federally-established disability standards, in which case Medicare serves as the primary payer and Medicaid as the secondary payer for any service not otherwise covered by Medicare that is covered under a State's Medicaid program.

Moreover, increasing efforts by governmental and third-party payers to cap or reduce healthcare costs may cause such payers 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 ZYNTEGLO, SKYSONA, or LYFGENIA. The reimbursement policies of reinsurers, stop-loss carriers, and self-insured employers, including those that exclude coverage for gene therapies, could negatively impact our ability to market our therapies. We expect to experience pricing pressures in connection with the sale of our products due to greater scrutiny on list prices and total prescription drug spending across all payer channels as well as additional legislative changes at the state and federal level; moreover, public pressure from payers or negative public opinion regarding our list prices could affect the perception of our company and the value or cost-effectiveness of our therapies, which could impact our ability to successfully market our products. Further, net prices for drugs may be reduced by mandatory discounts or rebates required by government or private payers. As a result, increasingly high barriers are being erected to the entry of new products, often in the form of limiting the patient population for whom a new therapy is deemed “medically necessary.” Even if coverage is provided, the amount payers are willing to reimburse may not be sufficient.

Furthermore, because a provider is responsible for costs associated not just with obtaining our medicines but also with the underlying hospital stay in which the administration of our therapies occurs, the pricing and reimbursement dynamics that impact patient access are not entirely within our control as providers and payers negotiate separately for the cost of the associated items and services, decisions in which we cannot and do not play a role. These services include the collection of HSCs from the patient, followed by chemotherapy and myeloablative treatments, and inpatient hospital stay following drug product infusion. If our customers are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our products will be adversely affected.

We have entered into and continue to engage with payers across all channels around outcomes-based contracts for ZYNTEGLO and LYFGENIA. In the event that a payer opts for the outcomes-based contract, we will need to reserve a certain portion of revenue from each sale to account for the potential that a rebate will be owed if the pre-established outcome metric is not achieved over a designated period of time, which differs depending on the product and the agreement, following drug product administration. The amount of revenue reserved for a potential rebate depends on the product and payer type; for instance, our outcomes-based contract for ZYNTEGLO could require us to remit up to 80% of the cost of the therapy to a payer based on patient outcomes achieved. In the event that rebates are due under these contracts, we may be required to adjust revenue previously recognized. Despite our efforts to engage with CMS and work with experts to ensure all of our payer contracting efforts comply with relevant federal and state regulations, including government price reporting obligations, given the complexity of these arrangements, it is not possible to completely mitigate the risk that our interpretation differs from that of the regulatory authorities such that we may not be able to satisfy the compliance requirements, which may result in significant fines and liability.

Collectively, these factors could affect our ability to successfully commercialize our products and generate or recognize revenues, which would adversely impact our business, financial condition, results of operations and prospects.

Risks related to the research and development of our products and any future product candidates

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

We are engaged in the development and commercialization of gene therapies for severe genetic diseases, which is a competitive and 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. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff, more experienced manufacturing capabilities, or more established commercial infrastructure. For instance, the FDA has

approved a gene therapy for the treatment of sickle cell disease and beta thalassemia from Vertex Pharmaceuticals, Inc., which does not have a boxed warning and has a lower wholesale acquisition cost in the United States than that of LYFGENIA and ZYNTEGLO. 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, safer, or less costly than any products that we may develop, or achieve patent protection, marketing approval, product commercialization and market penetration earlier than us. Additionally, technologies developed by our competitors may render our products or any future product candidates uneconomical or obsolete. As a result of any of these factors, we may not be successful in marketing our products against competitors.

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.

Clinical drug development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our products and any future product candidates.

In order to obtain and maintain marketing approval from regulatory authorities for the commercialization of our products and future product candidates, we must conduct extensive clinical studies to demonstrate the safety, purity and potency, and/or efficacy, of the product candidates in humans. Clinical testing is expensive, time-consuming and uncertain as to outcome. There is a high failure rate for therapies 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. 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 of our products and product candidates include:

- inability to generate sufficient preclinical, toxicology, or other in vivo or in vitro data to support the initiation or continuation of clinical trials;
- delays or failure in obtaining regulatory authorization to commence a trial;
- delays in reaching agreement on acceptable terms with prospective contract research organizations ("CROs"), and clinical trial sites, and QTCs participating in post-approval registry studies, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical trial sites;
- delays in identifying, recruiting and training suitable clinical investigators;
- delays in obtaining required IRB or ethics committee approvals at each clinical trial and/or QTC registry site;
- delays in manufacturing, testing, releasing, validating or importing/exporting sufficient stable quantities of our future product candidates for use in clinical trials or the inability to do any of the foregoing;
- insufficient or inadequate supply or quality of drug product or other materials necessary for use in clinical trials, or delays in sufficiently developing, characterizing or controlling a manufacturing process suitable for clinical trials;
- imposition of a clinical hold by regulatory agencies, including after review of an IND or amendment or equivalent foreign application or amendment, as a result of a new safety finding that presents unreasonable risk to clinical trial participants or after an inspection of our clinical study operations or study sites or due to unforeseen safety issues;
- failure by our CROs, other third parties or us to adhere to clinical trial protocols or failure to perform in accordance with the FDA's or any other regulatory authority's good clinical practice requirements ("GCPs") or applicable regulatory guidelines in other countries;
- occurrence of adverse events associated with the product or product candidate that are viewed to outweigh its potential benefits, or occurrence of adverse events in trial of the same class of agents conducted by other companies, particularly

due to the fact that we are required to follow patients in our clinical and registry studies for an extended period of time (up to 15 years);

- changes to the clinical trial protocols;
- clinical sites deviating from trial protocol or dropping out of a trial;
- changes in the standard of care on which a clinical development plan was based, which may require new or additional trials;
- selection of clinical endpoints that require prolonged periods of observation or analyses of resulting data;
- the cost of clinical trials of our products or future product candidates being greater than we anticipate;
- clinical trials of our products or future product candidates producing negative or inconclusive results, which may result in our deciding, or regulators requiring us, to conduct additional clinical trials or abandon development of such product candidates;
- transfer of manufacturing processes to larger-scale facilities operated by a CMO and delays or failure by our CMOs or us to make any necessary changes to such manufacturing process; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

Clinical trials must be conducted in accordance with the FDA and other applicable regulatory authorities' legal requirements, regulations or guidelines, and are subject to oversight by these governmental agencies and ethics committees or IRBs at the medical institutions where the clinical trials are conducted.

Further, conducting clinical trials in foreign countries, as we may do for our products or any future product candidates, presents additional risks that may delay completion of clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our products or product candidates.

Delays in the completion of any clinical trial of our products or product candidates will increase our costs, slow down our product candidate development and approval process and delay or potentially jeopardize our ability to commence or continue product sales and generate product revenue. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates. Any delays to our clinical trials that occur as a result could shorten any period during which we may have the exclusive right to commercialize our product candidates and our competitors may be able to bring products to market before we do, and the commercial viability of our product candidates could be significantly reduced. Any of these occurrences may harm our business, financial condition and prospects significantly.

We depend on enrollment of patients in our registry studies to complete required post marketing studies for our products, and on enrollment of patients in any future clinical trials we may conduct. If we experience delays or difficulties enrolling in

our registry studies or any future clinical trials, our research and development efforts, business, financial condition, and results of operations could be materially adversely affected.

Successful and timely completion of clinical trials, including additional trials that the FDA may require we complete prior to or as part of approval of our products or future product candidates, will require that we enroll a sufficient number of patient candidates. For instance, we are required to conduct long-term observational registry studies evaluating the safety of ZYNTEGLO, SKYSONA and LYFGENIA. These registry studies and other trials we may decide to conduct may be subject to delays for a variety of reasons, including as a result of patient enrollment taking longer than anticipated, patient withdrawal or adverse events. These types of developments could cause us to delay the study or halt further development. If we are unable to complete required registry studies or any other post-marketing requirements under the terms specified by the FDA, we could be subject to FDA enforcement action, including restrictions on our ability to sell our products, misbranding charges and civil monetary penalties.

Additionally, any future clinical trials we may conduct could compete with other clinical trials that are in the same therapeutic areas as any future product candidates, and this competition could reduce the number and types of patients available to us, as some patients who might have opted to enroll in our trials or to receive our commercial therapies may instead opt to enroll in a trial being conducted by one of our competitors. Because the number of qualified clinical investigators and clinical trial sites for the patient populations we pursue may be limited, we may conduct one or more future clinical trials at the same clinical trial sites that some of our competitors use, which will reduce the number of patients who are available for our clinical trials at such clinical trial sites. In addition, there may be limited patient pools from which to draw for clinical studies. In addition to the rarity of some diseases, the eligibility criteria of future clinical studies may further limit the pool of available study participants as we may require that patients have specific characteristics that we can measure or to assure their disease is either severe enough or not too advanced to include them in a study.

Patient enrollment depends on many factors, including:

- the size and nature of the patient population;
- the severity of the disease under investigation;
- eligibility criteria for the trial;
- the proximity of patients to clinical sites;
- the design of the clinical protocol;
- the ability to obtain and maintain patient consents;
- the ability to recruit clinical trial investigators with the appropriate competencies and experience;
- the risk that patients enrolled in clinical trials will drop out of the trials before the administration of our product candidates or trial completion;
- the availability of competing clinical trials;
- the availability of new drugs approved for the indication the clinical trial is investigating; and
- clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies.

These factors may make it difficult for us to enroll enough patients to complete our registry studies or any future clinical trials in a timely and cost-effective manner. Delays in the completion of any clinical trial may increase our costs, slow down our development process and could delay, or potentially jeopardize, our ability to obtain and maintain required regulatory approvals, commercialize our products or any future product candidates and generate revenue.

Data from our clinical trials that we announce or publish from time to time may change as more patient data become available either through long-term patient follow-up and/or as such data are audited and verified, which could result in material changes to clinical and safety profiles for our products.

From time to time, we may disclose top-line, interim or preliminary data from our clinical trials. Such data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available or as patients from our clinical trials continue other treatments for their disease. In addition, the clinical trials evaluating our products, and likely those evaluating any future product candidates, generally require that we continue to monitor and evaluate safety and efficacy in patients over an extended period of time following treatment, including for up to fifteen years for some studies, which may result in changes to the safety or efficacy profile over time. Changes in the efficacy and safety profile of our products or any future product candidates over time could significantly harm our business prospects including resulting in volatility in the price of our common stock.

Additionally, preliminary or top-line data are based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. Interim data from clinical trials that we may conduct are further subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. As a result, the top-line, interim or preliminary results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Further, disclosure of such data by us or by our competitors could also result in volatility in the price of our common stock. Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our Company in general. In addition, the information we choose to publicly disclose regarding a particular study or clinical trial is based on what is typically extensive information, and you or others may not agree with what we determine is material or otherwise appropriate information to include in our disclosure. If others, including regulatory authorities, disagree with the conclusions reached with respect to such information and assessments, our ability to obtain approval for, and commercialize, our products and any future product candidates may be harmed, which could harm our business, operating results, prospects or financial condition.

