bluebird bio, Inc.

2023
Annual Report

Fellow shareholders –

At the time of my last letter, bluebird bio was embarking on the penultimate chapter of a decades-long scientific odyssey, having just submitted our Biologics Licensing Application (BLA) for lovo-cel gene therapy for sickle cell disease. In parallel, we were progressing the simultaneous launches of ZYNTEGLO™ for beta-thalassemia and SKYSONA™ for cerebral adrenoleukodystrophy (CALD), bringing urgently needed treatment options to patients while building the foundation to realize our largest commercial opportunity yet.

In December, after nearly 10 years of clinical development, bluebird bio celebrated the approval of LYFGENIA™—a potentially transformative gene therapy for sickle cell disease. This was a watershed moment for the sickle cell disease community, for our company, and for our entire industry. Today, after decades of underinvestment in research and care, people living with sickle cell disease have transformational new options that can alter the course of their disease. It is a rare privilege to be part of an advancement of this magnitude. With your support, we made history—and we are continuing to work to make this long-awaited option a reality for patients in the commercial setting.

Timely and equitable access to new therapies is essential for people living with sickle cell disease. That principle is the cornerstone of our commercial strategy, which was honed over 18 months of experience prior to LYFGENIA's approval.

Our network of treatment centers was unparalleled at launch, with 27 centers ready to receive patient referrals on "Day 1"—and it remains unmatched today. Less than nine months after approval, that number has nearly tripled, with more than 70 centers currently activated or in the onboarding process across our network—what we believe to be a testament to providers' belief in the clinical profile of LYFGENIA and its potential to transform their patients' lives, as well as their trust in bluebird as an experienced partner. As I write this letter, nearly 100 patients have started or are scheduled to initiate a bluebird gene therapy across more than 30 centers, with capacity for significant growth to come across the full network.

Momentum on the payer side has been equally impressive. Within days of approval, bluebird announced that 100 million lives were covered by commercial contract. That figure doubled within a month, and we successfully implemented our first outcomes-based agreement with a Medicaid payer in the first quarter of 2024. We believe the breadth and quality of coverage we have secured less than a year following approval underscores that payers recognize the differentiated value LYFGENIA provides as the most deeply studied gene therapy approved for sickle cell disease, and are prepared to support access.

Underpinning this belief from patients, providers, and payers is our clinical experience and follow-up data. At the American Society of Hematology Annual Meeting in December, bluebird presented data on 47 patients through five years of follow up, including outcomes on patients with a history of stroke, which is unique to our clinical development program. These data represent the most patients treated, and longest follow-up, in the field—which is critical for a community that values trust and transparency.

As we have focused on building momentum behind the multi-year launch of LYFGENIA, we have continued to drive favorable and timely access to ZYNTEGLO and SKYSONA. The demand for

bluebird therapies among patients and providers is clear and we believe we have the commercial infrastructure, relationships, and real-world experience to deliver. Yet, as we know from our experience with ZYNTEGLO and SKYSONA, translating early commercial momentum into revenue takes time.

As I write you today, bluebird sits in a class of its own. We are the only independent company with 3 FDA approved gene therapies and we are making a difference in the world's most underserved diseases. Beyond our near-term, billion-dollar commercial opportunities, we occupy a unique strategic position within the industry, having amassed a completely integrated set of capabilities needed to advance gene therapy from research and clinical development, through regulatory review, to scaling commercial manufacturing. We plan to continue to take steps to strengthen our balance sheet and extend our cash runway so we can cross the bridge to sustainability and realize both our immediate commercial opportunities and long-term growth potential.

Like every pioneer, we have faced and overcome challenges—and we are continually reminded of the importance of perseverance by the patients and families we serve. We remain committed to realizing the commercial promise of gene therapy and forging the path for future innovation. Every day, we are persisting for patients—those who have put their trust in us today and whose lives we can transform tomorrow. Thank you for being on this journey alongside us.

Onward,

Andrew

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2023
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)

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

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. **Yes** ☐ **No** ☒

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. **Yes** ☐ **No** ☒

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, 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 has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☐

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☒

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to § 240.10D-1(b). ☐

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

The aggregate market value of common stock held by non-affiliates of the registrant based on the closing price of the registrant's common stock as reported on the Nasdaq Global Select Market on June 30, 2023, the last business day of the registrant's most recently completed second quarter, was $346,285,765.

As of September 11, 2024, there were 193,913,585 shares of the registrant's common stock, par value $0.01 per share, outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

None.

Table of Contents

EXPLANATORY NOTE

Background on Restatement of Previously Issued Financial Statements

As previously disclosed in the Current Report on Form 8-K filed by bluebird bio, Inc. (the "Company," "we", "our" or "us") with the Securities and Exchange Commission ("SEC") on March 26, 2024, in connection with the preparation of the financial statements of the Company for the year ended December 31, 2023, the Company identified certain accounting errors relating to the application of U.S. Generally Accepted Accounting Principles ("US GAAP") in connection with the Company's accounting for lease arrangements, including certain arrangements with contract manufacturing organizations ("CMOs") and a contract testing organization ("CTO") that are deemed to contain one or more leases for accounting purposes. On March 24, 2024, the Company's Audit Committee of the Board of Directors, based on the recommendation of management and after discussion with Ernst & Young LLP ("EY"), concluded that the Company's previously-issued audited consolidated financial statements for each fiscal year beginning January 1, 2019 and its previously-issued unaudited interim condensed consolidated financial statements for each of the first three quarters in such years, as well as the associated earnings releases and investor presentations or other communications describing such financial statements, were materially misstated and, accordingly, should no longer be relied upon.

The errors related to compliance with US GAAP in connection with the Company's accounting for lease arrangements, including the identification of embedded leases, the accounting for lease modifications and other elements of lease accounting, the inconsistent application of its accounting policy to combine lease and non-lease components in lease arrangements, including embedded leases and other matters described in Note 2 to the consolidated financial statements.

This Annual Report on Form 10-K restates the Company's previously issued consolidated financial statements as of and for the year ended December 31, 2022 and its previously issued unaudited condensed consolidated financial information for each of the first three quarters of the years ended December 31, 2023 and 2022 (each such annual and quarterly period to be restated, a "Restated Period" and collectively the "Restated Periods"). As a result of the Company's evaluation, additional immaterial errors, were also corrected and are further described within Note 2. Restatement of Previously Issued Financial Statements to our consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

Restatement of Previously Issued Financial Statements

This Annual Report on Form 10-K for the year ended December 31, 2023, includes the following information:

a. restated consolidated balance sheets as of December 31, 2022, and the related consolidated statements of operations and comprehensive loss, stockholders' equity, and cash flows for the year ended December 31, 2022;
b. restated Unaudited Quarterly Financial Data for the first three quarters of the years ended December 31, 2023 and 2022;
c. amended Management's Discussion and Analysis of Financial Condition and Results of Operations ("MD&A") related to the year ended December 31, 2022.

In addition to the misstatements related to the lease accounting matters, the Company also recorded other adjustments to correct previously uncorrected misstatements that were not material, individually or in the aggregate to its consolidated financial statements of prior periods. The Company has also restated impacted amounts within the notes to the consolidated financial statements, as applicable.

For a description of the financial impact of the restatement, see Note 2, Restatement of Previously Issued Financial Statements to our consolidated financial statements and Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations, contained in this Annual Report on Form 10-K. For the impact of these adjustments on the Unaudited Quarterly Financial Data, see Note 21, Quarterly Financial Information (Unaudited) to our consolidated financial statements included elsewhere in this Annual Report on Form 10-K. All amounts in this Annual Report on Form 10-K affected by the restatement reflect such amounts as restated.

Internal Control Considerations

In connection with the Company's review of its financial statements leading to the restatement, 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 financial statements will not be prevented or detected on a timely basis. Therefore, the Company's management concluded that due to the material weakness in the Company's internal control over financial reporting, the Company's internal control over financial reporting was not effective as of December 31, 2023, and in prior periods. In addition, as a result of the material weakness, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures were not effective as of December 31, 2023. See Item 9A. Controls and Procedures, contained in this Annual Report on Form 10-K for additional information and discussion related to material weakness in internal control over financial reporting and our related remediation activities.

FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K 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 Annual Report on Form 10-K 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;
- our ability to comply with Nasdaq continued listing rules;
- our ability to remediate the material weakness in our internal control over financial reporting; and
- other risks and uncertainties, including those listed under Part I, Item 1A. Risk Factors.

Any forward-looking statements in this Annual Report on Form 10-K 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 I, Item 1A. Risk Factors and elsewhere in this Annual Report on Form 10-K. 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 Annual Report on Form 10-K 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 Annual Report on Form 10-K 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.

- 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.

PART I

Item 1. Business

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), and 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 for 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 ("VOEs").

We are uniquely positioned as one of the only standalone commercial gene therapy companies today, and we are deploying a validated commercial strategy with the launch of LYFGENIA for SCD, while continuing to scale the launches of ZYNTEGLO and SKYSONA. We are focused on establishing a robust qualified treatment center ("QTC") network, ensuring timely, equitable access for patients, and optimizing the patient and provider experience. We estimate that there are approximately 22,000 individuals living with SCD, ß-thalassemia and CALD in the U.S. whose conditions may potentially be addressable with our gene therapies; of these, we estimate that approximately 20,000 individuals living with SCD could be addressed with LYFGENIA. We estimate that there are between 1,300-1,500 patients living with ß-thalassemia and 40 patients diagnosed with CALD each year.

Our treatments are administered at QTCs – a network of transplant centers that are carefully selected based on their expertise in cell and gene therapy and trained to administer our products. The QTC network for both ZYNTEGLO and LYFGENIA is highly synergistic. As of August 14, 2024, we had activated more than 70 total QTCs (defined as having a signed master services agreement) for both therapies. Six of those QTCs were also activated to administer SKYSONA. Additionally, we have put in place outcomes-based agreements for both ZYNTEGLO and LYFGENIA that provide payers with meaningful risk sharing tied to clinical outcomes that can be tracked in claims data.

We have a focused commercial footprint that is dedicated to the U.S., with wholly owned global rights for all three of our commercial programs. We continue to take steps to strengthen our balance sheet, including the completion of a $125 million equity raise in December 2023 and entrance into a term loan facility in March 2024 for up to $175 million, as we pursue a path to profitability.

Our Platform

We believe we have the largest and deepest ex vivo gene therapy data set in the industry, with more than 1,000 patient years of experience. We custom design each of our products to address the underlying cause of disease by introducing a functional copy of a gene to patients' own enriched hematopoietic stem cells ("HSCs"). Our LVV gene therapies are uniquely traceable, which allows us to identify and track LVV-modified cells after delivery to a patient. In a rapidly advancing field, we have developed in-depth analytical methods to understand the safety of our LVV technologies, which are designed to deliver a sustained, lifelong response from a one-time treatment and to improve upon allogeneic hematopoietic stem cell transplant ("HSCT"), which carries significant limitations, including difficulty in identifying matched donors, risk of transplant-related graft-vs-host disease ("GVHD") and death, as well as to improve upon current treatment approaches used with patients not currently eligible to receive allogeneic HSCT.

Our Programs

LYFGENIA™ (lovotibeglogene autotemcel)

On December 8, 2023, the FDA approved LYFGENIA (lovotibeglogene autotemcel, also known as lovo-cel) for the treatment of patients 12 years of age and older with SCD and a history of VOEs. The FDA approval of LYFGENIA builds on decades of research into LVV gene addition therapy and what we believe is the largest clinical development program of any gene therapy for SCD—with the most patients treated and longest follow-up.

SCD is a complex and progressive genetic disease associated with debilitating and unpredictable pain crises, anemia, irreversible damage to vital organs, and early death. In SCD, high concentrations of sickle hemoglobin (HbS) in red blood cells (RBCs) cause RBCs to become sickled, sticky, and rigid with a shorter life span, which manifests acutely as hemolytic anemia, vasculopathy, and vaso-occlusion. Pain onset can be sudden and unpredictable, often requiring hospitalization and transfusions, intravenous fluids, pain medications, and antibiotic therapy. Repeated VOEs result in end organ damage in fifty to sixty percent of adults with SCD, with 24 percent experiencing damage in multiple organs, and one in four patients experiencing a stroke by the age of 45. The impact of SCD is pervasive and affects every aspect of life for patients and their families and caregivers – from missed work and school, decreased quality of life and mental health, and diminished ability to complete daily tasks. In the U.S., there are approximately 100,000 people living with SCD, and the median age of death is 45 years of age.

LYFGENIA works by using an LVV to permanently add a functional beta-globin gene ($\beta^{A\text{-}T87Q}$-globin gene) into the patient's own HSCs. Durable production of adult hemoglobin with anti-sickling properties (HbAT87Q) is expected following successful engraftment. HbAT87Q has a similar oxygen-binding affinity to wild-type HbA and is designed to limit sickling of RBCs and reduce VOEs. The functional beta-globin gene is added into a patient's cells outside of the body (ex-vivo), and the modified cells are then administered to the patient via infusion. The treatment process is comprised of several steps that may take place over the course of several months.

Clinical Development Program

We believe we have the most deeply studied gene therapy program for SCD, with the longest follow up for any clinical program in the field. We are conducting, or have conducted, the following clinical studies to evaluate the safety and efficacy of lovo-cel in the treatment of patients with SCD:

- HGB-206 was a single-dose, open-label, non-randomized, multi-site Phase 1/2 clinical study in the U.S. evaluating the safety and efficacy of lovo-cel. A total of 45 patients were treated with lovo-cel in this study across three treatment cohorts. Patients must have been at least twelve years of age at enrollment with a diagnosis of SCD, with β^S/β^S, $\beta^S/\beta+$ or β^S/β^0 genotype. Patients must have had recurrent severe VOEs despite treatment with hydroxyurea or must have had intolerance to hydroxyurea. Five patients with a history of stroke were treated in HGB-206 Group C. We refer to patients treated in the HGB-206 study under the amended study protocol utilizing HSCs from peripheral blood after mobilization with plerixafor as patients in "Group C" (n=36) rather than utilizing HSCs collected via bone marrow harvest, as in Groups A (n=7) and B (n=2). A refined manufacturing process designed to increase vector copy number and further protocol refinements made to improve engraftment potential of gene-modified stem cells were used for Group B and Group C. The primary efficacy endpoint for this study was the percentage of patients with complete resolution of all VOEs, between six- and 18-months post-treatment, and the secondary efficacy endpoints for this study included the percentage of patients with complete resolution of severe VOEs and globin response based on $\beta^{A\text{-}T87Q}$ expression and total hemoglobin. Safety endpoints included monitoring for laboratory parameters and frequency and severity of adverse events; the success and kinetics of HSC engraftment; the incidence of treatment related mortality and overall survival; the detection of vector-derived replication-competent lentivirus in any patient; and the characterization of events of insertional mutagenesis leading to oligoclonality, or leukemia. Each patient remained on study for approximately 24 months post-treatment and was then invited to enroll in LTF-307, a long-term follow-up protocol that will assess safety and efficacy for an additional 13 years.

- HGB-210 is an ongoing single-dose, open-label, non-randomized, multi-site, Phase 3 clinical study in the U.S. designed to evaluate the efficacy and safety of lovo-cel in the treatment of patients with SCD, with a target enrollment of 35 pediatric and adult patients. Patients must be at least two years of age at enrollment with a diagnosis of sickle cell disease, with β^S/β^S, $\beta^S/\beta+$ or β^S/β^0 genotype. Patients must have recurrent VOEs despite treatment with hydroxyurea or must have had intolerance to hydroxyurea. The primary efficacy endpoint for this study is complete resolution of VOEs, between six- and 18-months post-treatment, and the secondary efficacy endpoint for this study includes the percentage of patients with complete resolution of severe VOEs and globin response based on $\beta^{A\text{-}T87Q}$ expression and total hemoglobin. Safety endpoints include monitoring for laboratory parameters and frequency and severity of adverse

events; the success and kinetics of HSC engraftment; the incidence of treatment related mortality and overall survival; the detection of vector-derived replication-competent lentivirus in any patient; and the characterization of events of insertional mutagenesis leading to oligoclonality or leukemia. Each patient remains on study for approximately 24 months post-treatment and is then invited to enroll in LTF-307, a long-term follow-up protocol that will assess safety and efficacy for an additional 13 years. Data from drug product manufactured at our commercial manufacturing facility for the HGB-210 study were included in the lovo-cel Biologics License Application ("BLA") submission to demonstrate analytical comparability and support validation of our commercial manufacturing process.

- HGB-205 was a proof-of-concept, single-center Phase 1/2 study in France of three patients with SCD which also enrolled patients with transfusion-dependent β-thalassemia ("TDT"). Those with SCD must have failed to achieve clinical benefit from treatment with hydroxyurea and have an additional poor prognostic risk factor (e.g., recurrent vaso-occlusive crises ("VOCs"), or acute chest syndrome). All patients must have been eligible for allogeneic HSCT, but without a matched sibling allogeneic HSCT donor. The primary objective of our HGB-205 study was to determine the safety, tolerability and success of engraftment of the drug product. The secondary objectives of the study were to quantify gene transfer efficiency and expression, and to measure the effects of treatment on disease-specific biological parameters and clinical events. In the case of patients with TDT and SCD, this meant the volume of RBC transfusions, and for patients with SCD, it also meant the number of VOCs and acute chest syndrome in each patient, compared with the two-year period prior to treatment. Safety evaluations performed during the study included success and kinetics of HSC engraftment, incidence of transplant-related mortality post-treatment, overall survival, detection of vector-derived replication-competent lentivirus in any patient and characterization of events of clonal predominance or leukemia/ lymphoma. Each patient remained on study for approximately 24 months post-treatment and then was invited to enroll in LTF-307, a long-term follow-up protocol that will assess safety and efficacy for an additional 13 years.

- LTF-307 is the long-term follow-up study for patients with SCD from our HGB-205, HGB-206, or HGB-210 studies who were treated with lovo-cel. After each original study protocol's follow-up period of approximately two years, patients will be followed under LTF-307 for approximately an additional 13 years for a total of approximately 15 years post-treatment.

Efficacy endpoints in the clinical studies focused on the resolution of VOEs and severe vaso-occlusive events ("sVOEs"), as well as globin response based on $\beta^{A\text{-}T87Q}$ expression, total hemoglobin and health related quality of life measures. In the studies, VOEs are defined as episodes of acute pain with no medically determined cause other than a vaso-occlusion, lasting more than two hours and severe enough to require care at a medical facility. This includes acute chest syndrome requiring oxygen treatment and/or blood transfusion, acute hepatic sequestration, acute priapism lasting two hours and requiring care at a medical facility, and acute splenic sequestration. sVOEs require a 24-hour hospital stay or emergency room visit, or at least two visits to a hospital or emergency room over a 72-hour period, with both visits requiring intravenous treatment; all VOEs of priapism are also considered sVOEs.

The FDA approved label for LYFGENIA is based on data from patients in the Phase 1/2 HGB-206 study, including safety data from 54 patients who initiated stem cell collection and efficacy data from 36 patients in Group C, following enhancements to the treatment and manufacturing processes made through the course of the clinical development program. 32 patients were evaluable for the endpoints of complete resolution of VOEs and severe VOEs in the six -18 months post-infusion including eight adolescent patients.