Although we have received accelerated approval from the FDA for SKYSONA, if our confirmatory trials do not verify clinical benefit, or if we do not comply with rigorous post-marketing requirements, the FDA may seek to withdraw any accelerated approval we have obtained.

In September 2022, SKYSONA received accelerated approval from the FDA and we may in the future seek accelerated approval for one or more future product candidates. Under the accelerated approval program, the FDA may grant accelerated approval to a product candidate designed to treat a serious or life-threatening condition that provides meaningful therapeutic benefit over available therapies upon a determination that the product candidate has an effect on a surrogate endpoint or intermediate clinical endpoint that is reasonably likely to predict clinical benefit. The FDA considers a clinical benefit to be a positive therapeutic effect that is clinically meaningful in the context of a given disease, such as irreversible morbidity or mortality. For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. An intermediate clinical endpoint is a clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit.

The accelerated approval pathway may be used in cases in which the advantage of a new drug over available therapy may not be a direct therapeutic advantage, but is a clinically important improvement from a patient and public health perspective. If granted, accelerated approval is usually contingent on the sponsor’s agreement to conduct, in a diligent manner, one or more additional confirmatory studies to verify and describe the drug’s clinical benefit. If such post-approval studies fail to confirm the drug’s clinical benefit or are not completed in a timely manner, the FDA may withdraw its approval of the drug on an expedited basis. For example, we agreed to provide confirmatory long-term clinical data to the FDA as a condition of the SKYSONA accelerated approval, and continued approval for the approved indication will be contingent upon verification of clinical benefit with confirmatory clinical data. Moreover, certain payers, including state Medicaid agencies, may scrutinize therapies that reach the market through accelerated approval, which can lead to delays in broader access after approval and require additional company resources to address any concerns.

In addition, in December 2022, President Biden signed an omnibus appropriations bill to fund the U.S. government through fiscal year 2023. Included in the omnibus bill is the Food and Drug Omnibus Reform Act of 2022, which among other things, provided FDA new statutory authority to mitigate potential risks to patients from continued marketing of ineffective drugs previously granted accelerated approval. Under these provisions, the FDA may require a sponsor of a product seeking accelerated approval to have a confirmatory trial underway prior to such approval being granted.

We did not receive a priority review voucher in connection with the FDA approval of LYFGENIA. Although we are pursuing the Formal Dispute Resolution process with the FDA, there is no guarantee that we will be successful or receive the voucher.

In 2012, Congress authorized the FDA to award priority review vouchers to sponsors of certain rare pediatric disease product applications. This program is designed to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Specifically, under this program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" that meets certain criteria may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease drug product receiving a priority review voucher may transfer (including by sale) the voucher to another sponsor. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application. The FDA may also revoke any priority review voucher if the rare pediatric disease drug for which the voucher was awarded is not marketed in the U.S. within one year following the date of approval.

We obtained a rare pediatric disease designation for lovo-cel for the treatment of SCD and in October 2023, we entered into an agreement to sell a priority review voucher, if received by March 31, 2024, for $103.0 million. However, upon FDA approval of LYFGENIA in December 2023, we did not receive a priority review voucher. We are pursuing the Formal Dispute Resolution process with the FDA to dispute this decision; however, the FDA dispute process is uncertain and there is no guarantee that we will receive the voucher. Moreover, the dispute process is time consuming and may result in substantial costs and distraction to our management. Because we did not receive a priority review voucher by March 31, 2024, the outside date under our previously announced sale agreement has passed and the buyer has the right to terminate the agreement at any time.

Our biological products may face competition sooner than anticipated.

The Affordable Care Act includes a subtitle called the Biologics Price Competition and Innovation Act of 2009 ("BPCIA"), which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a highly similar or "biosimilar" product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. The 12-year exclusivity blocks the submission and approval of biosimilars under the abbreviated pathway only. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. This exclusivity is only available to the "first licensure" of the reference biological product. If a biological product has a related structure to a previously licensed product from the same sponsor, it may not qualify as a first licensure. If LYFGENIA and ZYNTEGLO are considered to have a related structure, it is possible that LYFGENIA will not be granted its own 12-year exclusivity period and accordingly would be protected under ZYNTEGLO's 12-year exclusivity period.

Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for non-biological products is not yet clear, and will depend on a number of marketplace factors that are still developing. This may further incentivize the development of competing versions or our products under the full BLA pathway rather than the biosimilars pathway.

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

Public perception may be influenced by claims that gene therapy, including gene editing technologies, is unsafe or unethical, and research activities and adverse events in the field, even if not ultimately attributable to us or our products or any future product candidates, could result in increased governmental regulation, unfavorable public perception, challenges in recruiting patients to participate in our clinical studies, potential regulatory delays in the testing or approval of our products or any future product candidates, stricter labeling requirements for our approved products, and a decrease in demand for any such

product. 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 products or any future product candidates or reduce demand for any approved products.

Disruptions at the FDA and other government agencies caused by funding shortages or global health concerns could hinder their ability to hire, retain or deploy key leadership and other personnel, prevent new or modified products from being developed, reviewed, approved or commercialized in a timely manner or at all, which could negatively impact our business.

The ability of the FDA and foreign regulatory authorities to review and approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory, and policy changes, the FDA's or foreign regulatory authorities' ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's or foreign regulatory authorities' ability to perform routine functions. Average review times at the FDA and foreign regulatory authorities have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable. Disruptions at the FDA may also slow the time necessary for new drugs, medical devices and biologics or modifications to approved drugs and biologics to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities.

Separately, in response to the global COVID-19 pandemic, the FDA postponed most inspections of domestic and foreign manufacturing facilities at various points. Even though the FDA has since resumed standard inspection operations any resurgence of the virus or emergence of new variants may lead to inspectional or administrative delays. If a prolonged government shutdown occurs, or if global health concerns prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

A Regenerative Medicine Advanced Therapy designation by the FDA, even if granted for any future product candidate, may not lead to a faster development or regulatory review or approval process and does not increase the likelihood that such future product candidate will receive marketing approval.

We have obtained Regenerative Medicine Advanced Therapy ("RMAT") designation for LYFGENIA for the treatment of SCD, and we may seek additional RMAT designations for our future product candidates. A biological product candidate is eligible for RMAT designation if: (1) it meets the definition of a regenerative medicine therapy, which the FDA defines as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, with limited exceptions; (2) the candidate is intended to treat, modify, reverse, or cure a serious disease or condition; and (3) preliminary clinical evidence indicates that the candidate has the potential to address unmet medical needs for such disease or condition. RMAT designation provides potential benefits that include more frequent meetings with FDA to discuss the development plan for the product candidate, and eligibility for rolling review and priority review of BLAs. Product candidates granted RMAT designation may also be eligible for accelerated approval on the basis of a surrogate or intermediate endpoint reasonably likely to predict long-term clinical benefit, or through reliance upon data obtained from a meaningful number of sites, including through expansion to a sufficient number of sites, as appropriate. RMAT-designated product candidates that receive accelerated approval may, as appropriate, be able to fulfill their post-approval requirements through the submission of clinical evidence, clinical studies, patient registries, or other sources of real-world evidence (such as electronic health records); through the collection of larger confirmatory data sets; or via post-approval monitoring of all patients treated with such therapy prior to approval of the therapy.

RMAT designation is within the sole discretion of the FDA. Accordingly, even if we believe one of our future product candidates meets the criteria for RMAT designation, the FDA may disagree and instead determine not to make such designation. RMAT designation does not change the standards for product approval, and there is no assurance that such designation or eligibility for such designation will result in expedited review or approval or that the approved indication will not be narrower than the indication covered by the RMAT designation. Additionally, RMAT designation can be revoked if the product candidate fails to meet the qualifications as clinical data continue to emerge.

We have obtained orphan drug designation for our products, but we may be unable to maintain the benefits associated with orphan drug designation, including market exclusivity, which may cause our product revenue, if any, to be reduced.

We have obtained orphan drug exclusivity for certain diseases or conditions for LYFGENIA, ZYNTEGLO and SKYSONA. Under the Orphan Drug Act, the FDA may designate a biological product as an orphan drug if it is intended to

treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. Orphan drug designation must be requested before submitting a BLA. In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, waivers from certain pediatric clinical trial requirements, and application fee waivers. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA.

In addition, if a product candidate receives the first FDA approval for the disease or condition for which it has orphan designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same disease or condition for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer is unable to assure sufficient product quantity for the orphan patient population. Exclusive marketing rights in the United States may also be unavailable if we or our collaborators seek approval for a disease or condition broader than the orphan designated disease or condition and may be lost if the FDA later determines that the request for designation was materially defective.

Even if we obtain orphan drug designation for a future product candidate, we may not be the first to obtain marketing approval for any particular orphan disease or condition due to the uncertainties associated with developing pharmaceutical products. Further, we have received orphan drug exclusivity from the FDA for ZYNTEGLO for the treatment of adult and pediatric patients with beta-thalassemia who require regular red blood cell (RBC) transfusions; for SKYSONA for the slowing of progression of neurologic dysfunction in boys 4-17 years of age with early, active cerebral adrenoleukodystrophy; and for LYFGENIA for the treatment of patients 12 years of age or older with sickle cell disease and a history of vaso-occlusive events. These orphan drug exclusivities, and any exclusivities we may obtain in the future may not effectively protect the product from competition because different drugs can be approved for the same disease or condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is safer, more effective, or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

Risks related to our reliance on third parties

We rely on third parties to conduct some or all aspects of our LVV production, drug product manufacturing, and testing, and these third parties may not perform satisfactorily.

We do not independently conduct all aspects of our LVV production, drug product manufacturing, and testing. We currently rely, and expect to continue to rely, on third parties with respect to these items, including manufacturing and testing in the commercial context.

Our reliance on these third parties for manufacturing, testing, research and development activities reduces 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 products 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, and that our LVV and drug products are manufactured in accordance with GMP as applied in the relevant jurisdictions.

If these third parties do not successfully carry out their contractual duties, including as a result of insolvency, meet expected deadlines, conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, or manufacture our LVV and drug products in accordance with GMP, we will not be able to support commercialization of SKYSONA, ZYNTEGLO and LYFGENIA. Many of our agreements with these third parties contain termination provisions that allow these third parties to terminate their relationships with us at any time. If we need to enter into alternative arrangements, our product development and commercialization activities could be delayed.

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

- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms, including the inability to negotiate favorable terms to increase capacity to meet future forecasted demand;

- reduced control as a result of using third-party manufacturers for all aspects of manufacturing activities;

- the risk that these activities are not conducted in accordance with our study plans and protocols, including the potential for failed product batches that have resulted, and may in the future result, in delays in treatment of patients;

- 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, for example, the bankruptcy or financial condition of the manufacturer or supplier.

We may be forced to manufacture LVV and drug product ourselves, for which we may not have the capabilities or resources, or enter into an agreement with a different manufacturer, which we may not be able to do on reasonable terms, if at all. In some cases, the technical skills required to manufacture our LVV or future product candidates may be unique or proprietary to the original manufacturer, and we may have difficulty or there may be contractual restrictions prohibiting us from, transferring such skills to a back-up or alternate supplier, or we may be unable to transfer such skills at all. Any of these events could lead to clinical study delays or impact our ability to obtain required regulatory approvals or successfully commercialize our products or any future product candidates. 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.

All entities involved in the preparation of therapeutics for clinical studies or commercial sale, including our existing contract manufacturers for our products, or those we may use for any future product candidates, are subject to extensive regulation. Some 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 products or any future product candidates that may not be detectable in final product testing. We or our contract manufacturers must adhere to the FDA's or other regulator's good laboratory practices ("GLP"), and GMP regulations enforced by the FDA or other regulator through facilities inspection programs. Some of our contract manufacturers, particularly those we use for the commercial production of LYFGENIA, have not previously produced a commercially-approved product and therefore have not previously obtained the requisite FDA or other regulatory approvals to do so. Our facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors may be required to successfully complete a pre-approval inspection for compliance with GMPs and other applicable regulations as a condition of certain regulatory approvals. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If these facilities do not successfully complete any required inspections, it is possible FDA or other marketing approvals may be delayed, prevented or otherwise adversely affected.

The regulatory authorities also may, at any time following approval of a product for sale, audit the manufacturing facilities 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.

Further, any plans to expand our manufacturing capacity are subject to the review and approval of regulatory authorities and there is no guarantee that we will receive such approval on the timelines we anticipate. Delays in our expansion of manufacturing capacity could affect our ability to meet demand and could materially harm our business.

If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA or other regulators can impose regulatory sanctions including, among other things, refusal to approve a pending application for a 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. The number of manufacturers with the necessary manufacturing capabilities is limited. In addition, an alternative manufacturer would need to be qualified through a BLA supplement or similar regulatory submission 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 products or any future product candidates, cause us to incur higher costs and prevent us from successfully commercializing our products or any future product candidates. 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 or commercial production may be delayed and we could lose potential revenues.

We 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 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 and in accordance with applicable GCPs, GLPs and other 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 and other regulatory authorities' GCPs for conducting, recording and reporting the results of 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. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators and trial sites. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials complies with GCP regulations. 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 and other regulatory authorities may require us to perform additional clinical studies before approving any marketing applications.

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 successfully commercialize our products or any future product candidates. As a result, our financial results and the commercial prospects for our products or any future product candidates would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

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 drug products, 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 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 our financial condition and capital requirements

We have not generated material revenue from product sales and may never be profitable.

Our ability to generate revenues and achieve profitability depends on our ability, alone or with strategic collaboration partners, to successfully commercialize ZYNTEGLO, SKYSONA and LYFGENIA and other potential future product candidates (if and when approved). Our ability to generate revenues from product sales depends heavily on our success in:

- developing a sustainable, commercial-scale, reproducible, and transferable manufacturing process for our vectors and drug products;
- 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 for our product candidates and commercial demand for our approved products;
- launching and commercializing our approved products with a sustainable field-based team and marketing and distribution infrastructure;
- obtaining sufficient pricing and reimbursement for our approved products from private and governmental payers;
- obtaining market acceptance and adoption of our approved products and gene therapy as a viable treatment option;
- addressing any competing technological and market developments;
- completing research and preclinical and clinical development of future product candidates;
- seeking and obtaining regulatory and marketing approvals for future product candidates for which we complete clinical studies;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter; and
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how.

We expect to continue to incur significant expenditures for the foreseeable future, and we expect these expenditures to increase, which costs may increase further as competitors enter the market. Even if we are able to generate material product revenues, we may not become profitable and may need to obtain additional funding to continue operations.

If the estimates we make, or the assumptions on which we rely, in preparing our consolidated financial statements are incorrect, our actual results may vary from those reflected in our projections and accruals.

Our consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP"). The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, and related disclosure of contingent assets and liabilities. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. We cannot assure you, however, that our estimates, or the assumptions underlying them, will be correct. We may be incorrect in our assumptions regarding the applicability of drug pricing programs and rebates that may be applicable to our products and future product candidates, which may result in our under- or over-estimating our anticipated product revenues especially as applicable laws and regulations governing pricing evolve over time. In addition, to the extent payment for our products and future product candidates is subject to outcomes-based arrangements over

time, as it is for ZYNTEGLO and LYFGENIA, the total payments received from product sales may vary, our cash collection of future payments and revenue assumptions from product sales will be at risk, and the timing of revenue recognition will not correspond to the timing of cash collection.

Further, from time to time we issue financial guidance relating to our expectations for our cash, cash equivalents, and marketable securities available for operations, which guidance is based on estimates and the judgment of management. Moreover, our future net product revenues will depend upon the size of the markets in which the products have received approval, the ability to manufacture and deliver drug product to patients, the ability of such products to achieve sufficient market acceptance, reimbursement from third-party payers, adequate market share in those markets and performance of the drug product subject to outcome-based programs. If, for any reason, our expenses differ materially from our guidance or we utilize our cash more quickly than anticipated, we may have to adjust our publicly announced financial guidance. If we fail to meet, or if we are required to change or update any element of, our publicly disclosed financial guidance or other expectations about our business, including with respect to revenue generation, our stock price could decline.

Our operating results may fluctuate significantly, which makes our future operating results difficult to predict and could cause our operating results to fall below expectations or our guidance.

Our operating results are difficult to predict and will likely fluctuate from quarter to quarter and year to year. We expect that revenues from product sales will be difficult to predict from period to period, given the absence of significant historical sales data for ZYNTEGLO, SKYSONA, and LYFGENIA.

Further, changes in our operations, such as undertaking of additional programs, or business activities, or entry into strategic transactions, including potential future acquisitions of products, technologies or businesses may also cause significant fluctuations in our expenses.

The cumulative effects of these factors, further exacerbated by the impact of the ongoing volatility in macro-economic conditions, will likely result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-period basis may not be meaningful. Investors should not rely on our past results as an indication of our future performance. This variability and unpredictability could also result in our failing to meet the expectations of industry or financial analysts or investors for any period. If our revenue or operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially. Such a stock price decline could occur even when we have met any previously publicly stated revenue or earnings guidance we may provide.

Our existing and any future indebtedness could adversely affect our ability to operate our business.

On March 15, 2024, we entered into a Loan and Security Agreement, by and among the Company, the several banks and other financial institutions or entities party thereto, as lenders (the “Lender”), and Hercules Capital, Inc., as administrative agent and collateral agent, which we amended on April 30, 2024, July 9, 2024, August 13, 2024 and August 29, 2024 (as amended, the "LSA"). The LSA provides a secured term loan facility of up to $175.0 million (collectively, the “Term Loans”), consisting of: (a) an initial tranche of term loans in an aggregate amount of $75.0 million, which was funded at closing (the “Initial Loan”); (b) an additional tranche of term loans in an aggregate amount of $25.0 million, which will be available, subject to customary terms and conditions, during the period commencing on the date the Company has (x) received at least $75.0 million in gross cash proceeds from qualified financing transactions by December 20, 2024 and (y) completed patient starts (cell collections) for at least 50 LYFGENIA patients by March 31, 2025 or 70 LYFGENIA patients by June 30, 2025 (the “Tranche 2 Milestone”) and ending on the earlier of (i) the date that is 30 days immediately following achievement of the Tranche 2 Milestone and (ii) July 31, 2025; (c) an additional tranche of term loans in an aggregate amount of $25.0 million, which will be available, subject to customary terms and conditions, during the period commencing on the date the Company has (x) received at least $100.0 million in gross cash proceeds from qualified financing transactions by December 20, 2024 or at least $125.0 million by June 30, 2025 and (y) completed 70 drug product deliveries within a given six-month period ending no later than December 31, 2025, at least 40 of which are for LYFGENIA (the “Tranche 3 Milestone”) and ending on the earlier of (i) the date that is 30 days immediately following the date the Company achieves the Tranche 3 Milestone and (ii) December 31, 2025; and (d) an additional tranche of term loans of $50.0 million, available in the sole discretion of the lenders, and subject to customary terms and conditions, until December 15, 2026. Although our entry into the LSA and receipt of funds thereunder extends our cash runway, our outstanding indebtedness, including any additional indebtedness beyond our borrowings under the LSA, combined with our other financial obligations and contractual commitments could have significant adverse consequences, including:

- requiring us to dedicate a portion of our cash resources to the payment of interest and principal, reducing money available to fund working capital, capital expenditures, product candidate development and other general corporate purposes;
- increasing our vulnerability to adverse changes in general economic, industry and market conditions;
- subjecting us to restrictive covenants that may reduce our ability to take certain corporate actions or obtain further debt or equity financing;
- limiting our flexibility in planning for, or reacting to, changes in our business and the industry in which we compete; and
- placing us at a competitive disadvantage compared to our competitors that have less debt or better debt servicing options.

The Term Loans are secured by a lien on substantially all of our assets. We intend to satisfy our current and future debt service obligations with our then-existing cash and cash equivalents. However, we may not have sufficient funds, and may be unable to arrange for additional financing, to pay the amounts due under the LSA or any other debt instruments. Failure to make payments or comply with other covenants under the LSA or such other debt instruments could result in an event of default and acceleration of amounts due. Other events of default under the LSA include, among others: (i) the occurrence of any event that the Lender interprets as a material adverse effect (including potentially with respect to our declining cash position or negative data results), (ii) a change in control as delineated under the Loan Agreement, and (iii) breaches of covenants in the LSA, including, among others, a minimum liquidity requirement and a covenant that requires us to meet certain revenue levels; if we do not meet our projections, we may be unable to satisfy these covenants. Upon the occurrence and continuance of an event of default, the Lender has the right to require us to repay the Term Loans immediately, which we would be unable to do given our current cash position. Any declaration by the Lender of an event of default would significantly harm our business and prospects and could cause the price of our common stock to decline or force us to discontinue our operations immediately. If we raise any additional debt financing, the terms of such additional debt could further restrict our operating and financial flexibility.

Amounts under our factoring arrangement are subject to terms that may adversely affect our operations and financial condition.

We entered into an accounts receivable factoring agreement in December 2023. The factoring agreement provides for us to have access to up to $100.0 million on a revolving basis, measured by the outstanding balance of purchased accounts from time to time. Upon receipt of the upfront purchase price for any purchased accounts, we will have sold and assigned all of our rights in such purchased accounts and all proceeds thereof. The buyer has the right to require that we repurchase any purchased account that was ineligible as of the date of purchase or with respect to which any account debtor asserts a dispute that is not resolved by the related due date. The buyer does not have recourse to us for the insolvency or other credit risk of the account debtors. We have granted the buyer a security interest in the purchased accounts, and proceeds thereof, as more fully described in the agreement, in order to perfect the buyer's ownership interest in the purchased accounts and secure the payment and performance of all our obligations to the buyer under the agreement. If the buyer demands repurchase and we fail to do so, or if we cause or permit any other event of default as defined in the agreement, or fail to comply with covenants set forth in the agreement, we would be subject to additional expenses and lose access to this agreement to fund further accounts receivable. Such results could have a material adverse effect on our operations and financial condition.

Risks related to our business operations

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

We are highly dependent on our executive team and key employees, 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, consultants and advisors 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 our turnover rate has been 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, our financial condition has made it more challenging to recruit and retain qualified personnel. The inability to recruit or loss of the services of any executive, key employee, consultant or advisor may impede the progress of our research, development and commercialization objectives.