Data from the February 2023 data cut presented at the 65th American Society of Hematology (ASH) Annual Meeting & Exposition focused on 34 of the 47 patients treated in Phase 1/2 HGB-206 Group C and Phase 3 HGB-210 studies who were evaluable for the primary and secondary endpoints of complete resolution of VOEs and sVOEs with a median (min, max) follow-up of 36.3 months (12.1, 61). The data showed 32/34 evaluable patients (94%) experienced complete resolution of sVOEs, maintained for a median (min, max) of 35.8 months (20.2, 61). 30/34 patients (88.2%) experienced complete resolution of all VOEs, maintained for a median (min, max) of 35.8 months (20.2, 61). Additionally, 10/10 evaluable patients less than 18 years of age achieved complete resolution of VOEs. Five patients with history of stroke were treated in HGB-206 Group C. At 44-60 months follow-up, all 5 remain transfusion independent without recurrent stroke. Moreover, long-term follow up data support the durable, potentially curative benefits of LYFGENIA through stable production of anti-sickling adult hemoglobin and resolution of VOEs. Patients treated with LYFGENIA also experienced a sustained reduction in hemolysis markers, including total hemoglobin (Hb). Following engraftment, non-transfused total Hb and %Hb fractions stabilized by approximately 6 months after lovo-cel infusion, and median percent of gene-therapy derived anti-sickling adult hemoglobin (HbAT87Q) was maintained generally at >40% of non-transfused total Hb throughout follow-up. Based on data from patient reported outcomes, treatment also resulted in decreased pain scores and improved quality of life for patients with SCD.

The majority of adverse events in patients treated in HGB-206 Group C and HGB-210 were attributed to underlying SCD or conditioning with busulfan. Nonserious adverse events related to LYFGENIA included infusion reactions (abdominal discomfort, decreased diastolic blood pressure, and nasal congestion) each in one patient (2.1% each). Serious adverse events related to LYFGENIA were reported in two patients with comorbid alpha-thalassemia trait and they included two serious adverse events each of anemia (4.3%) and 1 serious adverse event of myelodysplastic syndrome ("MDS") (2.1%). The diagnosis of MDS remains under evaluation. This patient is being followed by their physician, is not being treated for MDS, and remained clinically stable and free of VOEs as of August 27, 2024. One patient died due to sudden cardiac death which was deemed unrelated to LYFGENIA. As previously reported, cases of acute myeloid leukemia ("AML") were observed in two patients from the HGB-206 Group A cohort who were treated with an earlier version of the therapy using a different manufacturing process and transplant procedure. Both patients died due to aforementioned leukemia. The LYFGENIA product label includes a boxed warning for the risk of hematologic malignancy. There have been no cases of insertional oncogenesis or graft failure observed across the entire clinical development program.

As part of the FDA approval, we are required to conduct a prospective, multi-center observational study (REG-503) which will evaluate the long-term safety of LYFGENIA, including the risk of secondary malignancies occurring after treatment with LYFGENIA in at least 250 patients.

Additionally, LYFGENIA was granted a rare pediatric disease designation in May 2020 and we anticipated, but did not receive, a Rare Pediatric Disease Priority Review Voucher (PRV) as part of the BLA approval in December 2023. In February 2024, we submitted a Request for Reconsideration, which was denied by the FDA in August 2024. We plan to continue to pursue the PRV through the Formal Dispute Resolution process.

ZYNTEGLO™ (betibeglogene autotemcel)

On August 17, 2022, the FDA approved ZYNTEGLO, the first gene therapy for people with β-thalassemia who require regular red blood cell transfusions. ZYNTEGLO is approved for patients of all ages and genotypes and offers patients a potentially curative benefit through the achievement of durable transfusion independence and normal or near normal total hemoglobin levels.

β-thalassemia is a rare, genetic blood disease caused by mutations in the beta-globin gene and characterized by significantly reduced or absent adult hemoglobin production. Patients with the most severe form, sometimes called transfusion-dependent β-thalassemia or β-thalassemia major, experience severe anemia and lifelong dependence on regular red blood cell transfusions, a lengthy process that patients typically undergo every 2-5 weeks. Despite advances in treatment and improved transfusion techniques, transfusions only temporarily address symptoms of anemia and people with β-thalassemia who require regular transfusions have an increased risk for morbidity and mortality due to complications from treatment-related iron overload. Data from the Cooley's Anemia Foundation indicate that the median age of death of patients with transfusion-dependent β-thalassemia in the U.S. who died during the last decade was just 37 years. We estimate that there are approximately 1,300-1,500 individuals with transfusion-dependent β-thalassemia in the U.S.

ZYNTEGLO works by using an LVV to add functional copies of a modified form of the beta-globin gene ($\beta^{A\text{-}T87Q}$-globin gene) into a patient's own HSCs, allowing them to make normal to near normal levels of total hemoglobin without regular red blood cell ("RBC") transfusions. The functional beta-globin gene is added into a patient's cells outside of the body (ex-vivo), and the modified cells are then administered to the patient via infusion. The treatment process is comprised of several steps that may take place over the course of several months.

Clinical Development Program

We believe we have the most deeply studied gene therapy program for β-thalassemia in the field of gene therapy, with the longest follow up for any clinical program in the field. We are conducting, or have conducted, the following clinical studies to evaluate the safety and efficacy of beti-cel in the treatment of patients with β-thalassemia:

- HGB-207 was a single-dose, open-label, non-randomized, international, multi-site Phase 3 clinical study that evaluated the safety and efficacy of beti-cel to treat patients with transfusion-dependent β-thalassemia ("TDT") and non-β^0/β^0 genotypes. This study was completed in March 2022. Twenty-three patients were enrolled and completed dosing in the study, consisting of 15 adolescent and adult patients between 12 and 34 years of age at enrollment, and eight pediatric patients less than 12 years of age at enrollment. Age at enrollment ranged from four to 34 years old. To be enrolled, patients with TDT and non-β^0/β^0 genotypes had to have received at least 100 mL/kg/year of RBCs or at least eight transfusions per year for the previous two years. All patients must have been eligible for HSCT, but without a known

and available matched family HSCT donor. The primary endpoint of this study was the proportion of treated patients who achieved transfusion independence, defined as weighted average hemoglobin levels ≥9.0 g/dL without any RBC transfusions for a continuous period of at least 12 months at any time during the study after treatment. The secondary endpoints of this study were designed to quantify gene transfer efficiency and expression, and to measure the effects of treatment with beti-cel on transfusion requirements and clinical events. Safety evaluations performed during the study included monitoring of laboratory parameters, frequency and severity of clinical adverse events, incidence of acute and/or chronic graft versus host disease, success and kinetics of platelet and neutrophil engraftment, incidence of transplant-related mortality post-treatment, overall survival, detection of vector-derived replication-competent lentivirus in any patient and characterization of events of clonal predominance or leukemia. Each patient remained on study for approximately 24 months post-treatment and then was invited to enroll in LTF-303, a long-term follow-up protocol that is assessing safety and efficacy for an additional 13 years.

- HGB-212 was a single-dose, open-label, non-randomized, international, multi-site Phase 3 clinical study that evaluated the efficacy and safety of beti-cel to treat patients with TDT who have either a β^0/β^0, β^0/IVS-I-110, or IVS-I-110/IVS-I-110 genotypes. This study was completed in November 2022. Eighteen patients were enrolled and completed dosing in the study, consisting of ten adolescent and adult patients between twelve and 34 years of age at enrollment, and eight patients less than twelve years of age at enrollment. To be eligible, patients must have received at least 100 mL/kg/year of RBCs or at least eight transfusions per year for the previous two years. All patients must have been clinically stable and eligible to undergo HSCT (but without a known and available matched family HSCT donor) and been treated and followed for at least the last two years in a specialized center that maintained detailed medical records, including transfusion history. The primary endpoint of this study was the proportion of treated patients who met the definition of transfusion independence, which was identical to the definition in our HGB-207 study. The secondary endpoints of this study were designed to measure the proportion of patients who meet the definition of transfusion reduction, which was defined as the reduction in volume of RBC transfusion requirements (in mL/kg) in the post-treatment time period of months 12 to 24 compared to the average annual transfusion requirement in the 24 months prior to enrollment, to quantify gene transfer efficiency and expression, and to measure the effects of treatment with beti-cel on transfusion requirements post-treatment and clinical events. Safety evaluations performed during the study included monitoring of laboratory parameters, frequency and severity of clinical adverse events, incidence of acute and/or chronic graft versus host disease, success and kinetics of platelet and neutrophil engraftment, incidence of transplant-related mortality post-treatment, overall survival, detection of vector-derived replication-competent lentivirus in any patient and characterization of events of clonal predominance or leukemia. Each patient remained on study for approximately 24 months post-treatment and then was invited to enroll in LTF-303, a long-term follow-up protocol that is assessing safety and efficacy for an additional 13 years.

- HGB-204 was a single-dose, open-label, non-randomized, international, multi-site Phase 1/2 clinical study designed to evaluate the safety and efficacy of beti-cel in increasing hemoglobin production and the proportion of treated patients who meet the definition of transfusion independence. This study was completed in February 2018, and patients in this study were enrolled in a long-term follow-up protocol to assess safety and efficacy beyond the study follow-up period. Eighteen adults and adolescents were treated in the study. To be eligible for enrollment in this study, patients were between 12 and 35 years of age with a diagnosis of TDT and received at least 100 mL/kg/year of RBCs or at least eight transfusions per year in each of the two years preceding enrollment. The patients were also medically eligible for allogeneic HSCT. Efficacy was evaluated primarily by the production of ≥2.0 g/dL of hemoglobin A containing β^{A-T87Q}-globin for the six-month period between 18- and 24-months post-treatment. Exploratory efficacy endpoints included RBC transfusion requirements per month and per year, post-treatment. Safety evaluations performed during the study included success and kinetics of HSC engraftment, incidence of transplant-related mortality post-treatment, overall survival, detection of vector-derived replication-competent lentivirus in any patient and characterization of events of insertional mutagenesis leading to clonal predominance or leukemia. Subjects were monitored by regular screening. Each patient remained on study for approximately 24 months from time of consent and then was invited to enroll in LTF-303, a long-term follow-up protocol that is assessing safety and efficacy for an additional 13 years.

- HGB-205 was a proof-of-concept, single-dose, open-label, non-randomized, Phase 1/2 clinical study conducted at a single site in France in four patients with β-thalassemia that also enrolled patients with SCD. The primary objective of the HGB-205 study was to determine the safety, tolerability and success of engraftment of the drug product. Each patient remained on study for approximately 24 months from time of consent and then was invited to enroll in LTF-303, a long-term follow-up protocol that is assessing safety and efficacy for an additional 13 years.

- LTF-303 is the long-term follow-up study for patients with β-thalassemia from our HGB-204, HGB-205, HGB-207, or HGB-212 studies. Patients were invited to enroll in the long-term follow-up study once they completed the original study protocol's follow-up period of approximately two years. Under LTF-303, patients will be followed for

approximately an additional 13 years for a total of approximately 15 years post-treatment. Each of HGB-204, HGB-205, HGB-207 and HGB-212 are complete, and all patients have transitioned to LTF-303.

The approval of ZYNTEGLO was based primarily on data from the Phase 3 HGB-207 and HGB-212 studies and the long-term follow-up study LTF-303.

As of the January 30, 2023 data cut presented at the American Society of Hematology (ASH) Annual Meeting in December 2023, 63 patients had received beti-cel across four clinical studies with a median follow-up of five years (60.1 months; range: 23.8-109.5). These include two Phase 3 studies (N=41) that led to the FDA approval of ZYNTEGLO in August 2022. Data from the Phase 3 studies demonstrated that 90.2% (37/41) of patients treated achieved transfusion independence as of the January 2023 data cut through last follow-up (up to a maximum of six years). Transfusion independence is defined as no longer needing red blood cell transfusions for at least 12 months while maintaining a weighted average total hemoglobin of at least 9 g/dL. Patients who achieved transfusion independence produced normal or near normal levels of total hemoglobin and demonstrated improvements in markers of iron overload and markers of ineffective erythropoiesis. 100% of patients who achieved transfusion independence maintained it, demonstrating durability of results as of last follow-up.

Data from all 63 patients treated across our β-thalassemia gene therapy clinical development program have demonstrated sustained treatment effect, improvements in iron management and quality of life improvements in patients with β-thalassemia who require regular red blood cell transfusions following treatment with beti-cel up to 9 years post-treatment (n=1), across ages and genotypes, and a median follow up of five years (60.1 months, range: 23.8-109.5). Based on testimonials collected at month 36 from patients who achieved transfusion independence, improvements in health-related quality of life ("HRQOL") measures were reported in both adult and pediatric patients up to 36 months after treatment. Additionally, improvements in iron management have been observed in patients who completed either a Phase 1/2 or Phase 3 beti-cel parent study and subsequently enrolled in the long-term follow-up study and were followed for up to 9 years. Across all studies, 37/51 patients restarted chelation, and 12 received phlebotomy post-infusion; however, 69% (35/51) were able to stop chelation therapy, demonstrating restoration of iron levels over time and reduced iron management burden in those patients.

Nineteen percent (12/63) of patients experienced ≥1 adverse event ("AE") considered related or possibly related to beti-cel; the most common beti-cel related AEs were abdominal pain (5/63 [8%]) and thrombocytopenia (3/63 [5%]). Five patients experienced serious veno-occlusive liver disease; all five received defibrotide and recovered. Three patients experienced acute events unrelated to their β-thalassemia that required packed red blood cell transfusions (Phase 1/2, n=1; Phase 3, n=2). No hematologic malignancies, insertional oncogenesis, or vector-derived replication competent lentivirus were observed.

As part of the FDA approval, we are currently conducting a required prospective, multi-center observational study (REG-501) which will evaluate the long-term safety of ZYNTEGLO, including the risk of secondary malignancies occurring after treatment with ZYNTEGLO in at least 150 patients.

SKYSONA™ (elivaldogene autotemcel)

On September 16, 2022, the FDA granted accelerated approval of SKYSONA (elivaldogene autotemcel), also known as eli-cel, to slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active CALD. The SKYSONA product label includes a boxed warning for the risk of hematologic malignancy.

CALD is the most severe form of adrenoleukodystrophy, a rare X-linked metabolic disorder caused by mutations in the *ABCD1* gene, which results in accumulation of very long-chain fatty acids ("VLCFAs") in plasma and tissues. CALD involves a progressive destruction of myelin, the protective sheath of the nerve cells in the brain that are responsible for thinking and muscle control. The disease, which primarily affects young boys, is associated with irreversible neurologic decline, including major functional disabilities such as loss of communication, cortical blindness, requirement for tube feeding, total incontinence, wheelchair dependence, or complete loss of voluntary movement. Nearly half of patients who do not receive treatment die within five years of symptom onset. Prior to the approval of SKYSONA treatment, effective options were limited to allo-HSCT, which is associated with the risk of serious potential complications including GVHD and death, which can increase dramatically in patients without a human leukocyte antigen matched donor.

Our approach involves the ex vivo insertion of a functional copy of the *ABCD1* gene into the patient's own HSCs via LVV. Following engraftment, we expect the transduced HSCs to differentiate into other cell types, including macrophages and cerebral microglia, which produce functional adrenoleukodystrophy protein ("ALDP"). We believe that the functional ALDP can then enable the local degradation of VLCFAs in the brain, which in turn can stabilize the disease by preventing further cerebral inflammation and demyelination that are characteristics of CALD.

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 this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

Clinical Development Program

The approval of SKYSONA was based on data from our Phase 2/3 study (ALD-102 (n=32)) and Phase 3 study (ALD-104 (n=35)).

Both open-label, single-arm studies enrolled patients with early, active CALD who had elevated VLCFA levels, a Loes score between 0.5 and 9 (inclusive), and gadolinium enhancement on magnetic resonance imaging (MRI) of demyelinating lesions. Additionally, patients were required to have a neurologic function score ("NFS") of ≤ 1, indicating limited changes in neurologic function. The efficacy of SKYSONA was compared to a natural history population.

Per protocol, patients treated with SKYSONA were assessed using the NFS and monitored for the emergence of six Major Functional Disabilities ("MFDs") associated with CALD progression including loss of communication, cortical blindness, requirement for tube feeding, total incontinence, wheelchair dependence, or complete loss of voluntary movement.

The accelerated approval of SKYSONA was based on an intermediate clinical endpoint of MFD-free survival. A post-hoc enrichment analysis in symptomatic patients assessed MFD-free survival from onset of symptoms (NFS ≥ 1) in SKYSONA treated (N=11) and untreated patients (N=7). SKYSONA treated patients had an estimated 72 percent likelihood of MFD-free survival at 24 months from time of first NFS ≥ 1, compared to untreated patients who had an estimated 43 percent likelihood of MFD-free survival.

The most common adverse events (incidence [≥ 20%]) were mucositis, nausea, vomiting, febrile neutropenia, alopecia, decreased appetite, abdominal pain, constipation, pyrexia, diarrhea, headache and rash. Hematologic malignancy has developed in patients treated with SKYSONA in clinical studies and, as of September 11, 2024, MDS, MDS relapse, or AML had been diagnosed in seven patients after administration of SKYSONA. One patient experienced relapse of MDS post-allogeneic transplant. The SKYSONA product label includes a boxed warning for the risk of hematologic malignancy. In April 2024, U.S. Prescribing Information for SKYSONA, including 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.

Studies ALD-102 and ALD-104 have been completed. All patients who completed 24 months of follow-up in studies ALD-102 or ALD-104 were encouraged to participate in a long-term follow-up study (LTF-304) to continue monitoring safety and efficacy outcomes through 15 years post-treatment.

As part of the accelerated approval process with the FDA, we have post-marketing requirements to follow patients who received SKYSONA in studies ALD-102 and ALD-104 for a minimum of ten years to assess event-free survival or need for hematopoietic stem cell transplant. In addition, as a post-marketing requirement, a long-term study is being conducted to assess the safety of SKYSONA and the risk of secondary malignancies occurring after treatment with SKYSONA. We plan to fulfill this latter post-marketing requirement through an observational study (REG-502) which will enroll at least 120 patients followed for 15 years who receive SKYSONA in the commercial setting, and will include studying the effectiveness and safety in 24 boys with more advanced early, active CALD for at least five years.

Manufacturing activities

We have entered into multi-year agreements with external manufacturing partners to support our programs in the United States. We have multi-year agreements with SAFC Carlsbad, Inc. ("SAFC", a subsidiary of MilliporeSigma), and Thermo Fisher Scientific, Inc. (“Thermo Fisher”, previously Novasep) in the production of LVV. We use an adherent cell culture process at SAFC to manufacture LVV for ZYNTEGLO and SKYSONA. In 2020, we transitioned the LVV manufacturing process for LYFGENIA from an adherent cell culture process to a suspension process at Thermo Fisher in order to meet anticipated commercial demand and lower our cost of production. In August 2024, we provided notice to SAFC of our intention to wind down adherent LVV manufacturing for ZYNTEGLO and SKYSONA, as we pursue alternative manufacturing methods and plans for LVV for these products. SAFC will continue to manufacture LVV for approximately twelve months pursuant to the notice period under the applicable work order and our Clinical and Commercial Supply Agreement with SAFC remains in place. In addition, we have entered into multi-year agreements with Lonza Houston, Inc. ("Lonza") to produce commercial ZYNTEGLO and SKYSONA drug product and with Minaris Regenerative Medicine ("Minaris") to produce clinical and commercial LYFGENIA drug product. We recently received FDA approval to expand our capacity at Lonza to

manufacture additional lots of ZYNTEGLO and SKYSONA. We also rely on specialized third parties for quality control testing with respect to our LVV and drug product.