Our restructuring and reduction in force undertaken to optimize our cost structure may not achieve our intended outcome.

In September 2024, we implemented a restructuring plan designed to support our commercial focus and reduce our cash operating expenses. This restructuring plan included a reduction of our workforce by approximately 25% of our headcount. These reductions in force may result in unintended consequences and costs, such as the loss of institutional knowledge and expertise, attrition beyond the intended number of employees, decreased morale among our remaining employees, and the risk that we may not achieve the anticipated benefits of the reduction in force. In addition, while positions have been eliminated, certain functions necessary to our operations remain, and we may be unsuccessful in distributing the duties and obligations of departed employees among our remaining employees. The reduction in workforce could also make it difficult for us to pursue, or prevent us from pursuing, new opportunities and initiatives due to insufficient personnel, or require us to incur additional and unanticipated costs to hire new personnel to pursue such opportunities or initiatives. If we are unable to realize the anticipated benefits from the reductions in force, or if we experience significant adverse consequences from the reductions in force, our business, financial condition, and results of operations may be materially adversely affected. We may undertake further similar cost-saving initiatives, which may include additional restructuring or workforce reductions. These types of cost-reduction activities can be complex and result in unintended consequences and costs, including further attrition beyond the intended number of employees due to decreased employee morale, loss of institutional knowledge and expertise and adversely impact our business.

Our products remain subject to regulatory scrutiny.

For any regulatory approvals that we have or may receive, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for our products and/or any future product candidates will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as ongoing compliance with cGMPs and GCPs for any clinical trials that we may conduct. In addition, manufacturers of drug products and their facilities are subject to continual review and periodic, unannounced inspections by the FDA and other regulatory authorities for compliance with cGMP regulations and standards. Even though we have obtained regulatory approval in the U.S. for ZYNTEGLO, SKYSONA and LYFGENIA, any regulatory approvals we receive will require the submission of reports to regulatory authorities and surveillance to monitor the safety and efficacy of such product, and such approvals may contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, and may include burdensome post-approval study or risk management requirements. For example, the FDA typically advises that patients treated with integrating gene therapy undergo follow-up observations for potential adverse events for a 15-year period. Furthermore, we have agreed to provide confirmatory long-term clinical data to the FDA as a condition of the SKYSONA accelerated approval, and continued approval for the approved indication will be contingent upon verification and description of clinical benefit in a confirmatory trial. If our confirmatory trials fail to adequately verify or describe the anticipated clinical benefit of SKYSONA, or if we fail to conduct such trials in a timely manner, the FDA could withdraw its approval for SKYSONA on an expedited basis.

Additionally, the holder of an approved BLA is obligated to monitor and report adverse events. 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. We have experienced interruptions in clinical programs due to safety concerns arising from our SKYSONA and LYFGENIA programs, and we can make no assurance that we will not experience interruptions in any clinical studies, marketing or other commercialization activities in the future, whether due to safety concerns in any approved or investigational products, or due to events arising from programs that utilize technologies similar to or related to ours.

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 ("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 marketing approval for a product, 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 marketing approval;
- suspend any ongoing clinical studies;
- refuse to approve a pending marketing application, such as a 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 any approved product and generate revenues.

The FDA's and other regulatory authorities' policies may change and additional government regulations may be promulgated that could prevent, limit or delay marketing authorization of any future product candidates we develop. We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may be subject to enforcement action and we may not achieve or sustain profitability.

The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses.

The FDA strictly regulates marketing, labeling, advertising and promotion of prescription drugs and biologics. These regulations include standards and restrictions for direct-to-consumer advertising, industry-sponsored scientific and educational activities, promotional activities involving the internet and off-label promotion. Any regulatory approval that the FDA grants is limited to those specific diseases and indications for which a product is deemed to be safe, pure and potent, or effective, by the FDA. For example, the current FDA-approved indication for ZYNTEGLO is limited to the treatment of adult and pediatric patients with β-thalassemia who require regular red blood cell transfusions; the FDA-approved indication for SKYSONA is limited to slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active CALD, which is defined to include to asymptomatic or mildly symptomatic (neurologic function score, NFS ≤ 1) boys who have gadolinium enhancement on brain magnetic resonance imaging and Loes scores of 0.5-9; and the FDA-approved indication for LYFGENIA is limited to the treatment of sickle cell disease in patients ages 12 and older who have a history of VOEs.

While physicians in the United States may choose, and are generally permitted, to prescribe drugs for uses that are not described in the product's labeling and for uses that differ from those tested in clinical trials and approved by the regulatory authorities, our ability to manufacture and promote any products will be narrowly limited to those indications that are specifically approved by the FDA. If we are found to have manufactured and promoted such off-label uses, we may become subject to significant liability. The U.S. federal government has levied large civil and criminal fines against companies for alleged improper promotion of off-label use and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed. If we cannot successfully manage the promotion of any of our products, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

We are subject, directly or indirectly, to federal and state healthcare fraud and abuse laws and false claims laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties, reputational harm, and diminished profits and future earnings.

In the United States, the research, manufacturing, distribution, sale, and promotion of drugs and biologic products are subject to regulation by various federal, state, and local authorities in addition to FDA, including CMS, other divisions of the HHS, (e.g., the Office of Inspector General), the United States Department of Justice offices of the United States Attorney, the Federal Trade Commission and state and local governments. Our operations are directly, or indirectly through our prescribers, customers and purchasers, subject to various federal and state fraud and abuse laws and regulations.

These laws apply to, among other things, our sales, marketing, patient services and educational programs and include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, either the referral of an individual, or the purchase, lease, order or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under the Medicare and Medicaid programs or other federal healthcare programs. A person or entity can be found guilty of violating the statute without actual knowledge of the statute or specific intent to violate it. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers, and formulary managers on the other. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution;

- the federal civil and criminal false claims laws, including the False Claims Act, or FCA, and civil monetary penalty laws, which prohibit any person or entity from, among other things, knowingly presenting, or causing to be presented, a false, fictitious or fraudulent claim for payment to, or approval by, the federal government or knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government. In addition, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the FCA;

- HIPAA, which created federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payer (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity can be found guilty of violating HIPAA without actual knowledge of the statutes or specific intent to violate them;

- the Physician Payments Sunshine Act, created under the ACA, and its implementing regulations, which require manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to CMS information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain non-physician practitioners (physician assistants, nurse practitioners, clinical nurse specialists, certified nurse anesthetists, anesthesiologist assistants and certified nurse midwives), and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;

- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers; and

- analogous or related foreign, state or local laws and regulations, including anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-

governmental third-party payers, including private insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; and state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures.

State and federal regulatory and enforcement agencies continue actively to investigate violations of health care laws and regulations, and the United States Congress continues to strengthen the arsenal of enforcement tools. Most recently, the Bipartisan Budget Act of 2018 increased the criminal and civil penalties that can be imposed for violating certain federal health care laws, including the Anti-Kickback Statute. Enforcement agencies also continue to pursue novel theories of liability under these laws. In particular, government agencies have recently increased regulatory scrutiny and enforcement activity with respect to programs supported or sponsored by pharmaceutical companies, including reimbursement and co-pay support, funding of independent charitable foundations and other programs that offer benefits for patients. Several investigations into these programs have resulted in significant civil and criminal settlements. In addition, in July 2024, the Office of Inspector General (OIG) issued two negative opinions to pharmaceutical companies seeking to offer fertility support for gene therapy patients insured by Medicaid and other federal healthcare programs. OIG stated that it lacked data to conclude that the fertility support programs would pose a sufficiently low risk of fraud and abuse under the federal Anti-Kickback Statute to grant prospective immunity.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our financial condition and divert the attention of our management from operating our business.

Actual or perceived failures to comply with applicable data protection, privacy and security laws, regulations, standards and other requirements could adversely affect our business, results of operations, and financial condition.

The global data protection landscape is rapidly evolving, and we are or may become subject to numerous state, federal and foreign laws, requirements and regulations governing the collection, use, disclosure, retention, and security of personal information, such as information that we may collect in connection with clinical trials. Implementation standards and enforcement practices are likely to remain uncertain for the foreseeable future, and we cannot yet determine the impact future laws, regulations, standards, or perception of their requirements may have on our business. This evolution may create uncertainty in our business, affect our ability to operate in certain jurisdictions or to collect, store, transfer use and share personal information, necessitate the acceptance of more onerous obligations in our contracts, result in liability or impose additional costs on us. The cost of compliance with these laws, regulations and standards is high and is likely to increase in the future. Any failure or perceived failure by us to comply with federal, state or foreign laws or regulations, our internal policies and procedures or our contracts governing our processing of personal information could result in negative publicity, government investigations and enforcement actions, claims by third parties and damage to our reputation, any of which could have a material adverse effect on our business, results of operation, and financial condition.

In the U.S., HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 and their implementing regulations (collectively, "HIPAA"), imposes requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information. In addition, we may obtain health information from third parties (including research institutions from which we obtain clinical trial data), which are subject to privacy and security requirements under HIPAA. Depending on the facts and circumstances, we could be subject to significant penalties if we violate HIPAA. Certain states have also adopted comparable privacy and security laws and regulations, which govern the privacy, processing and protection of health-related and other personal information. For example, California enacted the California Consumer Privacy Act ("CCPA") which creates new individual privacy rights for California consumers (as defined in the law) and places increased privacy and security obligations on entities handling personal data of consumers or households. The CCPA requires covered companies to provide certain disclosures to consumers about its data collection, use and sharing practices, and to

provide affected California residents with ways to opt-out of certain sales or transfers of personal information. While there is currently an exception for certain clinical trial data, as currently written, the CCPA may impact our business activities. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that has increased the likelihood of, and risks associated with data breach litigation. Further, the California Privacy Rights Act ("CPRA") generally went into effect on January 1, 2023, and significantly amends the CCPA. It imposes additional data protection obligations on covered businesses, including additional consumer rights processes, limitations on data uses, new audit requirements for higher risk data, and opt outs for certain uses of sensitive data. It also creates a new California data protection agency authorized to issue substantive regulations and could result in increased privacy and information security enforcement. Additional compliance investment and potential business process changes may also be required. Similar laws have passed in other states and are continuing to be proposed at the state and federal level, reflecting a trend toward more stringent privacy legislation in the United States. The enactment of such laws could have potentially conflicting requirements that would make compliance challenging. In the event that we are subject to or affected by HIPAA, the CCPA, the CPRA or other domestic privacy and data protection laws, any liability from failure to comply with the requirements of these laws could adversely affect our financial condition.

Furthermore, the Federal Trade Commission ("FTC") has authority to initiate enforcement actions against entities that make deceptive statements about privacy and data sharing in privacy policies, fail to limit third-party use of personal health information, fail to implement policies to protect personal health information or engage in other unfair practices that harm customers or that may violate Section 5(a) of the FTC Act. For example, according to the FTC, failing to take appropriate steps to keep consumers' personal information secure can constitute unfair acts or practices in or affecting commerce in violation of Section 5(a) of the Federal Trade Commission Act. The FTC expects a company's data security measures to be reasonable and appropriate in light of the sensitivity and volume of consumer information it holds, the size and complexity of its business, and the cost of available tools to improve security and reduce vulnerabilities. The FTC and many state Attorneys General continue to enforce federal and state consumer protection laws against companies for online collection, use, dissemination and security practices that appear to be unfair or deceptive, including by. regulating the presentation of website content.

We are also or may become subject to rapidly evolving data protection laws, rules and regulations in foreign jurisdictions. For example, in Europe, the European Union General Data Protection Regulation ("GDPR") went into effect in May 2018 and imposes strict requirements for processing the personal data of individuals within the European Economic Area ("EEA"). Companies that must comply with the GDPR face increased compliance obligations and risk, including more robust regulatory enforcement of data protection requirements and potential fines for noncompliance of up to €20 million or 4% of the annual global revenues of the noncompliant company, whichever is greater. Among other requirements, the GDPR regulates transfers of personal data subject to the GDPR to third countries that have not been found to provide adequate protection to such personal data, including the United States, and the efficacy and longevity of current transfer mechanisms between the EEA, and the United States remains uncertain. Case law from the Court of Justice of the European Union ("CJEU") states that reliance on the standard contractual clauses ("SCCs") - a standard form of contract approved by the European Commission as an adequate personal data transfer mechanism - alone may not necessarily be sufficient in all circumstances and that transfers must be assessed on a case-by-case basis. On July 10, 2023, the European Commission adopted its Adequacy Decision in relation to the new EU-US Data Privacy Framework ("DPF"), rendering the DPF effective as a GDPR transfer mechanism to U.S. entities self-certified under the DPF. We expect the existing legal complexity and uncertainty regarding international personal data transfers to continue. As supervisory authorities issue further guidance on personal data export mechanisms, including circumstances where the SCCs cannot be used, and/or start taking enforcement action, we could suffer additional costs, complaints and/or regulatory investigations or fines, and/or if we are otherwise unable to transfer personal data between and among countries and regions in which we operate, it could affect the manner in which we provide our services, the geographical location or segregation of our relevant systems and operations, and could adversely affect our financial results.

Since the beginning of 2021, after the end of the transition period following the United Kingdom's departure from the European Union, we are also subject to the United Kingdom data protection regime, which imposes separate but similar obligations to those under the GDPR and comparable penalties, including fines of up to £17.5 million or 4% of a noncompliant company's global annual revenue for the preceding financial year, whichever is greater. On October 12, 2023, the United Kingdom Extension to the DPF came into effect (as approved by the United Kingdom Government), as a data transfer mechanism from the United Kingdom to U.S. entities self-certified under the DPF. As we continue to expand into other foreign countries and jurisdictions, we may be subject to additional laws and regulations that may affect how we conduct business.

If we fail to comply with reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs, we could be subject to additional reimbursement requirements, penalties, sanctions and

fines, which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

We participate in governmental programs that impose extensive drug price reporting and payment obligations on pharmaceutical manufacturers. Medicaid is a joint federal and state program that is administered by the states for low income and disabled beneficiaries. Under the Medicaid Drug Rebate Program (the "MDRP"), as a condition of federal funds being made available for our covered outpatient drugs under Medicaid and certain drugs or biologicals under Medicare Part B, we pay a rebate to state Medicaid programs for each unit of our covered outpatient drugs dispensed to a Medicaid beneficiary and paid for by the state Medicaid program. Medicaid rebates are based on pricing data that we report on a monthly and quarterly basis to CMS, the federal agency that administers the MDRP and Medicare programs. For the MDRP, these data include the Average Manufacturer Price ("AMP") for each drug and, in the case of innovator products, best price. In connection with Medicare Part B, a pharmaceutical manufacturer must provide CMS with average sales price ("ASP") information for certain drugs or biologicals on a quarterly basis. ASP is calculated based on a statutorily defined formula, as well as regulations and interpretations of the statute by CMS. If we become aware that our MDRP price reporting submission for a prior period was incorrect or has changed as a result of recalculation of the pricing data, we must resubmit the corrected data for up to three years after those data originally were due. If we fail to provide information on a timely basis or are found to have knowingly submitted false information to the government, we may be subject to civil monetary penalties and other sanctions, including termination from the MDRP, in which case payment would not be available for our covered outpatient drugs under Medicaid or, if applicable, Medicare Part B.

Federal law requires that any company that participates in the MDRP also participate in the 340B program in order for federal funds to be available for the manufacturer's drugs under Medicaid and Medicare Part B. The 340B program is administered by HRSA and requires us, as a participating manufacturer, to agree to charge statutorily defined covered entities no more than the 340B "ceiling price" for our covered outpatient drugs when used in an outpatient setting.. To date, bluebird's therapies have been administered in the inpatient setting exclusively and we anticipate that most patients will continue to receive bluebird's therapies in an inpatient setting. However, in the event that patients are treated in an outpatient setting, the 340B "ceiling price" requirement may apply to these transactions if otherwise eligible under 340B legal standards. These 340B covered entities include a variety of community health clinics and other entities that receive health services grants from the Public Health Service, as well as hospitals that serve a disproportionate share of low income patients. A drug that is designated for a rare disease or condition by the Secretary of Health and Human Services is not subject to the 340B ceiling price requirement with regard to the following types of covered entities: rural referral centers, sole community hospitals, critical access hospitals, and free standing cancer hospitals. The 340B ceiling price is calculated using a statutory formula, which is based on the AMP and rebate amount for the covered outpatient drug as calculated under the MDRP. In general, products subject to Medicaid price reporting and rebate liability are also subject to the 340B ceiling price calculation and discount requirement. We must report 340B ceiling prices to the HRSA on a quarterly basis, and HRSA publishes them to 340B covered entities. HRSA has finalized regulations regarding the calculation of the 340B ceiling price and the imposition of civil monetary penalties on manufacturers that knowingly and intentionally overcharge covered entities for 340B eligible drugs. HRSA has also finalized a revised administrative dispute resolution process through which 340B covered entities may pursue claims against participating manufacturers for overcharges, and through which manufacturers may pursue claims against 340B covered entities for engaging in unlawful diversion or duplicate discounting of 340B drugs. In addition, legislation may be introduced that, if enacted, would further expand the 340B program, such as adding further covered entities or requiring participating manufacturers to agree to provide 340B discounted pricing on drugs used in an inpatient setting.

In order to be eligible to have drug products paid for with federal funds under Medicaid and Medicare Part B and purchased by certain federal agencies and grantees, we must also participate in the VA/FSS pricing program. Under the VA/FSS program, we must report the Non-FAMP for our covered drugs to the VA and charge certain federal agencies no more than the Federal Ceiling Price, which is calculated based on Non FAMP using a statutory formula. These four agencies are the VA, the U.S. Department of Defense, the U.S. Coast Guard, and the U.S. Public Health Service (including the Indian Health Service). We must also pay rebates on products purchased by military personnel and dependents through the TRICARE retail pharmacy program. If we fail to provide timely information or are found to have knowingly submitted false information, we may be subject to civil monetary penalties.

Individual states continue to consider and have enacted legislation to limit the growth of healthcare costs, including the cost of prescription drugs and combination products. A number of states have either implemented or are considering implementation of drug price transparency legislation that may prevent or limit our ability to take price increases at certain rates or frequencies. Requirements under such laws include advance notice of planned price increases, reporting price increase amounts and factors considered in taking such increases, wholesale acquisition cost information disclosure to prescribers, purchasers, and state agencies, and new product notice and reporting. Such legislation could limit the price or payment for certain drugs, and states may impose civil monetary penalties or pursue other enforcement mechanisms against manufacturers who fail to comply with

drug price transparency requirements. If we are found to have violated state law requirements, we may become subject to penalties or other enforcement mechanisms, which could have a material adverse effect on our business.

Pricing and rebate calculations vary across products and programs, are complex, and are often subject to interpretation by us, governmental or regulatory agencies, and the courts, which can change and evolve over time. Such pricing calculations and reporting, along with any necessary restatements and recalculations, could increase our costs for complying with the laws and regulations governing the MDRP and other governmental programs, and under the MDRP could result in an overage or undercharge in Medicaid rebate liability for past quarters. Price recalculations under the MDRP also may affect the ceiling price at which we are required to offer products under the 340B program. Civil monetary penalties can be applied if we are found to have knowingly submitted any false price or product information to the government, if we fail to submit the required price data on a timely basis, or if we are found to have charged 340B covered entities more than the statutorily mandated ceiling price. CMS could also terminate our Medicaid drug rebate agreement, in which case federal payments may not be available under Medicaid or Medicare Part B, if applicable, for our covered outpatient drugs. Pursuant to the Inflation Reduction Act of 2022 (the "IRA"), the AMP figures we report will also be used to compute rebates under Medicare Part D triggered by price increases that outpace inflation. We cannot assure you that our submissions will not be found to be incomplete or incorrect.

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 products or product candidates harm patients, or is perceived to harm patients even when such harm is unrelated to our products or product candidates, our marketing approvals could be revoked or otherwise negatively impacted and we could be subject to costly and damaging product liability claims.

The use of products and product candidates in clinical studies and the sale of products for which we have obtained marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by patients participating in clinical trials, consumers, healthcare providers, pharmaceutical companies or others selling or otherwise coming into contact with our products and any future product candidates. There is a risk that our products and any future product candidates may induce adverse events. For instance, each of the LYFGENIA and SKYSONA product labels includes a boxed warning for the risk of hematologic malignancy. 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;
- substantial monetary awards to patients or other claimants;
- the inability to develop our product candidates or commercialize any approved product; and
- decreased demand for any approved product.

We carry product liability insurance and we believe our product liability insurance coverage is sufficient in light of our current clinical programs and approved products; however, we may not be able to maintain insurance coverage at commercially reasonable cost or in sufficient amounts to protect us against losses due to liability. 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 products and 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 products and 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 marketing approval for any approved product, 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 and product candidates the investigation into the circumstance may be time-consuming or inconclusive. These investigations may interrupt our sales efforts, delay our marketing approval process in other countries, or impact and limit the type of marketing approval our product candidates may receive or our approved products 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.

The increasing focus on environmental sustainability and social initiatives could increase our costs, harm our reputation and adversely impact our financial results.

There has been increasing public focus by investors, patients, environmental activists, the media and governmental and nongovernmental organizations on a variety of environmental, social and other sustainability matters. We may experience pressure to make commitments relating to sustainability matters that affect us, including the design and implementation of specific risk mitigation strategic initiatives relating to sustainability. If we are not effective in addressing environmental, social and other sustainability matters affecting our business, or setting and meeting relevant sustainability goals, our reputation and financial results may suffer. In addition, even if we are effective at addressing such concerns, we may experience increased costs as a result of executing upon our sustainability goals that may not be offset by any benefit to our reputation, which could have an adverse impact on our business and financial condition.

In addition, this emphasis on environmental and social matters has resulted in the adoption of new laws and regulations, including new reporting requirements, and may result in the adoption of additional laws and regulations in the future. New reporting requirements may be particularly difficult or expensive to comply with and, if we fail to comply, we may be required to issue financial restatements, suffer harm to our reputation or otherwise have our business be adversely impacted. Such ESG matters may also impact our suppliers or patients, which may adversely impact our business, financial condition and results of operations.