We believe our team of technical personnel has extensive manufacturing, analytical and quality experience as well as strong project management discipline to oversee these contract manufacturing activities, and to compile manufacturing and quality information for our regulatory submissions and commercialization efforts. For the treatment of patients with our drug product in the commercial setting, we are collaborating with participating QTCs in the United States to be centers for collection of HSCs from the patient and for infusion of drug product to the patient.

In 2022, we paused efforts to demonstrate comparability of using cryopreserved patient starting material versus fresh patient starting material for the manufacture of LYFGENIA drug product to focus our resources on BLA approvals and product launches. We continue to evaluate plans to reinitiate and advance cryopreserved starting material. If these efforts are successful, including if cryopreservation is determined to be technically feasible and is approved by the FDA and implemented with respect to LYFGENIA, we believe cryopreservation could (i) expand the patient population that our therapies could potentially serve by improving patient and QTC experience by reducing the number of mobilization cycles, and (ii) lower our cost of production by reducing the need for multiple manufacturing runs per patient.

Commercial operations

In 2023, we established a commercial footprint with the launches of ZYNTEGLO and SKYSONA. Now in 2024, we are deploying a validated commercial strategy for the launch of LYFGENIA, which we have worked to enhance throughout our significant head start against our closest competitor.

Our commercial strategy centers on three key components:

- A robust and synergistic qualified treatment center network
- Timely and equitable access and reimbursement
- An optimized patient and provider experience

In order to treat patients, transplant centers must become part of our QTC network and supply chain. As of August 14, 2024, we had activated more than 70 total QTCs (defined as having a signed master services agreement) for ZYNTEGLO and LYFGENIA. ZYNTEGLO commercial launch operations are highly synergistic with LYFGENIA – with the same treatment centers, treaters, and patient services – which has allowed for a simplified activation process for LYFGENIA QTCs. QTCs were selected based on their expertise in areas such as transplant, cell, and gene therapy, and are trained to administer our therapies. Our fully activated network was designed with patients in mind to optimize the patient experience. We expect to continue to scale our QTC network throughout 2024.

We believe that our validated access and reimbursement strategy is driving a favorable coverage landscape for LYFGENIA and ZYNTEGLO. For ZYNTEGLO, we have set a wholesale acquisition cost of $2.8 million. To help enable timely and quality access for patients with beta-thalassemia, we have established outcomes-based agreements with both commercial and Medicaid payers, under which we will reimburse contracted commercial and government payers up to 80% of the cost of the therapy if a patient does not meet designated clinical outcomes. For ZYNTEGLO, we offer reimbursement to payers in the event that a patient fails to achieve and maintain transfusion independence up to two years following infusion. All patients in ZYNTEGLO Phase 3 studies who achieved transfusion independence have remained transfusion free as of September 11, 2024. Since launch, more than 200 million U.S. lives are covered through contracts or favorable coverage policies for ZYNTEGLO and there have been zero ultimate denials across both Medicaid and commercial payers.

For LYFGENIA, we are building upon these core components of our market access strategy. We have set a wholesale acquisition price of $3.1 million for LYFGENIA. Similar to ZYNTEGLO, we are offering outcomes-based agreements for LYFGENIA that provide government and commercial payers with meaningful risk sharing tied to clinical outcomes that can be tracked in claims data and offer payers reimbursement tied to VOE-related hospitalizations during a defined period of time (typically within three years of treatment). As of September 11, 2024, we have signed outcomes-based agreements for LYFGENIA with five national payer organizations representing dozens of downstream plans and covering approximately 200 million U.S. lives. These agreements are now being pulled through into favorable coverage policies, aligned to clinical trial criteria, and at parity to our closest competitor. In February 2024 we signed our first outcomes-based agreement with the state of Michigan. We continue to advance discussions with multiple Medicaid agencies and have secured favorable coverage policies or placement on the preferred drug list in over half of all states.

We continue to see strong linear growth for ZYNTEGLO with 19 patient starts (which we define as unique cell collection or apheresis) as of August 14, 2024 since the beginning of 2024. In 2023, there were 20 total patient starts completed for ZYNTEGLO. For LYFGENIA, as of August 14, 2024, we had completed four patient starts in 2024 and continue to anticipate patient starts to grow quarter over quarter with the majority occurring in the second half of 2024 as momentum builds. As of August 14, 2024, more than 40 patient starts were scheduled for ZYNTEGLO and LYFGENIA through the end of the year.

As of August 14, 2024, SKYSONA was available through a network of six activated QTCs. In 2024, we had completed four patient starts for SKYSONA as of August 14, 2024, in addition to the six patient starts that were completed for SKYSONA in 2023. We continue to anticipate five to ten patient starts for SKYSONA per year going forward. We believe payers recognize the value and urgency to treat these patients, and to date there have been no ultimate denials by payers for the therapy. We have set a wholesale acquisition cost of $3 million for SKYSONA.

In 2024, we anticipate approximately 85 patient starts combined across all three of our FDA approved therapies (LYFGENIA, ZYNTEGLO and SKYSONA).

For each of our products, following cell collection, the patient's cells are shipped to a manufacturing facility where they are transduced with the appropriate LVV and tested to ensure they meet stringent release specification criteria. They are then frozen and shipped back to the QTC where the patient receives treatment via IV infusion. Typically, patients receive their drug product infusion approximately two quarters after initial cell collection. Our supply chain for LYFGENIA is separate and distinct from our supply chain for SKYSONA and ZYNTEGLO. In all cases, delivering a consistent and reliable manufacturing process is a key part of delivering gene therapy and is essential for physicians, providers and for patients and their families.

For each of our therapies, we have established mybluebirdsupport, a patient support program that provides education and support navigating insurance and treatment.

While we believe we have largely established appropriate quality systems, compliance policies, systems and procedures, as well as internal systems and infrastructure necessary for supporting our complex supply chain and commercialization activities, we expect that we may make additional targeted investments as we continue our efforts to scale out capacity and our network of qualified treatment centers in support of the ZYNTEGLO and LYFGENIA launches, as well as establish patient-focused programs, educate healthcare professionals, and secure additional reimbursement.

Intellectual property

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

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

We have developed or in-licensed numerous patents and patent applications and possess substantial know-how and trade secrets relating to the development and commercialization of gene therapy products. Our proprietary intellectual property, including patent and non-patent intellectual property, is generally directed to, for example, certain genes, transgenes, methods of transferring genetic material into cells, genetically modified cells, processes to manufacture our lentivirus-based products and other proprietary technologies and processes related to our products. In connection with the wind down of our operations in Europe, we have decided to let certain non-U.S. patents and patent applications that we own lapse by ceasing further

prosecution of certain pending ex-U.S. patent applications and not paying future maintenance fees for certain ex-U.S. patents when due. As of September 11, 2024, our patent portfolio includes the following:

- approximately 12 patents or patent applications that we own or have exclusively in-licensed from third parties related to LVVs and vector systems;

- approximately 105 patents or patent applications that we own or have exclusively in-licensed from third parties related to LVV or drug product manufacturing and associated assays;

- approximately 16 patents or patent applications that we have non-exclusively in-licensed from third parties related to LVV or drug product manufacturing; and

- approximately 3 U.S. patents or patent applications that we own or have exclusively in-licensed from third parties related to therapeutic cellular product candidates and assays.

Our objective is to continue to expand our U.S. portfolio of patents and patent applications as needed in order to protect our gene therapy products and manufacturing processes. Examples of the products and technology areas covered by our intellectual property portfolio are described below. See also "—License agreements." From time to time, we also evaluate opportunities to sublicense our portfolio of patents and patent applications that we own or exclusively license, and we may enter into such licenses from time to time.

ZYNTEGLO and LYFGENIA

The ZYNTEGLO and LYFGENIA programs include the following patent portfolios described below.

- **RDF.** The in-licensed patent portfolio from Research Development Foundation ("RDF") in part, contains patents and patent applications directed to aspects of our LVV that may be utilized to produce ZYNTEGLO and LYFGENIA. As of September 11, 2024, we had an exclusive license (from RDF) to two issued U.S. patents related to our LVV platform. We expect the issued composition of matter patents to expire from 2025-2027 in the United States.

- **SIRION.** The in-licensed patent portfolio from SIRION Biotech GmbH ("SIRION") contains patents and patent applications directed to methods of manufacturing ex vivo gene therapy products with LVV. As of September 11, 2024, we had an exclusive license to three issued U.S. patents, one pending U.S. patent application, three corresponding foreign patent applications and 47 issued corresponding foreign patents. We expect the issued method patents to expire in 2033. We expect any other composition of matter and methods patents, if issued from the pending patent applications and if the appropriate maintenance, renewal, annuity or other governmental fees are paid, to expire in 2033. We expect any other patents and patent applications in this portfolio, if issued, and if the appropriate maintenance, renewal, annuity, or other governmental fees are paid, to expire in 2033 (worldwide).

SKYSONA

The SKYSONA program includes the following patent portfolios described below.

- **RDF.** The in-licensed RDF patent portfolio contains the patents and patent applications described above directed towards aspects of LVV that may be utilized to produce SKYSONA.

- **bluebird bio**. The bluebird bio patent portfolio contains patents and patent applications directed to compositions of matter for SKYSONA vectors and compositions and methods of using the vectors and compositions in cell-based gene therapy of adrenoleukodystrophy or adrenomyeloneuropathy. As of September 11, 2024, we owned three U.S. patents and 7 issued foreign patents. We expect the issued composition of matter patents for SKYSONA vectors to expire in 2032.

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

The lentiviral platform, which is potentially applicable across our programs in severe genetic disease, includes the following patent portfolios described below.

- **RDF.** The in-licensed RDF patent portfolio contains the patents and patent applications described above.

- **SIRION.** The in-licensed SIRION patent portfolio contains the patents and patent applications described above.

- **bluebird bio.** Another component of the bluebird bio patent portfolio includes LVV and drug product manufacturing platforms and is potentially applicable across our programs. This portion of the portfolio contains patents and patent applications directed to improved methods for transfection and transduction of therapeutic cells. As of September 11, 2024, we owned five issued U.S. patents, two pending U.S. patent applications and five corresponding foreign patent applications and 22 issued corresponding foreign patents. We expect the issued method patents to expire from 2032-2037. We expect composition of matter and method patents, if issued from the pending patent applications and if the appropriate maintenance, renewal, annuity or other governmental fees are paid, to expire from 2032-2037. We expect any other patents and patent applications in this portfolio, if issued, and if the appropriate maintenance, renewal, annuity, or other governmental fees are paid, to expire from 2032-2037 (worldwide).

- **2seventy bio.** The 2seventy bio patent portfolios include LVV manufacturing platforms and improvements and are potentially applicable across our programs. As of September 11, 2024, we had a non-exclusive license to four patent families that include three pending U.S. patent applications, 12 corresponding foreign patent applications and one pending PCT application. We expect any composition of matter or methods patents, if issued from a corresponding nonprovisional application or national stage application, or corresponding foreign applications, if applicable, and if the appropriate maintenance, renewal, annuity or other governmental fees are paid, to expire from 2040-2042 (worldwide). We expect any other patents and patent applications in this portfolio, if issued, and if the appropriate maintenance, renewal, annuity, or other governmental fees are paid, to expire from 2040-2042 (worldwide).

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the date of filing the non-provisional application. In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office in granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier-filed patent. A patent may be enforced during its term and/or up to six years after the patent has expired against past acts of infringement that occurred during the patent term.

We may rely, in some circumstances, on trade secrets to protect our technology. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and third parties. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our consultants or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

License agreements

Inserm-Transfert

In May 2009, we entered into an exclusive license with Inserm-Transfert SA, referred to hereafter as Inserm, which is a wholly-owned subsidiary of Institut national de la santé et de la recherche médicale, for use of certain patents and know-how related to the *ABCD1* gene and corresponding protein, for use in the field of human ALD therapy. The agreement was amended in 2012, 2013, 2014 and 2015. The last patent in the Inserm licensed patent portfolio expired in February of 2016.

We are obligated to pay Inserm a percentage of net sales as a royalty on any of our products that use the in-licensed intellectual property for the longer of the life of any in-licensed patents covering the product or 10 years from first commercial sale. Any net sales of SKYSONA are subject to this royalty which is in the low single digits.

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

We may unilaterally terminate the license agreement at any time. Either party may terminate the agreement in the event of the other party's material breach which remains uncured after 60 days of receiving written notice of such breach or in the event the other party becomes the subject of a voluntary or involuntary petition in bankruptcy and such petition is not dismissed with

prejudice within 120 days after filing. In addition, Inserm may terminate the license agreement in the event that we cannot prove within 60 days of written notice from Inserm that we have been diligent in developing products covered by in-licensed intellectual property and introducing them into the commercial market.

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

Institut Pasteur

In September 2011, we entered into a license with Institut Pasteur for certain patents relating to the use of DNA sequences, LVV and recombinant cells in the field of ex vivo gene therapy and CAR T cell-based therapy in a range of indications, excluding vaccinations. This agreement was amended twice in 2012, again in 2013 and most recently in 2015. The last patent in the Institut Pasteur licensed patent portfolio expired in 2023. The license is exclusive for products containing human and non-human LVV. Institut Pasteur retains the right, on behalf of itself, its licensees and research partners, to conduct research using the licensed intellectual property.

We have the right to grant sublicenses outright to third parties under the agreement. For the first sublicense including a product targeting β-hemoglobinopathies (including β-thalassemia and SCD) or ALD (including CALD and adrenomyeloneuropathy), we must pay Institut Pasteur an additional payment of €3.0 million. If we receive any income (cash or non-cash) in connection with sublicenses for products targeting indications other than β-hemoglobinopathies (including β-thalassemia and SCD) or ALD (including CALD and adrenomyeloneuropathy), we must pay Institut Pasteur a percentage of such income varying from low single digits if the sublicense also includes licenses to intellectual property controlled by us, and a percentage of sublicense income in the mid-range double digits if the sublicense does not include licenses to intellectual property controlled by us.

We are obligated to pay Institut Pasteur a percentage of net sales as a royalty on any of our commercialized products covered by the in-licensed intellectual property, which include ZYNTEGLO, SKYSONA and LYFGENIA. This royalty varies depending on the indication of the product but in any event is in the low single digits. The royalty is reduced if, during the five years following the first market authorization of a product covered by the in-licensed intellectual property, the last licensed patent expires. In addition, starting in 2016, we have been required to make an annual maintenance payment, offset by royalty payments on a year-by-year basis.

We are required to use all reasonable commercial efforts (as compared to a company of similar size and scope) to develop and commercialize one or more products in the license field and to obtain any necessary governmental approvals in respect of, and market the products in the license field, if any. Additionally, we have assumed certain development and regulatory milestone obligations. We must report on our progress towards achieving these milestones on an annual basis. We may unilaterally terminate the license agreement at any time by sending Institut Pasteur 90 days prior written notice. Either party may terminate the license in the event of the other party's substantial breach which remains uncured after 60 days of receiving written notice of such breach. Institut Pasteur may also terminate the agreement in the event bankruptcy proceedings are opened against us and not dismissed within 60 days.

Absent early termination, the agreement will automatically terminate upon the expiration of the last licensed patents or five years after first market authorization of the first product, whichever occurs later. In the event the agreement is terminated, while the license grant would cease, we would retain the right to manufacture, import, use and sell licensed products for a certain period of time post-termination. In addition, our ownership stake in certain jointly made improvements covered by the licensed patents would survive termination of the agreement. The longest-lived patent rights licensed to us under the agreement expired in 2023.

Stanford University

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

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

We are obligated to pay Stanford a percentage of net sales as a royalty on any of our commercialized products covered by the in-licensed intellectual property, which include ZYNTEGLO, SKYSONA and LYFGENIA. This royalty varies with net sales but in any event is in the low single digits and is reduced for each third-party license that requires payments by us with respect to a licensed product, provided that the royalty to Stanford is not less than a specified percentage which is less than one percent. Since April 2013, we have been paying Stanford an annual maintenance fee, which is creditable against our royalty payments on a year-by-year basis.

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

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

Research Development Foundation

In December 2011, we entered into an exclusive license with Research Development Foundation, which we refer to as RDF, to use certain patents that involve LVV. Any patents within this portfolio that have issued or may yet issue would have an expected statutory expiration date between 2021 and 2027. RDF retains the right, on behalf of itself and other nonprofit academic research institutions, to practice and use the licensed patents for any academic, non-clinical research and educational purposes. We have the right to grant sublicenses outright to third parties under the agreement.

We are obligated to pay RDF a percentage of net sales as a royalty on any of our commercialized products covered by the in-licensed intellectual property, which include ZYNTEGLO, SKYSONA and LYFGENIA. This royalty is in the low single digits and is reduced by half if during the following ten years from the first marketing approval the last valid claim within the licensed patent that covers the licensed product expires or ends.

We are required to use commercially reasonable and diligent efforts for a company of our size and resources to develop or commercialize products covered by the in-licensed intellectual property. We have also assumed certain regulatory milestone obligations and must report on our progress in achieving these milestones on an annual basis.

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

Absent early termination, the agreement will continue until its expiration upon the later of there being no more valid claims within the licensed patents or the expiration of our royalty obligations on licensed products that are subject to an earned royalty, if such earned royalty is based on the minimum 10-year royalty period described above. In the event the agreement is terminated, while the license grant would cease, RDF will grant our sublicensees a direct license. The longest-lived patent rights licensed to us under the agreement are in one U.S. patent currently expected to expire in 2027.

SIRION

In December 2015, we entered into a license agreement with SIRION Biotech Gmbh, which we refer to as SIRION, pursuant to which we exclusively licensed certain patents and patent applications directed towards aspects of manufacturing gene therapy products. Any patents within this portfolio that have issued or may yet issue would have an expected statutory expiration date in 2033. We have the right to grant sublicenses to third parties, subject to certain conditions.

We are obligated to pay SIRION a percentage of net sales as a royalty on any of our commercialized products covered by the in-licensed intellectual property, which includes ZYNTEGLO and LYFGENIA. These royalties are in the low single digits and are reduced for royalties payable to third parties, up to a cap.

We have also assumed certain development and regulatory milestones, and we must report on our progress in achieving those milestones on a periodic basis.

We may unilaterally terminate the license agreement at any time with prior written notice to SIRION. Either party may terminate the agreement in the event of a material breach that remains uncured following a notice period. Either party may also terminate the agreement in the event bankruptcy proceedings are opened against the other and are not dismissed within a specified period of time. Absent early termination, the agreement will automatically terminate upon the expiration of the patent rights covered by the agreement. The longest-lived patent rights licensed to us under the Agreement are currently expected to expire in 2033.

Competition

With three FDA approved gene therapies, we are now deploying a validated commercial strategy to bring our therapies to patients. We believe we are well-positioned to compete, with deep gene therapy expertise and a significant commercial head start against our closest competitor. We have established a robust QTC network to treat patients, the value of our therapy and the outcomes-based agreements we offer are resulting in rapid access, and we are optimizing the patient and provider experience based on our learnings from our ongoing launches.

However, the biotechnology and pharmaceutical industries are characterized by intense and rapidly changing competition to develop new technologies and proprietary products. Not only must we compete with other companies that are focused on gene therapy products, but any products that we commercialize will compete with existing therapies and new therapies that may become available in the future. Many of our competitors, either alone or with their strategic partners, have substantially greater financial, technical, and human resources than we do and significantly greater experience obtaining FDA and other regulatory approvals of therapies and commercializing those therapies. Accordingly, our competitors may be more successful than us in achieving widespread market acceptance in the U.S. and global markets. Our competitors' therapies may be more effective, or more effectively marketed and sold, than any therapy we may commercialize and may render our treatments obsolete or non-competitive before we can recover the expenses of developing and commercializing any of our therapies.