In addition, this emphasis on environmental, social and other sustainability matters has resulted and may result in the adoption of new laws and regulations, including new reporting requirements. If we fail to comply with new laws, regulations or reporting requirements, our reputation and business could be adversely impacted.

Healthcare legislative reform measures may have a material adverse effect on our business and results of operations.

The United States has enacted or proposed legislative and regulatory changes affecting the healthcare system that could prevent or delay marketing approval of any future product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell products for which we have obtained marketing approval. Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; (iv) additional record-keeping requirements; or (v) directly or indirectly limit the net price of sales to federal healthcare programs that form a substantial portion of our business. If any such changes were to be imposed, they could adversely affect the operation of our business.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and other third-party payers have attempted to control costs by limiting coverage and the level of reimbursement for particular medical products and services, implementing reductions in Medicare and other healthcare funding and applying new payment methodologies. For example, in March 2010, the Affordable Care Act ("ACA") was enacted, which, among other things, increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program; introduced a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected; extended manufacturer Medicaid rebate obligations to utilization by individuals enrolled in Medicaid managed care plans; established a Medicare Part D coverage gap discount program (to be replaced by a new program in 2025, as discussed below); subjected drug manufacturers to new annual fees based on pharmaceutical companies' share of sales to federal healthcare programs; imposed a new federal excise tax on the sale of certain medical devices; created a new Patient Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; and established a Center for Medicare and Medicaid Innovation at the CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending.

Since its enactment, there have been judicial, executive and Congressional challenges to certain aspects of the ACA. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted. The Budget Control Act of 2011, among other things, led to

reductions of Medicare payments to providers, which will remain in effect through 2032 unless additional Congressional action is taken. More recently, in March 2021, President Biden signed into law the American Rescue Plan Act of 2021, which eliminated the statutory cap on the Medicaid drug rebate beginning January 1, 2024. The rebate was previously capped at 100% of a drug's AMP.

Most significantly, in August 2022, President Biden signed the Inflation Reduction Act of 2022 ("IRA") into law. This statute marks the most significant action by Congress with respect to the pharmaceutical industry since adoption of the ACA in 2010. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare, with prices that can be negotiated subject to a cap (with resulting prices for the initial ten drugs first effective in 2026); imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023); redesigns the Medicare Part D benefit (beginning in 2024); and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the Department of Health and Human Services (HHS) to implement many of these provisions through guidance, as opposed to regulation, for the initial years. On August 29, 2023, HHS announced the list of the first ten drugs that will be subject to price negotiations. HHS has issued and will continue to issue and update guidance implementing the IRA, although the Medicare drug price negotiation program is currently subject to legal challenges. While the impact of the IRA on the pharmaceutical industry cannot yet be fully determined, it is likely to be significant.

In addition, the Center for Medicare and Medicaid Innovation initiated the Cell and Gene Therapy ("CGT") Access Model in 2023. This voluntary payment model is designed to test whether a CMS-led approach to developing and administering outcomes-based agreements (OBAs) for cell and gene therapies would improve Medicaid beneficiaries' access to innovative treatment. If CMS proceeds with implementing the CGT model as currently anticipated, states may begin to participate in the model in 2025. The possible impact of the CGT model is uncertain.

At the U.S. state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or provider reimbursement constraints, patient out-of-pocket cost caps for certain classes of therapy, discounts, restrictions on certain product access, marketing cost disclosure and other transparency measures, and, in some cases, measures designed to encourage importation from other countries and bulk purchasing.

We expect that the healthcare reform measures that have been adopted and may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product and could seriously harm our future revenues. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private third-party payers.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products and any future product candidates. Such reforms could have an adverse effect on anticipated revenue from products and any future product candidates that we may successfully develop and for which we may obtain marketing approval and may affect our overall financial condition and ability to develop such future product candidates.

Our information technology systems, or those of our third-party collaborators, service providers, contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of any future product candidates' development programs and activities related to our approved products and have a material adverse effect on our reputation, business, financial condition or results of operations.

We collect and maintain information in digital form that is necessary to conduct our business, and we are increasingly dependent on information technology systems and infrastructure to operate our business, including our mobile and web-based applications, our e-commerce platform and our enterprise software. In the ordinary course of our business, we collect, store and transmit large amounts of confidential information, including intellectual property, proprietary business information, clinical trial data, and personal information (collectively, "Confidential Information") of customers and our employees and contractors. It is critical that we do so in a secure manner to maintain the confidentiality and integrity of such Confidential Information.

Our information technology systems and those of our current or future third-party collaborators, service providers, contractors and consultants may fail and are vulnerable to attack, damage and interruption from computer viruses and malware (e.g. ransomware), misconfigurations, "bugs" or other vulnerabilities, malicious code, natural disasters, terrorism, war, telecommunication and electrical failures, hacking, cyberattacks, phishing attacks and other social engineering schemes, employee theft or misuse, human error, fraud, denial or degradation of service attacks, sophisticated nation-state and nation-

state-supported actors or unauthorized access or use by persons inside our organizations, or persons with access to systems inside our organization. Attacks on information technology systems are increasing in their frequency, levels of persistence, sophistication and intensity, and they are being conducted by increasingly sophisticated and organized groups and individuals with a wide range of motives and expertise. The prevalent use of mobile devices and unauthorized applications also increases the risk of data security incidents. As a result of the continued hybrid working environment, we may also face increased cybersecurity risks due to our reliance on internet technology and the number of our employees who are working remotely, which may create additional opportunities for cybercriminals to exploit vulnerabilities. Furthermore, because the techniques used to obtain unauthorized access to, or to sabotage, systems change frequently and often are not recognized until launched against a target, we may be unable to anticipate these techniques or implement adequate preventative measures. We may also experience security breaches that may remain undetected for an extended period. Even if identified, we may be unable to adequately investigate or remediate incidents or breaches due to attackers increasingly using tools and techniques that are designed to circumvent controls, to avoid detection, and to remove or obfuscate forensic evidence. There can also be no assurance that our and our current or future third-party collaborators', service providers', contractors' and consultants' cybersecurity risk management program and processes, including policies, controls or procedures, will be fully implemented, complied with or effective in protecting our systems, networks and Confidential Information.

We and certain of our service providers are from time to time subject to cyberattacks and security incidents. While we do not believe that we have experienced any significant system failure, accident or security breach to date, if we were to experience a system failure, accident or security breach that causes interruptions in our operations or the operations of third-party collaborators, service providers, contractors and consultants, it could result in significant reputational, financial, legal, regulatory, business or operational harm. For example, the loss of clinical trial data for any future product candidates could result in delays in our marketing approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications or other data or applications relating to our technology or our products and any future product candidates, or inappropriate disclosure of Confidential Information, we could incur liabilities and the further development of any future product candidates could be delayed. In addition, we rely on third-party service providers for management of the manufacture and delivery of drug product to patients in the commercial context, including for chain of identity and chain of custody. We also rely on third-party service providers for aspects of our internal control over financial reporting and such service providers may experience a material system failure or fail to carry out their obligations in other respects, which may impact our ability to produce accurate and timely financial statements, thus harming our operating results, our ability to operate our business, and our investors' view of us. In addition, our liability insurance may not be sufficient in type or amount to cover us against claims related to material failures, security breaches, cyberattacks and other related breaches.

Any failure or perceived failure by us or any third-party collaborators, service providers, contractors or consultants to comply with our privacy, confidentiality, data security or similar obligations to third parties, or any data security incidents or other security breaches that result in the unauthorized access, release or transfer of sensitive information, including personally identifiable information, may result in governmental investigations, enforcement actions, regulatory fines, litigation or public statements against us. These events could cause third parties to lose trust in us or could result in claims by third parties asserting that we have breached our privacy, confidentiality, data security or similar obligations, any of which could have a material adverse effect on our reputation, business, financial condition or results of operations. While we have implemented data security measures intended to protect our information technology systems and infrastructure, there can be no assurance that such measures will successfully prevent service interruptions or data security incidents. Further, our insurance coverage may not be sufficient to cover the financial, legal, business or reputational losses that may result from an interruption or breach of our systems.

Risks related to the separation of our oncology programs and portfolio

We may incur operational difficulties or be exposed to claims and liabilities as a result of the separation of 2seventy bio.

On November 4, 2021, we distributed all of the outstanding shares of 2seventy bio, Inc. ("2seventy") common stock to our stockholders in connection with the separation of our oncology programs and portfolio. In connection with the distribution, we entered into a separation agreement and various other agreements (including a tax matters agreement, an employee matters agreement, transition services agreements and an intellectual property license agreement). These agreements govern the separation and distribution and the relationship between us and 2seventy going forward, including with respect to the assignment and assumption of assets and liabilities and potential tax-related losses associated with the separation and distribution. They also provide for the performance of services by each company for the benefit of the other for a period of time.

As a result of the separation, we remain contractually liable in connection with certain agreements transferred to 2seventy; for instance, we may be liable in the event of a breach by 2seventy of an assigned lease agreement, which could result in material expenses. Although the separation agreement provides for indemnification obligations designed to make 2seventy financially responsible for many liabilities that may exist relating to its business activities, whether incurred prior to or after the distribution, including any pending or future litigation, we cannot guarantee that 2seventy will be able to satisfy its indemnification obligations, including as related to the lease agreement. It is also possible that a court would disregard the allocation agreed to between us and 2seventy and require us to assume responsibility for obligations allocated to 2seventy. Third parties could also seek to hold us responsible for any of these liabilities or obligations, and the indemnity rights we have under the separation agreement may not be sufficient to fully cover all of these liabilities and obligations. Even if we are successful in obtaining indemnification, we may have to bear costs temporarily. In addition, our indemnity obligations to 2seventy, including those related to assets or liabilities allocated to us, may be significant. These risks could negatively affect our business, financial condition or results of operations.

If the distribution of shares of 2seventy, together with certain related transactions, does not qualify as a transaction that is generally tax-free for U.S. federal income tax purposes, we and our stockholders could be subject to significant tax liabilities.

The completion of the distribution of shares of 2seventy was conditioned upon, among other things, our receipt of a private letter ruling from the U.S. Internal Revenue Service (the "IRS"), and an opinion from Goodwin Procter LLP, both satisfactory to our board of directors and both continuing to be valid, together confirming that the distribution, together with certain related transactions, generally is tax-free for U.S. federal income tax purposes under Sections 355 and 368(a)(1)(D) of the U.S. Internal Revenue Code of 1986, as amended (the "Code"). We have received a favorable private letter ruling from the IRS addressing one significant issue of the qualification of the distribution under Section 355 of the Code. However, the private letter ruling does not address the remaining issues that are relevant to determining whether the distribution, together with certain related transactions, qualifies as a transaction that is generally tax-free for U.S. federal income tax purposes. This can include events that occur following the distribution such as subsequent public offerings by us or 2seventy or share sales to persons that engaged in negotiations over share purchases prior to the distribution. Subsequent tax opinions have been obtained by us and 2seventy in connection with certain post-distribution sales of 2seventy's shares. The IRS private letter ruling, the opinion of Goodwin Procter LLP and tax opinions related to certain subsequent post-distribution sales of 2seventy shares were based, among other things, on various facts and assumptions, as well as certain representations, statements and undertakings from us and 2seventy (including those relating to the past and future conduct of us and 2seventy) and were subject to certain caveats. If any of these facts, assumptions, representations, statements or undertakings is, or becomes, inaccurate or incomplete, or if we or 2seventy breach any of our respective covenants relating to the separation, the IRS private letter ruling and tax opinion may be invalid. Moreover, the opinion is not binding on the IRS or any courts. Accordingly, notwithstanding receipt of the IRS private letter ruling and an opinion of Goodwin Procter LLP at the time of the distribution, the IRS could determine that the distribution and certain related transactions should be treated as taxable transactions for U.S. federal income tax purposes.