These competitors also compete with us in recruiting and retaining qualified commercial, scientific and management personnel, establishing clinical study sites, and registering patients for clinical studies, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

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

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize therapies or conditioning regimens that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any therapies that we may develop. Depending on how successful these competitive efforts are, it is possible they may increase the barriers to adoption and success for our therapies or result in greater access and reimbursement for competing products. In addition, our ability to compete may be affected in many cases by insurers or other third-party payers seeking to encourage the use of generic products or other lower-priced options. Competitors include the following:

Sickle cell disease — LYFGENIA's primary competitor is CASGEVY (exa-cel, marketed by Vertex Pharmaceuticals), which leverages the CRISPR/Cas9 gene editing platform to disrupt the BCL11A erythroid enhancer. Similar to LYFGENIA, CASGEVY is administered via autologous HSCT treatment. CASGEVY and LYFGENIA were both approved by the FDA on December 8, 2023.

The current standard of care for the treatment of SCD in the developed world is chronic blood transfusions or hydroxyurea (a generic drug). In addition, patients treated with chronic blood transfusions often receive iron chelation therapy to help manage the iron overload. We are aware of ongoing studies that continue to evaluate the efficacy and safety of hydroxyurea in various populations. In addition, a limited number of patients with SCD receive allogeneic HSCT treatment, particularly if a sufficiently well-matched source of donor cells is identified. There are a number of academic and industry-sponsored research

and development programs to improve the tolerability and safety of allogeneic HSCT with less well-matched sources of donor cells, while increasing the availability of suitable donors. There are also a number of other FDA approved therapies for SCD, including Endari (L-glutamine, marketed by Emmaus Life Sciences, Inc.), Oxbryta (voxelotor, an HbS polymerization inhibitor developed Global Blood Therapeutics, Inc., now marketed by Pfizer), and Adakveo (crizanlizumab, a p-selectin antibody marketed by Novartis). A number of different therapeutic approaches for the chronic treatment of SCD are under investigation targeting the various aspects of SCD pathophysiology, including: a pyruvate kinase receptor activator, mitapivat, in a Phase 2/3 trial supported by Agios Pharmaceuticals, Inc.; and a selective small molecule inhibitor of ectoderm development protein designed to increase the expression of fetal hemoglobin, pociredir, in a Phase 1 study supported by Fulcrum Therapeutics, Inc. There are additional gene therapy programs in development for SCD including programs from Editas Medicine, Inc. and Beam Therapeutics. Editas Medicine has reported early efficacy data from its Phase 1/2/3 study of EDIT-301 (reni-cel), which leverages the CRISPR/Cas12a gene editing platform to target the HBG1/2 promoter to upregulate HbF. Beam Therapeutics uses base editing to target the HGB1/2 promoter and upregulate HgF, and they continue to enroll patients in their Phase 1/2 trial.

β-thalassemia — ZYNTEGLO was the first FDA approved gene therapy for the treatment of adult and pediatric beta-thalassemia patients who require regular RBC transfusions in August 2022, and we initiated treatment of the first ZYNTEGLO patient in the commercial setting in December 2022. ZYNTEGLO's primary competitor is CASGEVY, which was approved by the FDA for the treatment of patients 12 and older with transfusion-dependent β-thalassemia in January 2024.

The current standard of care for the treatment of β-thalassemia in the developed world is chronic blood transfusions to address the patient's anemia. In addition, such patients often receive iron chelation therapy to help manage the iron overload associated with their chronic blood transfusions. Novartis and Chiesi, who provide the leading iron chelation therapies, are seeking to develop improvements to their product profile and accessibility. A number of different approaches are under investigation that seek to improve the current standard of care treatment options. Reblozyl (luspatercept), a subcutaneously-delivered protein therapeutic marketed by Merck and Bristol Myers Squibb that targets molecules in the TGF-β superfamily, has been approved in the United States for the treatment of anemia in adult patients with β-thalassemia who require regular red blood cell transfusions, and was approved in the European Union for the treatment of adult patients with transfusion-dependent anemia associated with β-thalassemia. Additionally, Agios is developing mitapivat for both transfusion-dependent and non-transfusion-dependent β-thalassemia, and has reported positive clinical data from two Phase 3 studies. Some patients with β-thalassemia receive HSCT treatment, particularly if a sufficiently well-matched source of donor cells is identified. In addition, there are a number of academic and industry-sponsored research and development programs to improve the outcomes of allogeneic HSCT, or the tolerability and safety of haploidentical HSCT, while increasing the availability of suitable donors.

CALD — The current standard of care for the treatment of CALD is allogeneic HSCT. Various academic centers around the world are seeking to develop improvements to allogeneic HSCT. Other possible treatments being investigated include Minoryx Therapeutics' MIN-102 (leriglitazone), and Viking Therapeutics' VK0214.

Government regulation

In the United States, biological products, including gene therapy products, are subject to regulation under the Federal Food, Drug, and Cosmetic Act ("FD&C Act") and the Public Health Service Act ("PHS Act") and other federal, state, local and foreign statutes and regulations. Both the FD&C Act and the PHS Act and their corresponding regulations govern, among other things, the testing, manufacturing, safety, efficacy, purity, potency, labeling, packaging, storage, record keeping, distribution, import, export, reporting, record keeping, post-approval monitoring, advertising and other promotional practices involving biological products. FDA approval must be obtained before marketing certain biological products, including our products and any future product candidates. The process of obtaining regulatory approvals for lifecycle management, including post-approval changes associated with our approved products, and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources and we may not be able to obtain the required regulatory approvals. Further, we have ongoing clinical studies requiring health authorities to oversee and approve ongoing clinical activities.

U.S. biological products development process

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

- completion of nonclinical laboratory tests and animal studies according to good laboratory practices ("GLPs"), and other applicable regulations;

- submission to the FDA of an investigational new drug application ("IND"), which must become effective before human clinical studies may begin;

- approval by an institutional review board ("IRB"), or ethics committee at each clinical site before the trial commences;

- performance of adequate and well-controlled human clinical studies according to the FDA's regulations commonly referred to as good clinical practices ("GCPs") and any additional requirements for the protection of human research subjects and their health information, to establish the safety, purity and potency or efficacy of the proposed biological product for its intended use;

- preparation and submission to the FDA of a BLA for marketing approval that includes substantial evidence of safety, and efficacy from results of nonclinical testing and clinical studies;

- satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biological product is produced to assess compliance with GMP, to assure that the facilities, methods and controls are adequate to preserve the biological product's safety, identity, strength, quality, purity and potency, and, if applicable, the FDA's good tissue practices ("GTPs") for the human cellular and tissue products;

- potential FDA audit of the nonclinical and clinical study sites that generated the data in support of the BLA; and

- FDA review and approval, or licensure, of the BLA.

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

The clinical study sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature, a proposed clinical protocol and investigator information, to the FDA as part of the IND. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical study on a clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical study can begin. The FDA may also impose clinical holds on a biological product candidate at any time before or during clinical studies due to safety concerns or non-compliance. If the FDA imposes a clinical hold, studies may not recommence without FDA authorization and then only under terms authorized by the FDA. Accordingly, the submission of an IND may not result in the FDA allowing clinical studies to begin, or that, once begun, that such studies will be allowed to continue.

In addition to the IND submission process, under the National Institutes of Health ("NIH"), Guidelines for Research Involving Recombinant DNA Molecules ("NIH Guidelines"), supervision of human gene transfer trials includes evaluation and assessment by an institutional biosafety committee ("IBC"), a local institutional committee that reviews and oversees research utilizing recombinant or synthetic nucleic acid molecules at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment, and such review may result in some delay before initiation of a clinical trial. While the NIH Guidelines are not mandatory unless the research in question is being conducted at or sponsored by institutions receiving NIH funding of recombinant or synthetic nucleic acid molecule research, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them.

Clinical studies involve the administration of the biological product candidate to patients under the supervision of qualified investigators, generally physicians not employed by or under the study sponsor's control and studies must be conducted and monitored in accordance with the FDA's regulations comprising the GCP requirements, including, among other things, the requirement that all research subjects provide informed consent. Clinical studies are conducted under protocols detailing, among other things, the objectives of the clinical study, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical study will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND. While the IND is active, annual progress reports detailing the results of the clinical trials and nonclinical studies performed since the last progress report, among other information, must be submitted to the FDA, and written IND safety reports must be promptly submitted to the FDA and the investigators for serious and unexpected adverse events, any findings from other studies, tests in laboratory animals or in vitro testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure.

Further, each clinical study must be reviewed and approved by an IRB at or servicing each institution at which the clinical study will be conducted. An IRB is charged with protecting the welfare and rights of study participants and considers such items as whether the risks to individuals participating in the clinical studies are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical study subject or his or her legal representative and must monitor the clinical study until completed. The FDA or the sponsor may suspend, or its data safety monitoring board may recommend suspension of a clinical study at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical study at its institution if the clinical study is not being conducted in accordance with the IRB's requirements or if the biological product candidate has been associated with unexpected serious harm to patients.

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

- *Phase 1.* The biological product is initially introduced into healthy human subjects or patients with the target disease or condition. These studies are designed to test, among other things, the safety, dosage tolerance, absorption, metabolism and distribution, of the investigational product in humans, the side effects associated with increasing doses, and if possible, to gain early evidence on effectiveness.

- *Phase 2.* The biological product is evaluated in a limited patient population with the specified disease or condition to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.

- *Phase 3.* The biological product is administered to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy, and to further test for safety, generally at geographically dispersed clinical study sites. These clinical studies are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product labeling.

Post-approval clinical studies, sometimes referred to as Phase 4 clinical studies, may be conducted after initial marketing approval. These clinical studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up. The FDA may also require, or sponsors may voluntarily decide, to continue evaluating patients in ongoing clinical trials to gather additional information regarding the long-term effects of any approved products. For example, the FDA recommends that sponsors of certain gene therapy clinical trials observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by ten years of annual queries, either in person or by questionnaire, of study subjects.

Concurrent with clinical studies, companies usually complete additional animal studies and must also develop additional information about the physical characteristics of the biological product as well as finalize a process for manufacturing the product in commercial quantities in accordance with GMP requirements. To help reduce the risk of the introduction of adventitious agents with use of biological products, the PHS Act emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the sponsor must develop methods for testing the safety, identity, strength, quality, potency and purity of the final biological product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that biological product quality is maintained over its defined shelf life.

U.S. review and approval processes

FDA approval of a BLA must be obtained before commercial marketing of the biological product candidate. The BLA must include results of product development, including results from laboratory and animal studies, human studies, information on the manufacture and composition of the product, proposed labeling and other relevant information. Data can come from company-sponsored clinical studies intended to test the safety and effectiveness of a use of the product, or from a number of alternative sources, including studies initiated by independent investigators. In addition, under the Pediatric Research Equity Act ("PREA") as amended, a BLA or supplement to a BLA must contain data to assess the safety and effectiveness of the biological product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers of the PREA requirements. Unless otherwise required by regulation, PREA does not apply to any biological product for an indication for which orphan designation has been granted.

Within 60 days following submission of the application, the FDA reviews a BLA submitted to determine if it is substantially complete before the agency accepts it for filing. The FDA may refuse to file any BLA that it deems incomplete or

not properly reviewable at the time of submission and may request additional information. In this event, the BLA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the BLA. The FDA reviews the BLA to determine, among other things, whether the proposed product is safe, pure and potent, or effective, for its intended use, and has an acceptable purity profile, and whether the product is being manufactured in accordance with GMP to assure and preserve the product's identity, safety, strength, quality, potency and purity. The FDA's goal is to review standard applications within ten months after the filing date, or, if the application qualifies for priority review, six months after the FDA accepts the application for filing. In both standard and priority reviews, the review process may also be extended by FDA to review additional information deemed a "major amendment" to the application.

The FDA may refer applications for novel biological products or biological products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review and evaluation, potentially including a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

During the biological product approval process, the FDA also will determine whether a Risk Evaluation and Mitigation Strategy ("REMS") is necessary to assure the safe use of the biological product. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

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

After the FDA evaluates a BLA and conducts inspections, the FDA may issue an approval letter or a Complete Response Letter ("CRL"). An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A CRL will describe all deficiencies that the FDA has identified in the BLA, except that where the FDA determines that the data supporting the application are inadequate to support approval, the FDA may issue the CRL without first conducting required inspections, testing submitted product lots, and/or reviewing proposed labeling. In issuing the CRL, the FDA may recommend actions that the applicant might take to place a resubmitted BLA in condition for approval, including requests for additional information or clarification, or requirements for additional clinical or nonclinical testing. The FDA may delay or refuse to approve a BLA if applicable regulatory criteria are not satisfied.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing, or dispensing in the form of a REMS, or otherwise limit the scope of any approval. In addition, the FDA may require post marketing clinical studies, sometimes referred to as Phase 4 clinical studies, designed to further assess a biological product's safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

Orphan drug designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biological product intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making a drug or biological product available in the United States for this type of disease or condition will be recovered from sales of the product. Orphan product designation must be requested before submitting a BLA. After the FDA grants orphan product designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the

FDA. Orphan product designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug or biological product for the same disease or condition for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity. The designation of such drug also entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, exemptions from PREA requirements and BLA user-fee waivers. Competitors, however, may receive approval of different products for the disease or condition for which the orphan product has exclusivity or obtain approval for the same product but for a different disease or condition for which the orphan product has exclusivity. Orphan product exclusivity also could block the approval of a product for seven years if a competitor obtains approval of the same biological product as defined by the FDA or if the product candidate is determined to be contained within the competitor's product for the same condition or disease. If a drug or biological product designated as an orphan product receives marketing approval for a disease or condition broader than what is designated, it may not be entitled to orphan product exclusivity.

Expedited development and review programs

The FDA has a Fast Track program that is intended to expedite or facilitate the process for reviewing drugs and biological products that meet certain criteria. Specifically, drugs and biological product candidates are eligible for Fast Track designation if they are intended to treat a serious or life-threatening condition and demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. The product candidate sponsor may request the FDA to designate the drug or biologic as a Fast Track product at any time during the clinical development of the product candidate. Fast Track designation provides opportunities for more frequent interactions with the FDA, and with regard to a Fast Track product candidate, the FDA may consider for review sections of the marketing application on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the application, the FDA agrees to accept sections of the application and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the application.

A biological product candidate intended to treat a serious or life-threatening disease or condition may also be eligible for Breakthrough Therapy designation to expedite its development and review. A product candidate can receive Breakthrough Therapy designation if preliminary clinical evidence indicates that the product candidate, alone or in combination with one or more other drugs or biologics, may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes all Fast Track designation features, as well as more intensive FDA interaction and guidance beginning as early as Phase 1 and an organizational commitment to expedite the development and review of the product candidate, including involvement of senior managers. Moreover, an investigational drug or biologic is eligible for RMAT designation if: (1) it meets the definition of a regenerative medicine therapy, which is defined 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) it is intended to treat, modify, reverse, or cure a serious disease or condition; and (3) preliminary clinical evidence indicates that the investigational drug has the potential to address unmet medical needs for such disease or condition. In a February 2019 final guidance, the FDA also stated that certain gene therapies that lead to a sustained effect on cells or tissues may meet the definition of a regenerative medicine therapy. RMAT designation provides potential benefits that include more frequent meetings with FDA to discuss the development plan for the product candidate, eligibility for rolling review and priority review of BLAs.

Any product candidate submitted to the FDA for marketing, including those receiving Fast Track designation, Breakthrough Therapy designation or RMAT designation, may be eligible for other types of FDA programs intended to expedite development and review, such as priority review. A BLA is eligible for priority review if the product candidate is designed to treat a serious condition, and if approved would provide a significant improvement safety or effectiveness compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for designated for priority review in an effort to facilitate the review. Additionally, depending on the design of the applicable clinical studies, a product candidate may be eligible for accelerated approval. In particular, drug or biological products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval, which means that they may be approved on the basis of the FDA's determination that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, such as an intermediate endpoint, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.

As a condition of approval, the FDA generally requires that a sponsor of a drug or biological product receiving accelerated approval perform adequate and well-controlled confirmatory clinical studies, and may require such confirmatory studies to be underway prior to granting any accelerated approval. Failure to conduct required confirmatory trials in a timely manner, or to verify a clinical benefit during such confirmatory trials, will allow the FDA to withdraw the approved biologic product from the market on an expedited basis. In addition, the FDA currently requires as a condition for accelerated approval pre-submission of promotional materials, which could adversely impact the timing of the commercial launch of the product.

Fast Track designation, Breakthrough Therapy designation, RMAT designation priority review and accelerated approval do not change the standards for approval but may expedite the development or approval process. Even if a product candidate qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

Post-approval requirements

Maintaining compliance with applicable federal, state, and local statutes and regulations requires the expenditure of substantial time and financial resources. Rigorous and extensive FDA regulation of biological products continues after approval, particularly with respect to GMP. Manufacturers of approved products are required to comply with applicable requirements in the GMP regulations, including quality control and quality assurance and maintenance of records and documentation. Other post-approval requirements applicable to biological products, include reporting of GMP deviations that may affect the identity, potency, purity and overall safety of a distributed product, record-keeping requirements, reporting of adverse effects, reporting updated safety and efficacy information, and complying with electronic record and signature requirements. After a BLA is approved, the product also may be subject to official lot release. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products before releasing the lots for distribution by the manufacturer.

Biological product manufacturers and other entities involved in the manufacture and distribution of approved biological products are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with GMPs and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain GMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved BLA, including withdrawal of the product from the market. In addition, changes to the manufacturing process or facility generally require prior FDA approval before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval. In addition, companies that manufacture or distribute drug or biological products or that hold approved BLAs must comply with other regulatory requirements, including submitting annual reports, reporting information about adverse drug experiences, and maintaining certain records. Newly discovered or developed safety or effectiveness data may require changes to a drug's approved labeling, including the addition of new warnings and contraindications, and also may require the implementation of other risk management measures, including a REMS or the conduct of post-marketing studies to assess a newly-discovered safety issue.

Manufacturers must comply with the FDA's advertising and promotion requirements, such as those related to direct-to-consumer advertising and advertising to healthcare professionals, the prohibition on promoting products for uses or in patient populations that are not described in the product's approved labeling (known as "off-label use"), industry-sponsored scientific and educational activities, and promotional activities involving the internet. Discovery of previously unknown problems or the failure to comply with the applicable regulatory requirements may result in restrictions on the marketing of a product or withdrawal of the product from the market as well as possible civil or criminal sanctions. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant or manufacturer to administrative or judicial civil or criminal sanctions and adverse publicity. Consequences could include refusal to approve pending applications, withdrawal of an approval, clinical hold, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, mandated corrective advertising or communications with healthcare professionals, debarment, restitution, disgorgement of profits, or civil or criminal penalties.

U.S. patent term restoration

Depending upon the timing, duration and specifics of the FDA approval of the use of our product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application. Only one patent applicable to an approved biological product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The U.S. PTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may intend to apply for restoration of patent term for one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical studies and other factors involved in the filing of the relevant BLA.

Biosimilars and Exclusivity

A biological product can obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing regulatory exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of all existing exclusivity protections or patent term, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued "Written Request" for such a study.

The Patient Protection and Affordable Care Act (the "Affordable Care Act") signed into law on March 23, 2010, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009 (the "BPCIA") which created an abbreviated approval pathway for biological products shown to be similar to, or interchangeable with, an FDA-licensed reference biological product. This amendment to the PHS Act attempts to minimize duplicative testing. Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product and, for products administered multiple times, the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic.

Under the BPCIA, an application for a 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. 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. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed "interchangeable" by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law. In addition, recent legislative and regulatory proposals have sought to reduce or altogether eliminate the distinctions between interchangeable products and conventional biosimilar products, making the long-term status of these products unclear.

Healthcare Laws

In addition to restrictions on marketing of pharmaceutical products, several other types of state/ federal laws and trade association membership codes of conduct have been applied to restrict certain marketing practices in the pharmaceutical industry in recent years. These laws include without limitation, state and federal anti-kickback, fraud and abuse, false claims, data privacy and security and transparency laws and regulations with respect to drug pricing and payments and other transfers of value made to healthcare professionals.

The U.S. federal Anti-Kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration, directly or indirectly, in cash or in kind, to induce or in return for purchasing, leasing, ordering or arranging for or recommending the purchase, lease or order of any healthcare item or service reimbursable, in whole or in part, under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand and prescribers, purchasers, and formulary managers on the other. A person or entity need not have actual knowledge of the federal Anti-Kickback Statute or specific intent to violate it in

order to have committed a violation. Although there are a number of statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution, the exemptions and safe harbors are drawn narrowly and practices that involve remuneration to those who prescribe, purchase, or recommend pharmaceutical and biological products, including certain discounts, or engaging healthcare professionals or patients as speakers or consultants, may be subject to scrutiny if they do not fit squarely within the exemption or safe harbor.

The U.S. federal civil False Claims Act prohibits, among other things, any person from knowingly presenting, or causing to be presented, a false or fraudulent claim for payment of government funds, or knowingly making, using, or causing to be made or used, a false record or statement material to an obligation to pay money to the government or knowingly concealing or knowingly and improperly avoiding, decreasing, or concealing an obligation to pay money to the federal government. Manufacturers can be held liable under the False Claims Act even when they do not submit claims directly to government payers if they are deemed to "cause" the submission of false or fraudulent claims. The False Claims Act also permits a private individual acting as a "whistleblower" to bring actions on behalf of the federal government alleging violations of the False Claims Act and to share in any monetary recovery. In recent years, several pharmaceutical and other healthcare companies have faced enforcement actions under the federal False Claims Act for, among other things, allegedly submitting false or misleading pricing information to government health care programs and providing free product to customers with the expectation that the customers would bill federal programs for the product. Other companies have faced enforcement actions for causing false claims to be submitted because of the company's marketing the product for unapproved, and thus non-reimbursable, uses. Federal enforcement agencies also have shown increased interest in pharmaceutical companies' product and patient assistance programs, including reimbursement and co-pay support services, and a number of investigations into these programs have resulted in significant civil and criminal settlements. 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 federal civil False Claims Act. Criminal prosecution is possible for making or presenting a false or fictitious or fraudulent claim to the federal government.

The federal Civil Monetary Penalties Law prohibits, among other things, the offering or transferring of remuneration to a Medicare or Medicaid beneficiary that the person knows or should know is likely to influence the beneficiary's selection of a particular supplier of Medicare or Medicaid payable items or services.

The Health Insurance Portability and Accountability Act of 1996 ("HIPAA") also created several new federal crimes, including healthcare fraud and false statements relating to healthcare matters. The healthcare fraud statute prohibits knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private third-party payers. The false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation.

The U.S. federal Physician Payment Sunshine Act, being implemented as the Open Payments Program, requires certain manufacturers of drugs, devices, biologics and medical supplies to engage in extensive tracking of payments and other transfers of value to physicians (defined to include doctors, dentists, optometrists, podiatrists, chiropractors, certain non-physician practitioners (physician assistants, nurse practitioners, clinical nurse specialists, certified registered nurse anesthetists, anesthesiologist assistants, and certified nurse midwives) and teaching hospitals, including physician ownership and investment interests, and public reporting of such data. Pharmaceutical and biological manufacturers with products for which payment is available under Medicare, Medicaid or the State Children's Health Insurance Program are required to track such payments, and must submit a report on or before the 90th day of each calendar year disclosing reportable payments made in the previous calendar year. A number of other countries, states and municipalities have also implemented additional payment tracking and reporting requirements, which if not done correctly may result in additional penalties.

In addition, the U.S. Foreign Corrupt Practices Act ("FCPA") prohibits corporations and individuals from engaging in certain activities to obtain or retain business or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any official of another country, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in that capacity. In many other countries, healthcare professionals who prescribe pharmaceuticals are employed by government entities, and the purchasers of pharmaceuticals are government entities. Our dealings with these prescribers and purchasers may be subject to the FCPA.

The majority of states also have statutes or regulations similar to the federal anti-kickback and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer. Several states also require pharmaceutical companies to report expenses relating to the marketing and promotion of

pharmaceutical products in those states and to report gifts and payments to individual health care providers in those states. Some of these states also prohibit certain marketing-related activities including the provision of gifts, meals, or other items to certain health care providers. In addition, some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring manufacturers to report information related to payments to physicians and other healthcare providers, marketing expenditures, and drug pricing information. Certain state and local laws require the registration of pharmaceutical sales representatives.

Violations of any of these laws or other applicable governmental regulations may result in significant penalties, including civil monetary penalties, damages, exclusion of an entity or individual from participation in government healthcare programs, additional reporting obligations and oversight if the government requires a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, criminal fines and imprisonment, as well as the potential curtailment or restructuring of our 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.

Data Privacy and Security Laws

Numerous state, federal and foreign laws, regulations and standards govern the collection, use, access to, confidentiality and security of health-related and other personal information, and could apply now or in the future to our operations or the operations of our partners. In the United States, numerous federal and state laws and regulations, including data breach notification laws, health information privacy and security laws and consumer protection laws and regulations govern the collection, use, disclosure, and protection of health-related and other personal information. In addition, certain foreign laws govern the privacy and security of personal data, including health-related data. Privacy and security laws, regulations, and other obligations are constantly evolving, may conflict with each other to complicate compliance efforts, and can result in investigations, proceedings, or actions that lead to significant civil and/or criminal penalties and restrictions on data processing.

Pricing, Coverage and Reimbursement

Uncertainty exists as to the coverage and reimbursement status of any drug products for which we obtain regulatory approval. In the United States, sales of any products for which we may receive regulatory approval for commercial sale will depend in part on the availability of reimbursement from third-party payers, including governments. In the United States, no uniform policy of coverage and reimbursement for drug products exists among third-party payers. Therefore, coverage and reimbursement for drug products can differ significantly from payer to payer. Third-party payers can include government healthcare systems, managed care providers, private health insurers and other organizations. The process for determining whether a payer will provide coverage for a drug product may be separate from the process for setting the price or reimbursement rate that the payer will pay for the drug product. Moreover, these processes vary in length and may take several weeks, months, or longer to conclude depending on the individual payer. Third-party payers may limit coverage to specific drug products on an approved list, or formulary, which might not include all FDA-approved drugs for a particular indication. Third-party payers may provide coverage, but place stringent limitations on such coverage, such as requiring alternative treatments to be tried first. These third-party payers are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety, efficacy, and overall value. In addition, significant uncertainty exists as to the reimbursement status of newly approved healthcare products. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to incurring the costs required to obtain FDA approvals. Our product candidates may not be considered medically reasonable or necessary or cost-effective. Even if a drug product is covered, a payer's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself or the treatment or procedure in which the product is used may not be available, which may impact physician utilization. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

The marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and third-party payers fail to provide adequate coverage and reimbursement. In addition, the emphasis on managed care in the United States has increased, and we expect will continue to exert downward pressure on pricing. Moreover, the policies of traditional insurance marketplace entities, like reinsurers and stop-loss carriers, toward gene therapies in general or our gene therapies in particular, can negatively impact payer decisions about coverage and reimbursement. Similarly, the policies of self-insured employers toward gene therapies in general or our gene therapies in particular, including the potential to

exclude coverage, can negatively impact our ability to market our therapies. Coverage policies, third-party reimbursement rates and pharmaceutical pricing regulations may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

We have proposed novel payment models, including outcomes-based contracts to assist with realizing the value and sharing the risk of a potential one-time treatment, such as for ZYNTEGLO and LYFGENIA. While we have signed outcomes-based contracts with several State Medicaid Agencies and with pharmacy benefit managers (PBMs) representing dozens of downstream insurance plans and are engaged in discussions with additional PBMs and national payers, there is no assurance that these payment models will be widely adopted. Even with these payment models, there may be substantial resistance to the cost of our products by payers and the public generally. These payment models may not be sufficient for payers to grant coverage and/or cover our therapy at parity with our competitors, and if we are unable to obtain adequate coverage for our products, the adoption of our products and access for patients may be limited. In addition, to the extent reimbursement for our products is subject to outcomes-based arrangements, our future revenues from product sales will be more at risk (though based on the structure of our contracts, this risk is quantifiable and relatively predictable). These factors could affect our ability to successfully commercialize our products and adversely impact our business, financial condition, results of operations and prospects.

Government Price Reporting

Medicaid is a joint federal and state program administered by the states for low income and disabled beneficiaries. Medicare is a federal program that is administered by the federal government covering individuals aged 65 and over as well as those with certain disabilities. We are enrolled in the Medicaid Drug Rebate Program ("MDRP"). Under the MDRP, as a condition of federal funds being made available for our covered outpatient drugs under Medicaid and for 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 the U.S. Centers for Medicare & Medicaid Services ("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. This information is used to compute Medicare Part B payment rates, which consist of ASP plus a specified percentage. If we become aware that our MDRP price reporting submission for a prior period was incorrect or has changed based on recalculation of the pricing data, we must resubmit the revised data for up to three years after the original due date. We may request to resubmit pricing data outside the three-year period if the change is a result of an outcomes-based agreement, when the outcome must be evaluated outside of the three-year period. If we fail to provide information timely 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 a manufacturer that participates in the MDRP also participate in the Public Health Service's 340B drug pricing program (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 the Health Resources and Services Administration ("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 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 U.S. Department of Veterans Affairs ("VA") Federal Supply Schedule ("FSS") pricing program. Under the VA/FSS program, we must report the Non-Federal Average Manufacturer Price ("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. 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 a number of states may impose civil monetary penalties or pursue other enforcement mechanisms against manufacturers who fail to comply with drug price transparency requirements.

Healthcare Reform and Potential Changes to Healthcare Laws

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 new Medicare Part D coverage gap discount program; 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 a 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 and on August 15, 2024, HHS announced the agreed upon prices. HHS has issued and will continue to issue guidance implementing the IRA, although the Medicare drug

price negotiation program is currently subject to legal challenges. While the direct impact of the IRA on the pharmaceutical industry and the indirect impact on our business cannot yet be fully determined, it is likely to be significant.

Additionally, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug and biologic pricing, reduce the cost of prescription drugs and biologics under Medicare, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies for drugs and biologics.

Individual states in the United States have also become increasingly active in passing legislation and implementing 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 cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that governments and third-party payers will pay for healthcare products and services.

Human capital

As of June 30, 2024, we had 375 full-time employees, 54 of whom have Ph.D., M.D. or Pharm.D. degrees. Of these full-time employees, 221 employees are engaged in research and development activities and 154 employees are engaged in commercial, finance, legal, business development, human resources, information technology, facilities and other general administrative functions. We have no collective bargaining agreements with our employees, and we have not experienced any work stoppages. We are an organization committed to creating an inclusive and engaged culture that meets the needs of the whole employee, through competitive total rewards and retention efforts and commitment to Diversity, Equity, Inclusion and Belonging.

Compensation and benefits programs

Our compensation programs are designed to align our employees' interests with the drivers of growth and stockholder returns by supporting the Company's achievement of its primary business goals. Our goal is to attract and retain employees whose talents, expertise, leadership, and contributions are expected to sustain growth and drive long-term stockholder value. Consequently, we provide employee wages and benefits that are competitive within our industry, and we engage a nationally recognized outside compensation and benefits consulting firm to independently evaluate the effectiveness of our compensation and benefit programs and to provide benchmarking against our peers within the industry. Through our pay-for-performance culture, we seek to align our employees' interests with those of stockholders by linking annual changes in compensation to overall Company performance, as well as each individual’s contribution to the results achieved. The emphasis on overall Company performance is intended to align the employee’s financial interests with the interests of stockholders. We are also committed to providing comprehensive benefit options and it is our intention to offer benefits that will allow our employees and their families to live healthier and more secure lives. All employees are eligible for medical, dental, and vision insurance, paid and unpaid leaves, employee stock purchase plan, 401(k) plan, and group life and disability coverage.

Employee development and training

The development, recruitment and retention of our employees is a critical success factor for our company. To ensure we provide a meaningful experience for our employees, we regularly measure organizational culture and engagement, to build on the competencies that are important for our future success. We are building a robust talent and succession planning process and have established programs to support the talent pipeline for critical roles throughout our organization, to help us identify, foster, and retain high performing employees. To empower our employees to realize their potential at bluebird, we provide a range of development programs, opportunities and resources they need to be successful, to improve performance and retention, increase our organizational learning and support the promotion of our current employees.

Diversity

We believe Diversity, Equity, Inclusion and Belonging ("DEIB") are the cornerstone to an engaged, successful, and innovative organization. We are committed to taking action to help address racial injustice and inequality. We established our

DEIB steering committee that includes employees at all levels to provide oversight and guidance to establishing meaningful measures and actions to continue to increase DEIB at all levels and experiences. With significant input from employees and leaders at bluebird, we have adopted corporate goals to increase diversity and representation across our employee population.

Corporate Information

We were incorporated in Delaware in April 1992 under the name Genetix Pharmaceuticals Inc., and subsequently changed our name to bluebird bio, Inc. in September 2010. Our mailing address and executive offices are at 455 Grand Union Boulevard, Somerville, Massachusetts, and our telephone number at that address is (339) 499-9300. We maintain an Internet website at the following address: www.bluebirdbio.com. The information on our website is not incorporated by reference in this annual report on Form 10-K or in any other filings we make with the Securities and Exchange Commission ("SEC").

We make available on or through our website certain reports and amendments to those reports that we file with or furnish to the SEC in accordance with the Securities Exchange Act of 1934, as amended. These include our annual reports on Form 10-K, our quarterly reports on Form 10-Q, and our current reports on Form 8-K, and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act. We make this information available on or through our website free of charge as soon as reasonably practicable after we electronically file the information with, or furnish it to, the SEC.

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 Annual Report on Form 10-K, 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 II, Item 7 "Management's Discussion and Analysis of Financial Condition and Results of Operations—Liquidity and Capital Resources" of this Annual Report on Form 10-K 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 have been delayed in filing this Annual Report on Form 10-K and the Quarterly Reports for each of the periods ended March 31, 2024 and 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 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 $\leq$ 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 I, Item 3, "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 Annual 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 have been delayed in filing this Annual Report on Form 10-K and our Quarterly Reports on Form 10-Q for each of the periods ended March 31, 2024 and June 30, 2024, and 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 "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 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 the three-month periods ended March 31, 2024 and June 30, 2024. In order to regain compliance with the Listing Rule, we must file our Annual Report and both Quarterly Reports by October 14, 2024. We have filed this Form 10-K and our Form 10-Q for the three months ended March 31, 2024; however, we have not yet filed our Form 10-Q for the quarter ended June 30, 2024.

If we fail to comply with the Listing Rule by October 14, 2024, 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. There can be no assurance that we will come back into compliance with the Listing Rule or that we will not receive future notifications regarding noncompliance with any of the requirements for continued listing on Nasdaq.

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 I, Item 3, "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 1B. Unresolved Staff Comments

Not applicable.

Item 1C. Cybersecurity

Cybersecurity Risk Management and Strategy

We have developed and implemented a cybersecurity risk management program intended to protect the confidentiality, integrity, and availability of our critical systems and information. Our cybersecurity risk management program includes a cybersecurity incident response plan.

We design and assess our program based on the National Institute of Standards and Technology Cybersecurity Framework (NIST CSF). This does not imply that we meet any particular technical standards, specifications, or requirements, only that we use the NIST CSF as a guide to help us identify, assess, and manage cybersecurity risks relevant to our business.

We evaluate cybersecurity risk as part of our overall enterprise risk management program, using systematic methodologies, reporting channels and governance processes that apply across the broader enterprise risk management program.

Our cybersecurity risk management program includes:

- risk assessments designed to help identify material cybersecurity risks to our critical systems, information, products, services, and our broader enterprise information technology environment;
- a security team principally responsible for managing (1) our cybersecurity risk assessment processes, (2) our security controls, and (3) our response to cybersecurity incidents;
- the use of external service providers, where appropriate, to assess, test or otherwise assist with aspects of our security controls;
- cybersecurity awareness training of our employees, incident response personnel, and senior management, including training on best practices for data privacy and security and safekeeping of patient information;
- a cybersecurity incident response plan that includes procedures for responding to cybersecurity incidents; and
- a third-party risk management process for information technology software service providers, suppliers, and vendors that have access to our critical systems and information.

We have not identified risks from known cybersecurity threats, including as a result of any prior cybersecurity incidents, that have materially affected or are reasonably likely to materially affect us, including our operations, business strategy, results of operations, or financial condition. For more information, see the section titled "Risk Factor— 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 our 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."

Cybersecurity Governance

Our Board considers cybersecurity risk as part of its risk oversight function and has delegated to the Audit Committee oversight of cybersecurity and other information technology risks. The Audit Committee oversees management's implementation of our cybersecurity risk management program.

The Audit Committee receives periodic reports from management on our cybersecurity risks. In addition, management updates the Audit Committee, as necessary, regarding any material cybersecurity incidents, as well as any incidents with lesser impact potential.

The Audit Committee reports to the full Board regarding its activities, including those related to cybersecurity. The full Board also receives briefings from management on our cyber risk management program. Board members receive presentations on cybersecurity topics from our Head of Information Technology, internal security staff or external experts as part of the Board's continuing education on topics that impact public companies.

Our management team, including our Head of Information Technology and our Head of Information Security, is responsible for assessing and managing our material risks from cybersecurity threats. The team has primary responsibility for our overall cybersecurity risk management program and supervises both our internal cybersecurity personnel and our retained external cybersecurity consultants. Our management team's experience includes more than 20 years of information technology operations management across the pharmaceutical industry.

Our management team supervises efforts to prevent, detect, mitigate, and remediate cybersecurity risks and incidents through various means, which may include briefings from internal security personnel; threat intelligence and other information obtained from governmental, public or private sources, including external consultants engaged by us; and alerts and reports produced by security tools deployed in the information technology environment.

Item 2. Properties

Below is a summary of our material owned and leased properties as of December 31, 2023:

Our current corporate headquarters encompasses approximately 61,180 square feet of office space and is located at 455 Grand Union Boulevard, Somerville, Massachusetts. The lease commenced in March 2022 and will continue until December 31, 2032.

In October 2022, we entered into a sublease with Finch Therapeutics, Inc. ("Finch") for 42,162 square feet of office and laboratory space at Finch's corporate headquarters located at 100 Hood Park Drive, Charlestown, Massachusetts which Finch leases from Hood Park, LLC. This space is primarily used for our laboratory space. This sublease commenced December 15, 2022 and is expected to terminate on December 14, 2025.

In April 2019, we entered into a sublease agreement for approximately 267,278 square feet of office space located at 50 Binney Street, Cambridge, Massachusetts. In December 2021, we entered into a sub-sublease agreement with Meta Platforms, Inc. to sublease the entirety of the 50 Binney Street premises which we have rights to under the sublease. The sub-sublease commenced April 1, 2022, and is expected to terminate on December 31, 2030.