If the distribution, together with certain related transactions, were to fail to qualify as a transaction that is generally tax-free under Sections 355 and 368(a)(1)(D) of the Code, in general, for U.S. federal income tax purposes, we would recognize taxable gain as if we have sold 2seventy’s distributed common stock in a taxable sale for its fair market value and our stockholders who receive shares of 2seventy common stock in the distribution would be subject to tax as if they had received a taxable distribution equal to the fair market value of such shares.

In connection with the distribution, we and 2seventy entered into a tax matters agreement pursuant to which each party is responsible for certain liabilities and obligations following the distribution. In general, under the terms of the tax matters agreement, if the distribution, together with certain related transactions, were to fail to qualify as a transaction that is generally tax-free for U.S. federal income tax purposes under Sections 355 and 368(a)(1)(D) of the Code, and if and to the extent that such failure results from a prohibited change of control in us under Section 355(e) of the Code, or an acquisition of our stock or assets or certain actions, omissions or failures to act, by us, then we will bear any resulting taxes, interest, penalties and other costs. If and to the extent that such failure results from a prohibited change of control in 2seventy under Section 355(e) of the Code or an acquisition of 2seventy stock or assets or certain actions by 2seventy, then 2seventy will be obligated to indemnify us for any resulting taxes, interest, penalties and other costs, including any reductions in our net operating loss carryforwards or other tax assets. If such failure does not result from a prohibited change of control in us or 2seventy under Section 355(e) of the Code and both we and 2seventy are responsible for such failure, liability will be shared according to relative fault. If neither we nor 2seventy is responsible for such failure, we will bear any resulting taxes, interest, penalties and other costs.

Risks related to our intellectual property

If we are unable to obtain or protect intellectual property rights related to our products, 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 products. The strength 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 products 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 products, 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 products 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 our products fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our products, it could dissuade companies from collaborating with us to develop product candidates, and threaten our ability to commercialize our current and future products. Several patent applications covering our products 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 future 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 or 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 products 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, and 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.

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, ex parte reexaminations, post-grant review, and inter partes review proceedings before the U.S. Patent and Trademark Office ("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 products may be subject to claims of infringement of the patent rights of third parties.

Third parties have asserted and in the future may assert that we are employing their proprietary technology without authorization. For example, as discussed in Part II, Item 1, "Legal Proceedings", San Rocco Therapeutics, LLC, formerly known as Errant Gene Therapeutics, LLC has alleged that our use of the BB305 lentiviral vector, including in connection with the beti-cel program infringes U.S. Patent Nos. 7,541,179 and 8,058,061, and seeks equitable, injunctive and monetary relief, including royalties, treble damages, attorney fees and costs. 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 products, 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 unless we obtained a license under the applicable patents, or until such patents expire. 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 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 commercialize one or more of our products. 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 attorney's 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 programs 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 manufacture and commercialize our products. Because our programs may involve additional technologies that may require the use of proprietary rights held by third parties, the growth of our business may depend in part on our ability to acquire, in-license or use these proprietary rights. For instance, we may elect to leverage advancements in complementary technologies such as in reduced toxicity conditioning or in stem cell mobilization. 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 clinical 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. Pursuant to an intellectual property license agreement with 2seventy, we granted sublicenses to 2seventy to certain existing license agreements. If we fail to comply with our obligations under these agreements, we or 2seventy materially breach 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.

We may need to obtain licenses from third parties to advance the development of future product candidates or allow commercialization of our products, 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 products or 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 approved products, or future products or product candidates, 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 approved products or 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. 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 patent eligible subject matter, 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 products and product candidates. Such a loss of patent protection would have a material adverse impact on our business.

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.

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 in the future, ownership disputes arising, for example, from conflicting obligations of consultants or others who are involved in developing our products and 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.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our potential 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 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 may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on our products and any future 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 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 price at which you purchase them.

Companies trading in the stock market in general, and The Nasdaq Global Select 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 biotechnology and pharmaceutical industry factors, such as the recent volatility and disruption experienced in the global economy and rising interest and inflation rates, may negatively affect the market price of our common stock, regardless of our actual operating performance.

The market price of our common stock has been volatile in the past, and may continue to be volatile for the foreseeable future. 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, either from patients participating in our clinical trials or in connection with sales of our commercial products or other gene therapy products in the market;
- inability to obtain additional funding;
- failure to successfully manage and sustain the commercial launch of ZYNTEGLO, SKYSONA or LYFGENIA, including failure to manage our supply chain operations in the coordination and delivery of drug product to patients at qualified treatment centers;
- failure to obtain sufficient pricing and reimbursement for ZYNTEGLO, SKYSONA or LYFGENIA from private and governmental payers;
- failure to obtain market acceptance and adoption of ZYNTEGLO, SKYSONA or LYFGENIA;
- 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 ZYNTEGLO, SKYSONA or LYFGENIA, or the inability to do so at acceptable prices;
- adverse regulatory decisions;
- announcements of clinical trial results or progress in the development of programs by our competitors, and the 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;
- global macroeconomic conditions, including as impacted by geopolitical conflicts and war;
- sales of our common stock by us or our stockholders in the future; and
- trading volume of our common stock.

The restatement of our consolidated financial statements for the year ended December 31, 2022 and the quarterly periods in the years ended December 31, 2022 and 2023 has subjected us to a number of additional risks and uncertainties, including increased possibility of legal proceedings.

As discussed elsewhere in this Quarterly Report, our management determined that our consolidated financial statements for the year ended December 31, 2022 and the quarterly periods in the years ended December 31, 2022 and 2023 should be restated due to errors relating to our accounting for lease arrangements, including embedded leases, and the application of our accounting policy for the treatment of non-lease components in lease arrangements, including embedded leases. The restatement of our consolidated financial statements has caused us to incur substantial expenses for legal, accounting, and other professional services and has diverted our management's attention from our business and could continue to do so. As a result of the restatement, we were delayed in filing our Annual Report on Form 10-K and our Quarterly Reports on Form 10-Q for each of the three-month periods ended March 31, 2024 and June 30, 2024. There can be no assurance that we will be able to timely file our required reports for future periods. In addition, as a result of the restatement, investors may lose confidence in our financial reporting, the price of our common stock could decline and we may be subject to litigation or regulatory enforcement actions.

We have identified a material weakness in our internal control over financial reporting and may identify additional material weaknesses in the future or otherwise fail to maintain an effective system of internal controls.

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of a company's annual or interim financial statements will not be prevented or detected on a timely basis. We identified a material weakness in our internal controls related to our accounting for lease arrangements, including embedded leases, and the application of our accounting policy for the treatment of non-lease components in lease agreements, including embedded leases. The material weakness resulted in the restatement of the Company's previously issued consolidated financial statements for the year ended December 31, 2022 and the quarterly periods in the years ended December 31, 2022 and 2023. As a result of the material weakness, our management concluded that our internal control over financial reporting was not effective as of December 31, 2023. Additionally, the material weakness in our internal control over financial reporting has resulted in our management concluding that our disclosure controls and procedures were not effective as of December 31, 2023.

Our management, under the oversight of the Audit Committee of our Board of Directors and in consultation with outside advisors, has begun evaluating and implementing measures designed to remediate the material weakness. Management intends to implement enhancements to its internal control over financial reporting, which are expected to include refinements and enhancements to the complement of personnel, design and operation of its controls related to the accounting for, and identification of, leases. The Company intends to begin to implement these enhancements to the design of its controls during 2024. However, this material weakness will not be considered remediated until management designs and implements effective controls that operate for a sufficient period of time and management has concluded, through testing, that these controls are effective. The Company will monitor the effectiveness of the remediation plan and will refine the remediation plan, as needed. Until remediated, the material weakness could result in future errors to the Company's financial statements.

In addition, we cannot assure you that the measures we are taking will be sufficient to remediate the material weakness or avoid the identification of additional material weaknesses in the future. Our failure to implement and maintain effective internal

control over financial reporting could result in the identification of additional errors in our consolidated financial statements that could result in a further restatement of our financial statements and could cause us to fail to meet our periodic reporting obligations, any of which could diminish investor confidence in us, cause a decline in the price of our common stock and subject us to litigation or regulatory enforcement actions.

There can be no assurance that we will be able to comply with the continued listing standards of Nasdaq.

On April 24, 2024, we received a notification from the listing qualifications department of Nasdaq indicating that as a result of the untimely filing of our Annual Report on Form 10-K for the fiscal year ended December 31, 2023, we were not in compliance with the requirements for continued listing under Listing Rule 5250(c)(1) (the "Filing Listing Rule"), which requires listed companies to timely file all required periodic financial reports with the SEC. On July 15, 2024, Nasdaq granted us a grace period of 180 calendar days from the due date of the Form 10-K, or until October 14, 2024, in which to regain compliance with the Filing Listing Rule. On August 20, 2024, we received additional notifications from Nasdaq with respect to the untimely filing of our Quarterly Reports on Form 10-Q for each of the three-month periods ended March 31, 2024 and June 30, 2024.

Although we have completed our delayed filings, there can be no assurance that we will not receive future notifications regarding noncompliance with any of the requirements for continued listing on Nasdaq.

If we fail to comply with Nasdaq's continued listing requirements, our common stock could be delisted from Nasdaq. The delisting of our common stock from Nasdaq may make it more difficult for us to raise capital on favorable terms in the future. Such a delisting would likely lead to a limited amount of analyst coverage, have a negative effect on the price of our common stock and impair our stockholders' ability to sell or purchase our common stock. In addition, a delisting could cause our stock to be deemed a "penny stock," which would require brokers trading in our common stock to adhere to more stringent rules and possibly result in a reduced level of trading activity in the secondary trading market for our securities.

Actual or potential sales of our common stock by our employees, including our executive officers, pursuant to pre-arranged stock trading plans could cause our stock price to fall or prevent it from increasing for numerous reasons, and actual or potential sales by such persons could be viewed negatively by other investors.

In accordance with the guidelines specified under Rule 10b5-1 of the Securities Exchange Act of 1934, as amended, and our policies regarding stock transactions, a number of our employees, including executive officers and members of our board of directors, have adopted and may continue to adopt stock trading plans pursuant to which they have arranged to sell shares of our common stock from time to time in the future. Generally, sales under such plans by our executive officers and directors require public filings. Actual or potential sales of our common stock by such persons could cause the price of our common stock to fall or prevent it from increasing for numerous reasons.