We believe that our existing facilities are adequate for our current needs.

Item 3. 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 October 7, 2024, SRT's responsive brief is due November 13, 2024, and our reply brief is due December 4, 2024. We have requested an extension of the opening brief until December 6, 2024, which will adjust the responsive and reply brief deadlines if granted. 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 our reply is currently due September 17, 2024. 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, which remain pending. 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. Parties must submit a scheduling order to the Court by September 23, 2024.

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. Parties must submit a scheduling order to the Court by September 23, 2024.

Item 4. Mine Safety Disclosures

Not applicable.

PART II

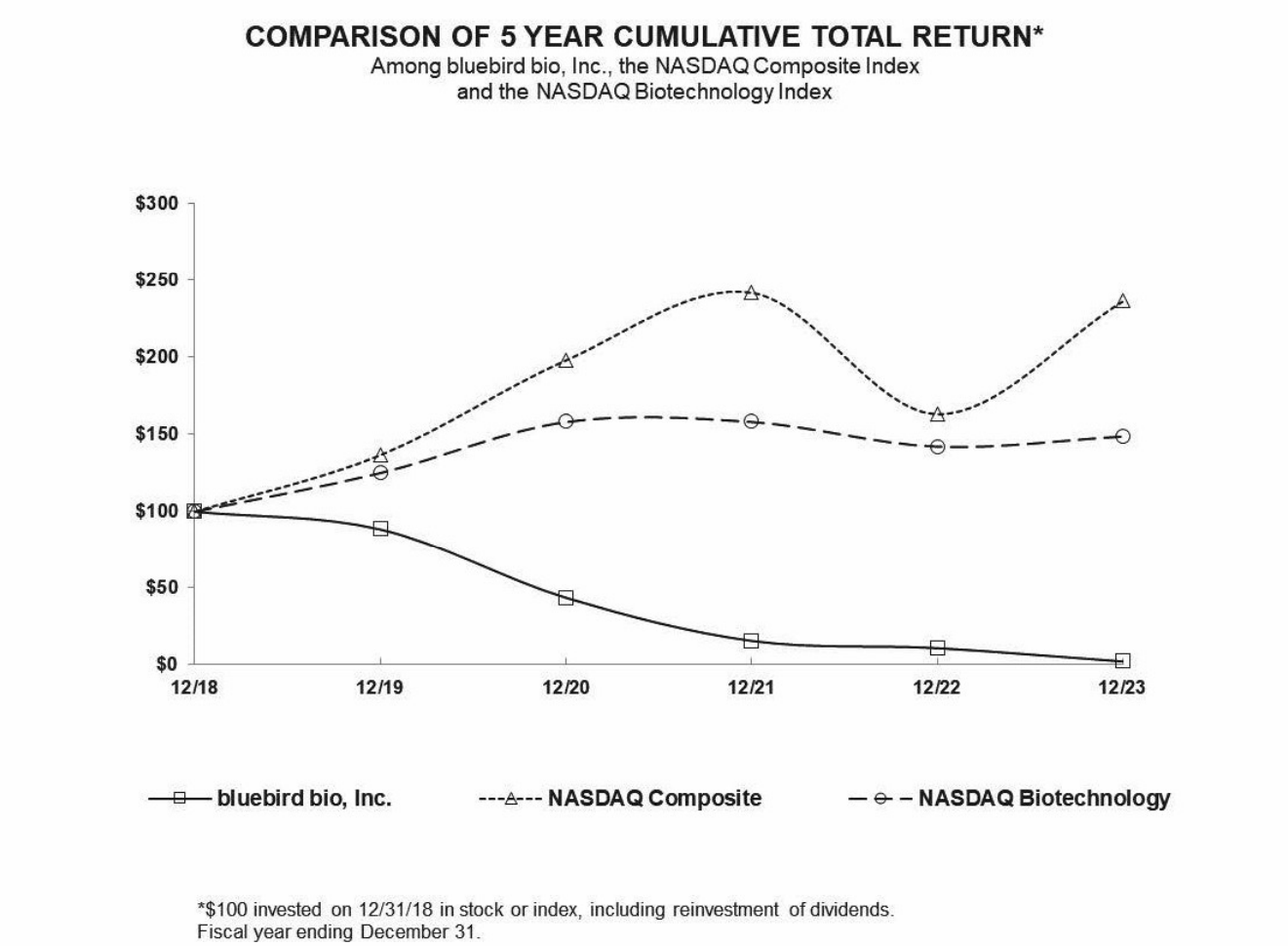
Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities

Our common stock has been traded on the Nasdaq Global Select Market under the symbol "BLUE."

Stock Performance Graph

The graph set forth below compares the cumulative total stockholder return on our common stock between December 31, 2018 and December 31, 2023, with the cumulative total return of (a) the Nasdaq Biotechnology Index and (b) the Nasdaq Composite Index, over the same period. This graph assumes the investment of $100 on December 31, 2018 of our common stock, the Nasdaq Biotechnology Index and the Nasdaq Composite Index and assumes the reinvestment of dividends, if any.

The comparisons shown in the graph below are based upon historical data. We caution that the stock price performance shown in the graph below is not necessarily indicative of, nor is it intended to forecast, the potential future performance of our common stock.



Holders

As of September 11, 2024, there were approximately 8 holders of record of our common stock. The actual number of stockholders is greater than this number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Dividends

We have not paid any cash dividends on our common stock since inception and do not anticipate paying cash dividends in the foreseeable future.

Securities Authorized for Issuance Under Equity Compensation Plans

Information about our equity compensation plans is provided in Item 12 of Part III of this Annual Report on Form 10-K.

Item 6. Reserved

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations

The following is a discussion of the results of operations for our fiscal years ended December 31, 2023 and 2022, and changes in financial condition during those years. The Consolidated Financial Statements for the year ended December 31, 2022, have been restated to correct prior period misstatements. The discussion and tables included below have been corrected to reflect the restatements. For more information see Note 2 – Restatement of Previously Issued Financial Statements in the Consolidated Financial Statements of this Annual Report on Form 10-K. The following information should be read in conjunction with the consolidated financial statements and related notes thereto included in this Annual Report on Form 10-K.

In addition to historical information, this report contains forward-looking statements that involve risks and uncertainties which may cause our actual results to differ materially from plans and results discussed in forward-looking statements. We encourage you to review the risks and uncertainties discussed in the sections entitled Item 1A. "Risk Factors" and "Forward-Looking Statements" included at the beginning of this Annual Report on Form 10-K. The risks and uncertainties can cause actual results to differ significantly from those forecast in forward-looking statements or implied in historical results and trends.

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 April 2022, we initiated a comprehensive restructuring plan intended to reduce operating expenses. As part of the restructuring, we reduced our workforce by approximately 30% in the second and third quarters of 2022. See Note 19, Reduction in Workforce, to our consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K for more information on this restructuring plan.

As of December 31, 2023, we had cash and cash equivalents of approximately $221.8 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 $211.9 million for the year ended December 31, 2023 and our accumulated deficit was $4.3 billion as of December 31, 2023. 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. 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 $221.8 million as of December 31, 2023. 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 December 31, 2023 was $52.8 million. The restricted cash was unavailable for use as of December 31, 2023, and we believe at least $43.6 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 for the year ended December 31, 2023. Our revenues were derived from product revenues associated with the sale of ZYNTEGLO in Germany for the year ended December 31, 2022.

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 and ZYNTEGLO in Germany for the years ended December 31, 2023 and 2022, respectively.

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 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 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 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:

	Year ended December 31,	
	2023	2022
	(in thousands)	
		(As Restated)
ZYNTEGLO (beti-cel)	$ 10,767	$ 30,317
LYFGENIA (lovo-cel)	72,404	61,809
SKYSONA (eli-cel)	13,671	22,068
Preclinical programs	1,254	7,278
Total direct research and development expense	98,096	121,472
Employee- and contractor-related expenses	29,413	29,792
Stock-based compensation expense	9,000	19,259
Laboratory and related expenses	3,859	776
License and other collaboration expenses	2,417	—
Facility expenses	24,867	29,140
Total other research and development expenses	69,556	78,967
Total research and development expense	$ 167,652	$ 200,439

Restructuring expenses

Restructuring expenses consist of costs associated with postemployment nonretirement benefits in accordance with ASC 712, *Postemployment Nonretirement Benefits*. Such costs are based on the estimate of fair value in the period the expenses were incurred.

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 second 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.

In the fourth quarter of 2022, we sold our first PRV for $102.0 million. We received the PRV in August 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.

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 judgments and estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our financial statements, which have been prepared in accordance with 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.

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

Revenue recognition

Revenue recognition

Under Topic 606, *Revenue from Contracts with Customers,* 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. We only apply 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, we assess the goods or services promised within each contract and determine 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. We assess if these options provide a material right to the customer and if so, they are considered performance obligations.

We assess whether each promised good or service is distinct for the purpose of identifying 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, we estimate the amount of consideration to which we will be entitled in exchange for transferring the promised goods or services to a customer. We determine the amount of variable consideration by using the expected value method or the most likely amount method. We include 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, we re-evaluate the estimated variable consideration included in the transaction price and any related constraint, and if necessary, adjust our estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis in the period of adjustment.

If an arrangement includes development and regulatory milestone payments, we evaluate whether the milestones are considered probable of being reached and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within our control or the licensee's control, such as regulatory approvals, are generally not considered probable of being achieved until those approvals are received.

We then recognize 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

We recognize product revenue in accordance with ASC 606, *Revenue from Contracts with Customers*. In 2023, product revenue represents sales of ZYNTEGLO and SKYSONA in the United States. We recognize revenue from product sales at the point in time that we satisfy our performance obligation, which is upon patient infusion. Costs to manufacture and deliver the product are included in cost of product revenue.

Leases

Under ASU 2016-02, *Leases (Topic 842),* ("ASU 2016-02" or "ASC 842"), at the inception of an arrangement, we determine whether the arrangement is or contains a lease based on the unique facts and circumstances present in the arrangement. We lease real estate, principally office and lab space with administrative equipment, and have leases related to our third-party manufacturing and related operations.

We determine if an arrangement is a lease at inception of the contract and we perform the lease classification test as of the lease commencement and subsequent modification date. Right-of-use assets represent our right to use an underlying asset for the lease term and lease liabilities represent our obligation to make lease payments arising from the lease.

Lease liabilities and their corresponding right-of-use assets are initially recorded based on the present value of lease payments over the expected remaining lease term. Certain adjustments to the right-of-use asset may be required for items such as incentives received. Our leases do not provide an implicit interest rate. We use our estimated incremental borrowing rate based on the information available at commencement date in determining the present value of future payments. The incremental borrowing rate reflects the fixed rate at which we could borrow on a collateralized basis the amount of the lease payments in the same currency, for a similar term, in a similar economic environment. To estimate our incremental borrowing rate, a credit rating applicable to us is estimated using a synthetic credit rating analysis since we do not currently have a rating agency-based credit rating.

For real estate leases deemed operating leases, lease amortization expense is primarily presented in selling, general and administrative expenses in operating expense in the income statement based on the nature of the lease. In cases when a real estate lease supports research and development or commercial production, we classify the amortization expense as research and development expense or assess the costs for capitalization to inventory, respectively. For financing leases which are principally related to our manufacturing operations, we present finance right-of-use assets in PP&E and recognize amortization expense associated with those assets on a straight-line basis over the shorter of the life of the leased asset or the related lease term and interest expense as a non-operating expense. When the finance lease right-of-use asset is used for research and development, we classify the amortization expense related to the finance lease right-of-use asset as research and development pursuant to the guidance in ASC 730, Research and Development Costs.

We have elected not to recognize leases with an original term of one year or less on the balance sheet. We typically only include an initial lease term in our assessment of a lease arrangement. Options to renew a lease are not included in our assessment unless there is reasonable certainty that we will renew.

Assumptions that we made at the commencement date are re-evaluated upon occurrence of certain events, including a lease modification. A lease modification results in a separate contract when the modification grants the lessee an additional right-of-use not included in the original lease and when lease payments increase commensurate with the standalone price for the additional right-of-use. When a lease modification results in a separate contract, it is accounted for in the same manner as a new lease. When a lease modification does not result in a separate contract, we reassess the classification of the existing lease at the effective date of the modification and remeasure the lease liability and adjust the carrying amount of the right-of-use asset by the amount of the remeasurement of the lease liability, including an update of the incremental borrowing rate used to measure the lease liability. Lease modifications associated with embedded leases in contract manufacturing arrangements may arise in greater frequency based on the complexity of gene therapy manufacturing processes and the related services provided by contract manufacturing organizations, which are combined with the lease components. We apply our judgment in determining whether the ongoing delivery of services to us by contract manufacturing organizations represent a change in the scope or consideration of the arrangement, which are accounted for as lease modifications, or are variable lease payments recognized as incurred.

In accordance with ASC 842, components of a lease should be split into three categories: lease components, non-lease components, and non-components.

Entities may elect not to separate lease and non-lease components. Rather, entities would account for each lease component and related non-lease component together as a single lease component. We have elected to account for lease and non-lease components together as a single lease component for both our real-estate leases as well as our embedded drug product and drug substance contract manufacturing leases. Accordingly, we allocate all of the contract consideration to the lease component only.

ASC 842 allows for the use of judgment in determining whether the assumed lease term is for a major part of the remaining economic life of the underlying asset and whether the present value of lease payments represents substantially all of the fair

value of the underlying asset. We apply the guidance referenced in ASC 842-10-55-2 to assist in determining leases classification across our portfolio of leases.

Accrued research and development expenses

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

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

Other examples of estimated accrued research and development expenses include fees paid to:

- investigative sites in connection with clinical studies;
- vendors in connection with preclinical development activities; and
- vendors related to the development, manufacturing, and distribution of clinical trial materials, where such fees are variable in nature and are not included in the measurement of embedded lase liabilities to CMOs.

Stock-based compensation

We issue stock-based awards to employees and non-employees, generally in the form of stock options and restricted stock units. We account for our stock-based awards in accordance with FASB ASC Topic 718, *Compensation—Stock Compensation* ("ASC 718"). ASC 718 requires all stock-based payments to employees, including grants of employee stock options and modifications to existing stock options, to be recognized in the consolidated statements of operations and comprehensive loss based on their fair values. Prior to the adoption of Accounting Standards Update ("ASU") No. 2018-07, *Compensation - Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting* ("ASU 2018-07"), the measurement date for non-employee awards was generally the date the services are completed, resulting in financial reporting period adjustments to stock-based compensation during the vesting terms for changes in the fair value of the awards. After the adoption of ASU 2018-07, the measurement date for non-employee awards is the date of grant without changes in the fair value of the award. Stock-based compensation costs for non-employees are recognized as expense over the vesting period on a straight-line basis.

Our stock-based awards are subject to either service or performance-based vesting conditions. Compensation expense related to awards to employees, non-employees, and directors, with service-based vesting conditions is recognized on a straight-line basis based on the grant date fair value over the associated service period of the award, which is generally the vesting term. Compensation expense related to awards to employees and non-employees with performance-based vesting conditions is recognized based on the grant date fair value over the requisite service period using the accelerated attribution method to the extent achievement of the performance condition is probable. We estimate the probability that certain performance criteria will be met and do not recognize compensation expense until it is probable that the performance-based vesting condition will be achieved.

We estimate the fair value of our stock-based awards to employees, non-employees, and directors, using the Black-Scholes option pricing model, which requires the input of subjective assumptions, including (i) the expected volatility of our stock, (ii) the expected term of the award, (iii) the risk-free interest rate, and (iv) expected dividends. Effective January 1, 2020, we eliminated the use of a representative peer group and use only our own historical volatility data in our estimate of expected volatility given that there is now a sufficient amount of historical information regarding the volatility of our own stock price. We estimate the expected life of our employee stock options using the "simplified" method, whereby, the expected life equals the average of the vesting term and the original contractual term of the option, unless there are more appropriate indicators of

the expected life when measuring the fair value of a modified award. The risk-free interest rates for periods within the expected life of the option were based on the U.S. Treasury yield curve in effect during the period the options were granted.

Recent accounting pronouncements

See Note 3, *Summary of significant accounting policies and basis of presentation,* in the notes to consolidated financial statements for a description of recent accounting pronouncements applicable to our business.

Results of Operations

Comparison of the years ended December 31, 2023 and 2022:

	Year ended December 31,		
	2023	2022 (As Restated)	Change
	(in thousands)		
Revenue:			
Product revenue, net	$ 29,065	$ 2,739	$ 26,326
Other revenue	432	858	(426)
Total revenues	29,497	3,597	25,900
Cost of product revenue	33,527	10,077	23,450
Gross margin	(4,030)	(6,480)	2,450
Operating expenses:			
Selling, general and administrative	165,510	140,326	25,184
Research and development	167,652	200,439	(32,787)
Restructuring expense	—	4,940	(4,940)
Total operating expenses	333,162	345,705	(12,543)
Gain from sale of priority review voucher, net	92,930	102,000	(9,070)
Loss from operations	(244,262)	(250,185)	5,923
Interest income	9,869	1,032	8,837
Interest expense	(16,353)	(6,322)	(10,031)
Other income, net	38,707	25,250	13,457
Loss before income taxes	(212,039)	(230,225)	18,186
Income tax benefit (expense)	126	(117)	243
Net loss	$ (211,913)	$ (230,342)	$ 18,429

Revenue. Total revenue was $29.5 million for the year ended December 31, 2023, compared to $3.6 million for the year ended December 31, 2022 and is comprised of product revenue from sales of ZYNTEGLO and SKYSONA in the United States in 2023 and ZYNTEGLO in Germany in 2022, respectively.

Selling, general and administrative expenses. Selling, general and administrative expenses were $165.5 million for the year ended December 31, 2023, compared to $140.3 million for the year ended December 31, 2022. The increase of $25.2 million was primarily due to the following:

- $14.6 million of increased information technology and facility-related costs;
- $8.8 million of increased commercial costs driven by marketing activities for ZYNTEGLO and SKYSONA in the United States and the performance of commercial readiness activities in the United States for LYFGENIA, which received FDA approval in December 2023;
- $5.5 million of increased professional fees driven by increased legal, audit, and public relations costs; and
- $0.8 million of increased net employee compensation, benefit, and other headcount-related expenses, driven by increased headcount in selling, general and administrative costs in 2023 offset by a decrease in stock-based compensation expense due to an overall decrease in the value of awards.

These increased costs were partially offset by $5.3 million of decreased costs attributable to consulting fees.

Research and development expenses. Research and development expenses were $167.7 million for the year ended December 31, 2023, compared to $200.4 million for the year ended December 31, 2022. The decrease of $32.8 million was primarily attributable to the following:

- $25.2 million of decreased net employee compensation, benefit, and other headcount related expenses, including a decrease of $10.3 million in stock-based compensation expense, driven by related expenses being included in inventory and cost of product revenue for our commercial products;
- $7.3 million of decreased manufacturing costs primarily driven by material production being included in inventory and cost of product revenue for our commercial products;
- $7.0 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;
- $3.7 million of decreased consulting fees; and
- $2.7 million of decreased non-clinical costs.

These decreased costs were partially offset by the following:

- $11.2 million of increased clinical trial costs primarily driven by clinical trials for LYFGENIA; and
- $1.4 million of increased laboratory costs.

Cost of product revenue. Cost of product revenue was $33.5 million for the year ended December 31, 2023, compared to $10.1 million for the year ended December 31, 2022. The increase is attributable to increased product sales during 2023.

Restructuring expenses. There were no restructuring expenses recorded for the year ended December 31, 2023, compared to $4.9 million for the year ended December 31, 2022. The restructuring expenses are primarily related to the costs associated with the reduction in workforce in 2022.

Gain from sale of priority review voucher, net. The decrease in gain from sale of priority review voucher, net was related to higher net proceeds received for the sale of the priority review voucher in the fourth quarter of 2022 compared to the sale of the second priority review voucher in the first quarter of 2023.

Interest income. The increase in interest income was primarily related to higher interest income earned on investments.

Interest expense. The increase in interest expense was primarily related to increases in the finance lease liability from our embedded leases related to certain CMOs and a CTO.

Other income, net. The increase in other income, net was primarily related to increased sublease rental income in 2023.

Liquidity and Capital Resources

As of December 31, 2023, we had cash and cash equivalents of approximately $221.8 million. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation. As of December 31, 2023, 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 December 31, 2023, we had an accumulated deficit of $4.3 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.

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 $52.8 million, which was unavailable for use as of December 31, 2023, and we believe at least $43.6 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 June 2022, we entered into an Equity Distribution Agreement with Goldman Sachs & Co. LLC ("Goldman") to sell shares of our common stock up to $75.0 million, from time to time, through an “at the market” equity offering program under which Goldman will act as manager. As of December 31, 2022, we sold 10.7 million shares of common stock at-the-market under the Equity Distribution Agreement, resulting in gross proceeds to us of approximately $56.2 million ($54.2 million net of offering costs). This agreement was terminated in August 2023 and there was no activity in 2023.
- In December 2022, we sold a PRV for aggregate net proceeds of $102.0 million.
- In January 2023, we sold our second PRV for aggregate net proceeds of $92.9 million.
- In January 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 December 31, 2023, 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:

	Year ended December 31,	
	2023	2022
	(in thousands)	
		(As Restated)
Net cash used in operating activities	$ (235,046)	$ (316,219)
Net cash provided by investing activities	154,950	250,453
Net cash provided by financing activities	196,248	17,519
Increase (decrease) in cash, cash equivalents and restricted cash	$ 116,152	$ (48,247)

Operating Activities. The net cash used in operating activities was $235.0 million for the year ended December 31, 2023 and primarily consisted of a net loss of $211.9 million adjusted for non-cash items including a gain from the sale of priority review voucher of $92.9 million and change in net working capital of $49.7 million, offset by non-cash items including stock-based compensation of $19.4 million, the recognition of a reserve for excess inventories of $15.4 million, depreciation and amortization of $28.5 million, non-cash items related to finance leases of $21.2 million, non-cash items related to operating leases of $29.8 million, and proceeds from factoring arrangement of accounts receivable of $5.0 million.

The net cash used in operating activities was $316.2 million for the year ended December 31, 2022 and primarily consisted of a net loss of $230.3 million adjusted for non-cash items including a gain from the sale of priority review voucher of $102.0 million, gain on foreign currency exchange rates of $1.8 million primarily related to our embedded leases and change in net working capital of $75.0 million, offset by non-cash items including stock-based compensation of $35.1 million, the recognition of a reserve for excess inventories of $7.5 million, depreciation and amortization of $8.3 million, non-cash items related to finance leases of $11.2 million, non-cash items related to operating leases of $23.6 million, unrealized loss on equity securities of $3.1 million, and other non-cash items of $4.1 million.

Investing Activities. Net cash provided by investing activities for the year ended December 31, 2023 was $155.0 million and was primarily due to proceeds from the maturities of available-for-sale marketable securities of $108.5 million, sale of priority review voucher of $92.9 million, and proceeds from sales of marketable securities of $5.9 million, offset by the purchase of $43.3 million of marketable securities, the purchase of $4.2 million of property, plant and equipment, and the capitalization of FDA approval milestones related to LYFGENIA and SKYSONA of $4.9 million.

Net cash provided by investing activities for the year ended December 31, 2022 was $250.5 million and was primarily due to proceeds from the maturities of available-for-sale marketable securities of $131.4 million, sale of priority review voucher of $102.0 million, and proceeds from sales of marketable securities of $30.2 million, offset by the purchase of $8.2 million of property, plant and equipment and the capitalization of two FDA approval milestones related to SKYSONA and ZYNTEGLO of $5.0 million.

Financing Activities: Net cash provided by financing activities for the year ended December 31, 2023 was $196.2 million and was primarily due to proceeds from the secondary public offering, net of issuance costs of $248.2 million and proceeds from factoring arrangement of $2.5 million, offset by $54.4 million in principal payments on finance leases.

Net cash provided by financing activities for the year ended December 31, 2022, was $17.5 million and was primarily due to net cash proceeds from our At The Market ("ATM") equity offering program, net of issuance costs of $54.2 million, offset by $36.7 million in principal payments on finance leases.

Contractual Obligations and Commitments

Operating lease commitments

60 Binney Street lease & sublease

In October 2021, we entered into a consent to assignment and amendment to our lease agreement for our 60 Binney Street lease (the "Assignment"). The Assignment transfers our interest in the lease to 2seventy bio and releases us from our obligation to maintain the $13.8 million collateralized letter of credit required under the original 60 Binney Street lease. Although the lease was legally transferred to 2seventy bio, the lessor required that we remain secondarily liable as a guarantor for payment obligations accruing under the 60 Binney Street lease from the date of Assignment until the later of (i) we have completely vacated the premises and (ii) 2seventy bio has reached a market capitalization of $900 million. Upon Separation, the lease assignment was accounted for as the termination of the original lease and we de-recognized the right-of-use asset and lease liability related to the 60 Binney Street lease and recognized the fair value of the guarantee pursuant to ASC 405, Liabilities. The fair value of the guarantee was not material.

Concurrent with the Assignment of the 60 Binney Street lease, we entered into a sublease agreement with 2seventy bio for office, laboratory and storage space located at 60 Binney Street (the "60 Binney Street Sublease") while we construct and outfit our new office and laboratory space. The lease was modified in July 2022 to reflect the decreased use of the office and lab space. Under the terms of the 60 Binney Street Sublease, we leased 72,988 square feet for $1.0 million per month in base rent for the period beginning from the Assignment through March 2022 and 58,004 square feet for $0.8 million per month in base rent for the period from April 2022 through July 2022. Under the terms of the modification, beginning in July 2022, we are required to pay $0.6 million monthly until no later than December 2023. We also pay monthly fees for use of the facilities and support personnel, calculated based on our pro-rata share of operating costs during the term of the 60 Binney Street Sublease. We accounted for the 60 Binney Street Sublease as a new lease under ASC 842, and in November 2021, we recognized a right-of-use asset and lease liability related to the 60 Binney Street Sublease. We terminated the 60 Binney Street Sublease in August 2023 and derecognized the right-of-use asset and lease liability.

50 Binney Street sublease & sub-sublease

In April 2019, we entered into a sublease agreement for office space located at 50 Binney Street in Cambridge, Massachusetts (the "50 Binney Street Sublease") to supplement our then-current corporate headquarters located at 60 Binney Street in Cambridge, Massachusetts. Under the terms of the 50 Binney Street Sublease, we lease 267,278 square feet of office space for $99.95 per square foot, or $26.7 million per year in base rent subject to certain operating expenses, taxes and annual rent increases of approximately 3%. The lease commenced in April 2022. Upon signing the 50 Binney Street Sublease, we executed a $40.1 million cash-collateralized letter of credit, which may be reduced in the future subject to the terms of the 50 Binney Street Sublease and certain reduction requirements specified therein. The $40.1 million of cash collateralizing the letter of credit is classified as restricted cash and other non-current assets on our consolidated balance sheets. In connection with the execution of the 50 Binney Street Sublease, we also entered into a purchase agreement for furniture and equipment (the "Furniture Purchase Agreement") located on the premises upon lease commencement. Upon execution of the Furniture Purchase Agreement, we made an upfront payment of $7.5 million. We made another $7.3 million payment under the Furniture Purchase Agreement upon lease commencement.

In December 2021, we entered into a sub-sublease agreement (the "Sub-Sublease") with Meta Platforms. Inc. ("Meta"). Under the terms of the Sub-Sublease, we are subleasing the entirety of the 50 Binney Street premises which we have rights to under the 50 Binney Street Sublease. We are sub-subleasing the premises for $29.4 million in the first year (inclusive of parking costs) with 3% annual increases in each subsequent year. Meta received access to 50 Binney Street at the lease commencement date, which is the same point that we received access under the 50 Binney Street Sublease. We remain liable under the 50 Binney Street Sublease, including for the maintenance of the $40.1 million collateralized letter of credit. The Company recognizes monthly sublease income of $2.6 million in other income, net for the sub-subleased space. In connection with the execution of the sub-sublease, we sold the furniture in the premises to Meta for $1 and wrote down the carrying value of the furniture to its recoverable amount under the sub-sublease.

Assembly Row lease

In November 2021, we entered into a lease agreement with Assembly Row 5B, LLC ("Landlord") for office space located at 455 Grand Union Boulevard in Somerville, Massachusetts to serve as our future corporate headquarters. Under the terms of the arrangement, we lease approximately 61,180 square feet starting at an annual rate of $45 per square foot, subject to annual increases of 2.5%, plus operating expenses and taxes. In addition, we are eligible to receive a tenant work allowance of $160 per rentable square foot of the premises. The lease commenced on March 1, 2022, the date on which the Landlord tendered possession of the premises to us with any tenant work required to be performed by the Landlord substantially completed. Since the lease commencement date, we have recognized rent expense of $0.3 million monthly.

Hood Park lease

In October 2022, we entered into a sublease with Finch Therapeutics, Inc. ("Finch") for office and laboratory space at Finch's corporate headquarters located at 100 Hood Park Drive, Charlestown, Massachusetts. Under the terms of the arrangement, we lease 42,261 square feet for $55 per square foot, subject to annual increases of 3.0%, plus operating expenses and taxes. This sublease commenced December 15, 2022, the date on which the landlord tendered us possession of the premises and is expected to terminate on December 14, 2025. Since the lease commencement date, we have recognized substantially all of the monthly $0.2 million costs as rent expense.

Finance lease commitments – Embedded leases

Drug Product Manufacturing

In June 2016, we entered into a manufacturing agreement for the future commercial production of our ZYNTEGLO and SKYSONA drug products with a CMO. Under this 12-year agreement, the CMO will complete the design, construction, validation and process validation of the leased suites prior to anticipated commercial launch of the product candidates. From 2016 through March 2018, while the suites were under construction, we paid a total of $12.0 million in contractual milestone payments. Construction was completed in March 2018, and beginning in April 2018 we paid $5.1 million per year in fixed suite reservation fees and certain fixed labor, raw materials, testing and shipping costs for manufacturing services. We may terminate this agreement at any time upon payment of a one-time termination fee and up to 24 months of slot fees and 12 months of labor fees. We concluded that this agreement contains an embedded lease as the suites are designated for our exclusive use during the agreement's term, that we were not the deemed owner during construction, and the lease was not a capital lease under ASC 840-10, Leases – Overall. As a result, we initially accounted for the agreement as an operating lease under ASC 840 and recognized straight-line rent expense over the non-cancellable term of the embedded lease. As part of our adoption of ASC 842,

effective January 1, 2019, we carried forward the existing lease classification under ASC 840. The lease was modified and reclassified as a finance lease as of March 2019. In September 2023, we amended our agreement with the CMO to change the fee structure in the arrangement from a fixed suite reservation fee to a fixed slot manufacturing fee. In addition, separate from the suite and existing equipment, we also have a forward starting embedded equipment lease that has not yet commenced. As the CMO is required to provide operational equipment throughout the contract term, the forward starting lease was established to account for the equipment that the CMO is obligated to lease to us in the future once the current equipment reaches its useful life and the lease expires. This forward starting lease is expected to commence in 2025 with an initial lease term of three years and has fixed commitments of approximately $54.5 million. We have prepaid approximately $3.7 million, which is accounted for as prepaid asset on the Company's balance sheet that would adjust the right-of-use asset when the forward starting embedded equipment lease commences in 2025.

In November 2016, we entered into an agreement for clinical and commercial production of our ZYNTEGLO, SKYSONA and LYFGENIA drug products with a CMO at an existing facility. We concluded this agreement contains an embedded operating lease as the clean rooms are designated for our exclusive use during the agreement's term. The term of the agreement is five years with subsequent three-year renewals at the mutual option of each party. We recognized a right-of-use asset and lease liability and were recognizing rent expense on a straight-line basis throughout the estimated remaining term of the embedded lease upon effective date of ASC 842. In January 2019, we amended this agreement along with the execution of new work orders and additional contracts for the delivery of non-lease services from the CMO, resulting in a lease modification under ASC 842. Under the terms of the amended agreement, we are required to pay annual maintenance and production fees of up to €16.5 million, depending on production needs, and may terminate this agreement with twelve months' notice and a one-time termination fee. The amendment also provides for an option to reserve an additional clean room for a one-time option fee and annual maintenance fees. In connection with the lease modification, in January 2019, we reconsidered the lease classification and accounted for this embedded lease as a finance lease under ASC 842. In September 2021, we reassessed the term of this lease due to the planned orderly wind down of operations in Europe. As a result, we reduced the right-of-use asset and related lease liability to reflect the shortened expected term of the agreement. In November 2021, we exercised our right to terminate the lease agreement, and such termination became effective in November 2022. Under the amended agreement, we were obligated to pay a one-time termination fee of €1.0 million upon termination, which was paid in December 2021, and we paid for services rendered through the date of termination based on work completed and expenses incurred prior to the date of termination.

During July 2020, we amended an existing arrangement to reserve additional manufacturing capacity with an existing CMO to manufacture LYFGENIA. We concluded that this amendment contains an embedded lease as a controlled environment room at the facility is designated for our exclusive use during the term of the agreement, with the option to sublease the space if we provide notice that we will not utilize it for a specified duration of time. Under the amended agreement, we are required to pay up to $5.4 million per year in maintenance fees in addition to the cost of any services provided and may terminate this agreement with eighteen months' notice. The term of the agreement is five years, with the option to extend. When the additional manufacturing capacity became available that served as lease commencement in March 2021, we classified the embedded lease as a finance lease.

In February 2021, we amended another agreement to reserve additional manufacturing capacity. We concluded that this amended agreement contains an embedded lease as a controlled environment room at the facility is designated for our exclusive use during the agreement's term. Under the amended agreement, we must pay $4.2 million per year in maintenance fees and per-batch fees for the cost of any services provided. Either party may terminate this agreement with eighteen months' notice at any time or with eight months' notice after defined milestones in the agreement have been met. The term of the agreement is five years, with the option to extend. The lease commenced in November 2021 when the suites became available, and upon commencement we classified it as finance lease. The lease has been subsequently modified based on changes in the delivery of non-lease component services, which have been combined with lease components under our accounting policy.

In August 2022, we amended another agreement to reserve additional controlled environmental rooms. We concluded this amended agreement contains an embedded finance lease as the controlled environment room at the facility is designated for our exclusive use during the agreement's term. Under this amended agreement, an existing deposit of $10.8 million was applied towards the monthly lease payments through September 2023. The term of the agreement was 14 months, with an option to extend. We exercised the extension option to extend through December 31, 2023.

Drug Substance Manufacturing

In November 2017, we entered a commercial manufacturing services agreement with a CMO to establish commercial production of our suspension vector. We concluded this agreement contained an embedded finance lease when production commenced in the new facility in 2019 as we have dedicated suite space and reserved production capacity at a rate that allows

us to take more than substantially all the capacity of certain manufacturing space and equipment within the facility. Under the agreement, we are required to pay capacity reservation fees, minimum purchase commitment fees, and milestone fees for achievement of certain activities. In addition, we prepaid approximately €13.5 million between 2018 and 2019 towards certain milestone payments and in exchange for production credits. As of December 31, 2021, those credits have been fully used. We modified the lease to extend its term in October 2022, April 2023, and finally in October 2023, which extends the lease term to end in March 2024 in addition to changes in the delivery of non-lease component services, which have been combined with lease components under our accounting policy.

In January 2018, we entered a clinical and commercial supply agreement with a CMO to manufacture ZYNTEGLO and SKYSONA vector products. We concluded this agreement contained an embedded finance lease. We agreed to wind down manufacturing activities with the CMO originally in December 2021. In December 2022, we reestablished this manufacturing agreement and concluded the revised arrangement contains an embedded finance lease as we are using the entire capacity of a manufacturing suite at the facility. The term of the agreement is three years and requires us to pay suite reservation fees of $13.5 million in 2023 and $18.0 million per year in 2024 and 2025, in addition to the cost of any services provided. The lease has been subsequently modified based on changes in the delivery of non-lease component services, which have been combined with lease components under our accounting policy.

Quality Testing

In 2018, we entered a 2-year master contract services agreement (MCSA) with a CTO to provide clinical development services (other than manufacturing services). The original MCSA expired in June 2020 but was reinstated through December 2024 under the amendment of the MCSA effective in April 2021. We concluded this agreement contained an embedded finance lease as we had certain lab suites implicitly dedicated to us for various clinical testing procedures. We are required to pay the fixed price outlined in each of the various work orders covering quality, stability and other services. The lease has been subsequently modified based on changes in the delivery of non-lease component services, which have been combined with lease components under our accounting policy.

Contingent Milestone and Royalty Payments

We also have obligations to make future payments to third parties that become due and payable on the achievement of certain development, regulatory and commercial milestones (such as the start of a clinical trial, filing of a BLA, approval by the FDA or product launch). We do not recognize these commitments in our financial statements until they become payable or have been paid.

Research and development costs are expensed as incurred. These expenses include the costs of our proprietary research and development efforts, as well as costs incurred in connection with certain licensing arrangements. Before a product candidate receives regulatory approval, we record upfront and milestone payments we make to third parties under licensing arrangements as expense. Upfront payments are recorded when incurred, and milestone payments are recorded when the specific milestone has been achieved. Once a product receives regulatory approval, we record any milestone payments in identifiable intangible assets, less accumulated amortization and, unless the asset is determined to have an indefinite life, we typically amortize the payments on a straight-line basis over the remaining agreement term or the expected product life cycle, whichever is shorter.

Based on our development plans as of December 31, 2023, we may be obligated to make future development, regulatory and commercial milestone payments and royalty payments on future sales of specified products associated with our collaboration and license agreements. Payments under these agreements generally become due and payable upon the achievement of such milestones or sales. Because the achievement of these milestones or sales had not occurred as of December 31, 2023, such contingencies have not been recorded in our financial statements. Amounts related to contingent milestone payments and sales-based royalties are not yet considered contractual obligations as they are contingent upon success.

- Under a license agreement with Inserm-Transfert pursuant to which we license certain patents and know-how for use in adrenoleukodystrophy therapy, we will be required to make payments based upon development, regulatory and commercial milestones for any products covered by the in-licensed intellectual property. To date, we have paid €1.3 million pursuant to the terms of this license agreement, and we may be obligated to pay up to €0.8 million for future milestones. We will also be required to pay a royalty on net sales of products covered by the in-licensed intellectual property in the low single digits.