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, 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 2023 Incentive Award Plan (the "2023 Plan") we are authorized to grant stock options and other equity-based awards to our employees, directors and consultants. The 2023 Plan authorizes the issuance of up to 5.2 million shares. We also make equity grants to certain new employees joining the Company pursuant to an inducement plan, and our compensation committee may elect to increase the number of shares available for future grant under the inducement plan without stockholder approval. We also have an Employee Stock Purchase Plan and any shares of common stock purchased pursuant to that plan will also cause dilution.

We may be subject to litigation, which may result in substantial costs and a diversion of management's attention and resources, which could harm our business.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities, and we have in the past litigated class action complaints in the United States District Court for the District of Massachusetts and for the District of Delaware, filed by purported stockholders against us and certain of our directors and officers. For instance, on March 28, 2024, a class action lawsuit captioned Garry Gill v. bluebird bio, Inc. et al., Case No. 1:24-cv-10803-PBS, was filed against us in the United States District Court for the District of Massachusetts and we may face additional securities class action litigation in the future. This risk is especially relevant for us because biotechnology and pharmaceutical companies have experienced significant stock price volatility in recent years, and we expect to experience continued stock price volatility. Separately, two purported bluebird shareholders, derivatively and purportedly on behalf of bluebird, have each filed a shareholder derivative action in the United States District Court for the District of Massachusetts against our directors and certain members of management alleging, among other things, breaches of their fiduciary duties. Defending against our current and any future litigation could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

We are also a party to litigation and subject to claims incident to the ordinary course of business. For instance, as discussed in Part II, Item 1, "Legal Proceedings", San Rocco Therapeutics, LLC, has filed a complaint against us in the United States District Court for the District of Massachusetts, alleging, among other things, civil violations of the Federal Racketeer Influenced and Corrupt Organizations Act, and antitrust violations under state and federal law and seeking declaratory relief and money damages. Although we believe that these claims have no merit, the outcome of litigation is inherently unpredictable. An adverse result in any litigation could materially harm our financial condition, reputation and business. Regardless of the outcome, litigation can have an adverse impact on us because of defense costs, diversion of management resources and other factors.

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

Under Sections 382 and 383 of the Code, if a corporation undergoes an "ownership change," generally defined as a cumulative change of greater than 50% (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards ("NOLs") and other pre-change tax attributes (such as research and development tax credits) to offset its post-change income and taxes may be limited. We have completed several financings since our inception, which we believe have resulted in shifts in our equity ownership. We completed a study through December 2023 confirming no ownership changes have occurred since our initial public offering in 2013. We may have experienced ownership changes since December 2023, and we may also experience ownership changes in the future as a result of subsequent shifts in our equity ownership, some of which are outside our control. There is a significant likelihood that we will experience an ownership change as a result of future equity offerings, although whether we experience an ownership change will depend on the specific facts that apply at the time of any offering. If finalized, Treasury Regulations currently proposed under Section 382 of the Code may further limit our ability to utilize our pre-change NOLs or other pre-change tax attributes if we undergo a future ownership change. Accordingly, if we earn net taxable income, our ability to use our pre-change NOLs and other pre-change tax attributes to offset U.S. federal taxable income may be subject to limitations, which could potentially result in significant increased future tax liability to us, which could materially adversely affect our profitability and cash position. In addition, at the state level, there may be periods during which the use of NOLs and other pre-change tax attributes is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

We do not intend to pay cash 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, 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;
- 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.

Our amended and restated certificate of incorporation and amended and restated by-laws designate specific courts as the exclusive forum for certain litigation that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us.

Our amended and restated certificate of incorporation and amended and restated by-laws specify that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for most legal actions involving claims brought against us by stockholders, other than suits brought to enforce any liability or duty created by the Securities Exchange Act of 1934, as amended (the "Exchange Act") or any other claim for which the federal courts have exclusive jurisdiction and any action that the Court of Chancery of the State of Delaware has dismissed for lack of subject matter jurisdiction, which may be brought in another state or federal court sitting in the State of Delaware. Our amended and restated by-laws also specify that, unless we consent in writing to the selection of an alternate forum, the United States District Court for the District of Massachusetts shall be the sole and exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act of 1933, as amended (the "Securities Act"). Any person or entity purchasing or otherwise acquiring any interest in shares of our capital stock shall be deemed to have notice of and to have consented to the provisions of our amended and restated certificate of incorporation and amended and restated by-laws described above.

We believe these provisions benefit us by providing increased consistency in the application of Delaware law by chancellors particularly experienced in resolving corporate disputes or federal judges experienced in resolving Securities Act

disputes, efficient administration of cases on a more expedited schedule relative to other forums and protection against the burdens of multi-forum litigation. However, the provision may have the effect of discouraging lawsuits against our directors, officers, employees, and agents as it may limit any stockholder's ability to bring a claim in a judicial forum that such stockholder finds favorable for disputes with us or our directors, officers, employees, or agents and result in increased costs for stockholders to bring a claim. The enforceability of similar choice of forum provisions in other companies' certificates of incorporation and by-laws has been challenged in legal proceedings, and it is possible that, in connection with any applicable action brought against us, a court could find the choice of forum provisions contained in our amended and restated certificate of incorporation and amended and restated by-laws to be inapplicable or unenforceable in such action. If a court were to find the choice of forum provision contained in our amended and restated certificate of incorporation or amended and restated by-laws to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could adversely affect our business, financial condition, or results of operations.

Changes in tax law and regulations could adversely affect our business, financial condition and results of operations.

New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could affect the tax treatment of any of our future earnings. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. Generally, future changes in applicable tax laws and regulations, or their interpretation and application, potentially with retroactive effect, could have an adverse effect on our business, financial conditions and results of operations. We are unable to predict whether such changes will occur and, if so, the ultimate impact on our business. We urge investors to consult with their legal and tax advisers regarding the implications of potential changes in tax laws on an investment in our common stock.

Unstable market and economic conditions may have serious adverse consequences on our business, financial condition and share price.

The global economy, including credit and financial markets, has recently experienced extreme volatility and disruptions, including severely diminished liquidity and credit availability, rising interest and inflation rates, declines in consumer confidence, declines in economic growth, increases in unemployment rates, geopolitical conflicts and war, and uncertainty about economic stability. If the equity and credit markets continue to deteriorate or the United States enters a recession, it may make any necessary debt or equity financing more difficult to obtain in a timely manner or on favorable terms, more costly or more dilutive. In addition, there is a risk that one or more of our CROs, suppliers or other third-party providers may not survive an economic downturn or recession. As a result, our business, results of operations and price of our common stock may be adversely affected.

Item 2. Unregistered Sales of Equity Securities, Use of Proceeds, and Issuer Purchases of Equity Securities

None

Item 3. Defaults Upon Senior Securities

None

Item 4. Mine Safety Disclosures

None

Item 5. Other Information

(a) None.

(b) None.

(c) During the quarter ended June 30, 2024, no director or officer (as defined in Rule 16a-1(f) under the Exchange Act) of the Company adopted, modified, or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement," as each term is defined in Item 408(a) of Regulation S-K.

Item 6. Exhibits

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth in the Exhibit Index below, which is incorporated herein by reference.

Exhibit Index

Exhibit Number	Exhibit Title	Incorporated by Reference			
		Form	File no.	Exhibit	Filing Date
2.1*	Separation Agreement, dated as of November 3, 2021, by and between the Registrant and 2seventy bio, Inc.	8-K	001-35966	2.1	November 4, 2021
2.2*	Asset Purchase Agreement, dated as of November 29, 2022, by and between bluebird bio, Inc. and argenx BV	8-K	001-35966	2.1	November 30, 2022
2.3*	Asset Purchase Agreement, dated as of January 5, 2023, by and between bluebird bio, Inc. and Bristol-Myers Squibb Company	8-K	001-35966	2.1	January 6, 2023
2.4*	Asset Purchase Agreement, dated as of October 26, 2023, by and between bluebird bio, Inc. and Novartis Pharma AG	8-K	001-35966	2.1	October 30, 2023
3.1	Amended and Restated Certificate of Incorporation of the Registrant	8-K	001-35966	3.1	June 24, 2013
3.2	Certificate of Amendment to the Amended and Restated Certificate of Incorporation of the Registrant	8-K	001-35966	3.1	June 20, 2023
3.3	Amended and Restated By-laws of the Registrant	8-K	001-35966	3.1	December 18, 2023
4.1	Specimen Common Stock Certificate	S-1/A	333-188605	4.1	June 4, 2013
4.2**	Form of Warrant Agreement	8-K	001-35966	4.1	August 14, 2024
4.3	Form of Warrant Agreement Amendment	8-K	001-35966	4.2	August 14, 2024
10.1**†	First Amendment to Loan and Security Agreement, dated as of April 30, 2024, between the Registrant, as Borrower, and Hercules Capital, Inc., as Lender	8-K	001-35966	10.2	May 2, 2024
31.1	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.	—	—	—	Filed herewith
31.2	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.	—	—	—	Filed herewith
32.1	Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.	—	—	—	Furnished herewith
101.INS	Inline XBRL Instance Document (the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document)				Filed herewith
101.SCH	Inline XBRL Taxonomy Extension Schema Document.	—	—	—	Filed herewith
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document.	—	—	—	Filed herewith
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document.	—	—	—	Filed herewith
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document.	—	—	—	Filed herewith
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document.	—	—	—	Filed herewith
104	Cover Page Interactive Data File (formatted as Inline XBRL with applicable taxonomy extension information contained in Exhibits 101)	—	—	—	Filed herewith

* Schedules and exhibits have been omitted pursuant to Item 601(b)(2) of Regulation S-K. The Registrant will furnish copies of any such schedules and exhibits to the SEC upon request.

** Exhibits have been omitted pursuant to Item 601(a)(5) of Regulation S-K. The Registrant will furnish copies of any such exhibits to the SEC upon request.

† Portions of this exhibit (indicated by asterisks) have been omitted in accordance with the rules of the SEC.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

bluebird bio, Inc.

Date: September 27, 2024

By: /s/ Andrew Obenshain

Andrew Obenshain
President, Chief Executive Officer and Director
(Principal Executive Officer and Duly Authorized Officer)

Date: September 27, 2024

By: /s/ O. James Sterling

O. James Sterling
Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 31.1

CERTIFICATIONS

I, Andrew Obenshain, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of bluebird bio, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: September 27, 2024

By: /s/ Andrew Obenshain

Andrew Obenshain
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, O. James Sterling, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of bluebird bio, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: September 27, 2024

By: /s/ O. James Sterling

O. James Sterling
Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of bluebird bio, Inc. (the "Company") for the period ended June 30, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers of the Company hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: September 27, 2024

By: /s/ Andrew Obenshain

Andrew Obenshain
President, Chief Executive Officer and Director
(Principal Executive Officer and Duly Authorized Signer)

Date: September 27, 2024

By: /s/ O. James Sterling

O. James Sterling
Chief Financial Officer
(Principal Financial Officer, Principal Accounting Officer and Duly Authorized Signer)