- Under a license agreement with Institut Pasteur pursuant to which we license certain patents for use in *ex vivo* gene therapy, we are required to make payments per product covered by the in-licensed intellectual property upon the achievement of development and regulatory milestones, depending on the indication and the method of treatment. The

maximum aggregate payments we may be obligated to pay for each of these milestone categories per product is €0.7 and €3.5 million, respectively. To date, we have paid €0.7 million pursuant to the terms of such license arrangement, and we may be obligated to pay up to €0.5 million for future milestones. We are also required to pay a royalty on net sales of products covered by the in-licensed intellectual property in the low single digits, which varies slightly depending on the indication of the product. We have the right to sublicense our rights under this agreement, and we will be required to pay a percentage of such license income varying from the low single digits to mid-range double digits depending on the nature of the sublicense and stage of development. We are required to make an annual maintenance payment, which is offset by royalty payments on a year-by-year basis.

- Under a license agreement with the Board of Trustees of the Leland Stanford Junior University ("Stanford") pursuant to which we license the HEK293T cell line for use in gene therapy products, we are required to pay a royalty on net sales of products covered by the in-licensed intellectual property in the low single digits that varies with net sales. The royalty is reduced for each third-party license that requires payments by us with respect to a licensed product, provided that the royalty to Stanford is not less than a specified percentage that is less than one percent. We have been paying Stanford an annual maintenance fee, which will be creditable against our royalty payments.

- Under a license agreement with Research Development Foundation pursuant to which we license patents that involve LVV, we will be required to make payments of $1.0 million based upon a regulatory milestone for each product covered by the in-licensed intellectual property. To date, we have paid $2.0 million pursuant to the terms of this license agreement. We will also be required to pay a royalty on net sales of products covered by the in-licensed intellectual property in the low single digits, which is reduced by half if during the ten years following first marketing approval the last valid claim within the licensed patent that covers the licensed product expires or ends.

- Under a license agreement with SIRION Biotech GmbH ("Sirion") pursuant to which we license certain patents directed to manufacturing of gene therapy products, we are required to make certain payments related to certain development milestone obligations. We may be obligated to pay up to $13.4 million in the aggregate for each product covered by the in-licensed intellectual property. To date, we have paid $16.0 million pursuant to the terms of this license agreement. Should we seek regulatory approval outside the U.S., we may be obligated to pay up to $8.0 million for future milestones. Upon commercialization of our products covered by the in-licensed intellectual property, we became obligated to pay Sirion a percentage of net sales as a royalty in the low single digits. The royalties payable under this license agreement are subject to reduction for any third-party payments required to be made, with a minimum floor in the low single digits.

Item 7A. Quantitative and Qualitative Disclosures About Market Risks

We are exposed to market risk related to changes in interest rates. As of December 31, 2023 and 2022, we had cash, cash equivalents and marketable securities of $221.8 million and $181.7 million, respectively, primarily invested in U.S. government agency securities and treasuries, corporate bonds, commercial paper 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 8. Financial Statements and Supplementary Data

The financial statements required to be filed pursuant to this Item 8 are appended to this report. An index of those financial statements is found in Item 15.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

None.

Item 9A. 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 the end of the period covered by this Annual Report on Form 10-K. 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.

Management's Annual Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Rules 13(a)-15(f) and 15(d)-15(f) under the Exchange Act. Our internal control over financial reporting is a process designed 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. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Management conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2023 based on the framework in *Internal Control-Integrated Framework* (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). Based on that evaluation, management has concluded that the Company's internal control over financial reporting was not effective as of December 31, 2023 due to the material weakness 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 elsewhere in this Annual Report on Form 10-K), 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.

As a "non-accelerated filer", we are not required to provide an attestation report of our registered public accounting firm on the effectiveness of our internal control over financial reporting.

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 fourth quarter of 2023 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information

(a) Disclosure in lieu of reporting on a Current Report on Form 8-K

None.

(b) Insider Trading Arrangement and Policies.

During the three months ended December 31, 2023, no director or "officer" (as defined in Rule 16a-1(f) under the Exchange Act) of the Company adopted 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 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections

Not applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance

The following table sets forth the name, age and position of each of our executive officers and directors.

Name	Age	Position(s)
Executive Officers:		
Andrew Obenshain	50	President, Chief Executive Officer and Director (Principal Executive Officer)
O. James Sterling	54	Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)
Richard A. Colvin, M.D., Ph.D.	58	Chief Medical Officer
Thomas J. Klima	52	Chief Commercial and Operating Officer
Joseph Vittiglio	53	Chief Legal and Business Officer
Non-Employee Directors:		
Mark Vachon (1) (2)	65	Chair of the Board of Directors
John O. Agwunobi, M.D. (1) (3)	59	Director
Michael Cloonan	53	Director
Charlotte Jones-Burton, M.D., M.S. (3)	51	Director
Elisabeth Leiderman, M.D. (1) (2)	47	Director
Nick Leschly	52	Director
Richard Paulson	57	Director
Najoh Tita-Reid (2) (3)	51	Director

(1) Member of the Audit Committee
(2) Member of the Compensation Committee
(3) Member of the Nominating & Corporate Governance Committee

Executive Officers

Andrew Obenshain – Mr. Obenshain has served as our President, Chief Executive Officer and member of our Board of Directors (the "Board") since November 2021. Mr. Obenshain previously served as bluebird's President, Severe Genetic Diseases from August 2020 to November 2021, and as bluebird's Senior Vice President, Head of Europe from 2016 to August 2020. Prior to that, from September 2015 to September 2016, Mr. Obenshain was the general manager of France and Benelux at Shire Pharmaceuticals, Inc. and from 2007 to 2015, he held roles of increasing responsibility at Genzyme/Sanofi. Mr. Obenshain received his M.B.A. from Northwestern University's Kellogg School of Management, and his B.A. in genetics, cell and developmental biology from Dartmouth College. Mr. Obenshain has deep operating and historical experience with our Company gained from serving as our President, Chief Executive Officer and in other roles. Mr. Obenshain also has significant management experience in the biotechnology and pharmaceutical fields.

O. James Sterling – Mr. Sterling has served as our Chief Financial Officer since June 2024. Previously, Mr. Sterling served as Chief Financial Officer of Renalytix plc from November 2018 to June 2024, where he led the finance department. From 2015 to 2018, Mr. Sterling served as managing partner of Renwick Capital LLC. Prior to that, he served as a managing director at investment banks Brock Capital Group LLC and Aleutian Capital Group. Mr. Sterling is currently a director of Star Mountain Lower Middle-Market Capital Corp. Mr. Sterling received his B.A. from Boston University and an M.B.A. from Columbia Business School.

Richard A. Colvin, M.D., Ph.D. – Dr. Colvin has served as our Chief Medical Officer since October 2022. Previously, Dr. Colvin was our interim Chief Medical Officer from March 2021 through September 2022 and our Vice President and head of severe genetic diseases clinical research and development from January 2020 to March 2021. Dr. Colvin joined us in 2018 as Vice President, Medical Lead Thalassemia Program, for the successful European submission and approval of the Company's beti-cel gene therapy for the treatment of patients with beta-thalassemia. Dr. Colvin is on faculty at Harvard Medical School and also sees patients as a Clinical Associate in Medicine, Infectious Diseases Clinic, at Massachusetts General Hospital Chelsea Health Care Center. Prior to joining bluebird, Dr. Colvin was an executive director in translational medicine at Novartis, where he led anti-infective drug development programs for the treatment of patients with certain infections from 2014 to 2018. Previously, Dr. Colvin completed his clinical and research fellowships in the Mass General Brigham Infectious

Diseases Program and completed his internship and residency at the Brigham and Women's Hospital. Dr. Colvin received his M.D. and Ph.D. from Duke University School of Medicine and his B.S. in Biology from Cornell University.

Thomas J. Klima – Mr. Klima has served as our Chief Operating Officer since September 2022 and our Chief Commercial Officer since May 2021. Prior to joining bluebird, Mr. Klima served as Chief Commercial Officer at Gamida Cell Ltd. from January 2019 to December 2020, where he led the strategic vision and commercial growth transforming its R&D organization to a commercially ready company. In 2018, Mr. Klima served as senior vice president of global commercial planning and operations at Atara Biotherapeutics. From 2015 to 2017, Mr. Klima served as senior vice president and chief commercial officer at Navidea Biopharmaceuticals Ltd. (acquired by Cardinal Health). Before that, Mr. Klima served as head of sales and commercial operations at Algeta U.S. (acquired by Bayer Healthcare) from 2012 to 2015. Before Algeta, he held various commercial leadership positions at Dendreon from 2009 to 2012. Mr. Klima began his pharmaceutical career at Eli Lilly where he held several positions of increasing responsibility from 2000 to 2009. Mr. Klima received a B.A. in Business Administration and Marketing from Western State College.

Joseph Vittiglio, Esq. – Mr. Vittiglio has served as our Chief Legal and Business Officer since January 2023. Prior to joining bluebird, Mr. Vittiglio served as Chief Business and Legal Officer at Finch Therapeutics from December 2020 to December 2022 where he guided its initial public offering in 2021. Prior to joining Finch, Mr. Vittiglio was the General Counsel and Chief Business Officer for AMAG Pharmaceuticals from August 2015 to November 2020 where he led its legal and business initiatives, including its successful sale to private equity investors and multiple out-licensing and partnership collaborations. Prior to AMAG, Mr. Vittiglio held leadership roles at Flexion Therapeutics, AVEO Pharmaceuticals, and Oscient Pharmaceuticals. Mr. Vittiglio began his career as a corporate and securities attorney at Mintz, Levin, Cohn, Ferris, Glovsky and Popeo, P.C. Mr. Vittiglio received a B.A. in International Relations from Tufts University and a J.D. from Northeastern University School of Law.

Non-Employee Directors

Mark Vachon – Mr. Vachon has served on our Board since 2014. He has served as President and Executive Vice President at Change Healthcare Holdings, Inc. from November 2016 to April 2018. For over 30 years, Mr. Vachon held a variety of leadership positions across the General Electric organization, and was a company officer beginning in 1999 and a member of GE's Corporate Executive Council. Mr. Vachon was President and CEO of GE Healthcare Americas from 2009 to 2010, and prior to that he was President and CEO of Global Diagnostics Imaging, GE Healthcare, between 2006 and 2009. Between 2003 and 2006, Mr. Vachon was Executive Vice President and CFO of GE Healthcare. Mr. Vachon holds a B.S. in Finance from Northeastern University and an M.A. from Boston College. Mr. Vachon's corporate leadership experience and financial expertise make him a valuable contributor to our Board. In addition, Mr. Vachon has extensive experience in executive operating roles and in the healthcare field on a global basis. He is an "audit committee financial expert" with particular experience in matters faced by the audit committee of a life sciences company.

John O. Agwunobi, M.D. – Dr. Agwunobi has served on our Board since 2017. He was Chief Executive Officer and Chairman of the Board of Herbalife Nutrition Inc. from 2020 to 2022. Previously, Dr. Agwunobi served as Co-President of Herbalife from May 2018 to 2020. He also served as Chief Health and Nutrition Officer at Herbalife, responsible for training, education, science strategy and product development from 2016 to 2018. Prior to joining Herbalife, Dr. Agwunobi was an independent consultant, advising a number of privately-held health-related companies, including serving as an advisory board member of Shopko Stores Operating Co., LLC on behalf of the private equity firm Sun Capital Partners. From September 2007 to April 2014, Dr. Agwunobi served as Senior Vice President and President of Health and Wellness for Wal-Mart (NYSE: WMT) in the United States where he grew the business and provided insight and advice on the company's health reform position. From December 2005 to September 2007, he served as the Assistant Secretary of Health for the U.S. Department of Health and Human Services, where he was responsible for disease prevention and health promotion. His responsibilities included the oversight of the Centers for Disease Control, National Institute of Health, the U.S. Food and Drug Administration, the Office of the U.S. Surgeon General, and numerous other public health offices and programs. Dr. Agwunobi also serves on the Board of the Ensign Group, Inc. and the Board of the U.S. African Development Foundation. Dr. Agwunobi has significant experience as a senior executive and board member in the health and wellness field. In addition, he has deep expertise in public health programs and governmental agencies relevant to the healthcare industry, from his prior service and experience with the public sector. The insights he has developed from these roles provides our Board with important perspectives on the issues facing our company.

Michael Cloonan – Mr. Cloonan has served on our Board since 2024. Since May 2021, Mr. Cloonan has served as President and Chief Executive Officer at Sionna Therapeutics, where he leads company strategy and operations. From May 2017 to April 2021, Mr. Cloonan served as Chief Operating Officer at Sage Therapeutics, where he led all business functions (commercial, medical affairs, government affairs, business development, technical operations, strategy and program

management) as well as general and administrative functions. Prior to Sage, Mr. Cloonan served in various business and commercial roles at Biogen for fourteen years, including most recently as Senior Vice President, U.S. Commercial, where he was the general manager of the multi-billion-dollar multiple sclerosis, hemophilia, and spinal muscular atrophy franchises. Prior to Biogen, Mr. Cloonan worked at Bain & Company as a consultant specializing in healthcare. Mr. Cloonan earned his M.B.A. from the Darden Graduate School of Business Administration at the University of Virginia and a B.A. from College of the Holy Cross. Mr. Cloonan has extensive operating experience gained from serving as President and Chief Executive Officer at Sionna and leadership roles at Sage and Biogen.

Charlotte Jones-Burton, M.D., M.S. – Dr. Jones-Burton has served on our Board since 2022. Since May 2024, Dr. Jones-Burton has been Director and Head of Life Science Product Development and Strategy at 2Flo Ventures, an emerging venture capital firm and startup studio focused on the discovery, development, and commercialization of healthcare solutions. From January 2022 to November 2023, Dr. Jones-Burton served as Senior Vice President, Product Development and Strategy at Chinook Therapeutics, a clinical-stage biotechnology company discovering, developing and commercializing precision medicines for rare, severe kidney diseases. Prior to her role at Chinook Therapeutics she was VP, Global Clinical Development Head, Nephrology at Otsuka Pharmaceutical Development & Commercialization, Inc. from September 2019 to December 2021. From October 2016 until September 2019, Dr. Jones-Burton held various positions at Bristol-Myers Squibb Company with increasing responsibilities, most recently as Executive Director, Full Development Team Leader of Cardiovascular, Anti-Thrombosis. Prior to that, Dr. Jones-Burton was with Merck & Co. from 2007 to May 2011. With more than 20 years of experience as a clinical development leader, internal medicine and nephrology physician and academician, Dr. Jones-Burton is dedicated to creating healthier communities through drug development, patient advocacy and people engagement. Dr. Jones-Burton is also active in numerous professional associations and organizations and founded Women of Color in Pharma (WOCIP), a non-profit professional society focused women of color in the pharmaceutical industry. Dr. Jones-Burton earned a medical degree and Master of Science degree in Epidemiology and Preventive Medicine, with a concentration in Clinical Research, from the University of Maryland School of Medicine. Dr. Jones-Burton's postgraduate training included an internal medicine residency and a nephrology fellowship at the University of Maryland Medical Systems. Dr. Jones-Burton has extensive experience as an executive in the pharmaceutical industry in drug development, physician and patient engagement and advocacy. The insights she has developed in her work, together with her involvement in professional service organizations, also provide her with important perspectives on the issues facing our company in the development and potential commercialization of our therapies, as well as matters of diversity, equity and inclusion.

Elisabeth Leiderman, M.D. – Dr. Leiderman has served on our Board since 2021. Since June 2024, Dr. Leiderman has been Chief Financial Officer and Corporate Development Officer at Dewpoint Therapeutics, a biotechnology company applying biomolecular condensates to drug discovery. From November 2022 to November 2023, Dr. Leiderman served as Chief Financial Officer and Chief Business Officer at Atsena Therapeutics, a clinical-stage gene therapy company. Before joining Atsena, from September 2020 to October 2022, Dr. Leiderman was Chief Financial Officer and Head of Corporate Development at Decibel Therapeutics, a clinical stage biotechnology company developing novel gene therapeutics for restoration of hearing loss and balance disorders. From January 2020 to August 2020, Dr. Leiderman served as Chief Business Officer for Complexa, Inc., a clinical stage biopharmaceutical company focused on life-threatening fibrosis and inflammatory diseases. Prior to Complexa, Dr. Leiderman was Senior Vice President, Head of Corporate Development at Fortress Biotech from November 2016 to November 2019. Earlier in her career from 2007 to 2016, Dr. Leiderman developed her transaction and capital markets expertise in the healthcare investment banking groups at Nomura, Credit Suisse, Jefferies and UBS. Dr. Leiderman began her career in medical affairs at AstraZeneca, where she analyzed product and industry trends related to the central nervous system. Since December 2023, Dr. Leiderman has served on the Board of Directors of Autolus Therapeutics and is a member of the Audit Committee. Dr. Leiderman earned an M.D. from the American Medical Program at Tel Aviv University, an M.B.A. from The Wharton School at the University of Pennsylvania and a B.A. from The University of Pennsylvania. Dr. Leiderman has over 20 years of experience in finance, strategy and business development in the life sciences industry. She is an "audit committee financial expert" with particular experience in matters faced by the audit committee of a life sciences company.

Nick Leschly – Mr. Leschly has served on our Board since 2010. He is Chairman of the Board of 2seventy bio, Inc., a cell and gene therapy company, and from November 2021 to March 2024, Mr. Leschly served as its Chief Executive Officer. Mr. Leschly previously served as bluebird's President and Chief Executive Officer from October 2010 to November 2021. Formerly a partner of Third Rock Ventures, L.P. since its founding in 2007 until 2010, Mr. Leschly played an integral role in the overall formation, development and business strategy of several of Third Rock's portfolio companies, including Agios Pharmaceuticals, Inc. and Edimer Pharmaceuticals, Inc. Prior to joining Third Rock, he worked at Millennium Pharmaceuticals, Inc. (now a subsidiary of Takeda), leading several early-stage drug development programs and served as the product and alliance leader for VELCADE. Mr. Leschly also founded and served as Chief Executive Officer of MedXtend Corporation. He received his B.S. in molecular biology from Princeton University and his M.B.A. from The Wharton School of the University of Pennsylvania. Mr. Leschly has deep operating and historical experience with our Company gained from serving as our

President, Chief Executive Officer and member of the Board. In addition, Mr. Leschly also has significant experience in the venture capital industry and drug research and development.

Richard Paulson – Mr. Paulson has served on our Board since 2023. Since 2021, Mr. Paulson has been President, Chief Executive Officer and Director at Karyopharm Therapeutics, a commercial-stage pharmaceutical company. Previously, Mr. Paulson was Executive Vice President of Ipsen Pharmaceuticals and Chief Executive Officer of Ipsen North America from 2018 to 2021 where he focused on innovative therapies and specialty care for oncology, neuroscience and rare diseases. Before joining Ipsen, Mr. Paulson work at Amgen for 10 years holding various leadership positions across Europe and North America. Mr. Paulson received his M.B.A. from the University of Toronto and his B.Com in marketing and finance from the University of Saskatchewan. Mr. Paulson has deep operating experience gained from serving as President, Chief Executive Officer and Director at Karyopharm and leadership roles at Ipsen and Amgen. Mr. Paulson also has significant experience in sales, marketing, and market access in the biotechnology and pharmaceutical fields.

Najoh Tita-Reid – Ms. Tita-Reid has served on our Board since 2021. Since November 2023, Ms. Tita-Reid has served as Chief Brand and Experience Officer at Mars Petcare, to lead brand, experience, digitalization and technology to drive business transformation. From April 2021 to October 2023, Ms. Tita-Reid served as Global Chief Marketing Officer for Logitech, a global manufacturer of computer peripherals, software and services, where she led the global marketing function. Prior to this role, Ms. Tita-Reid served as Global Commercial Marketing Head from June 2020 to March 2021 and on the Global Marketing Reinvention team from February 2020 to May 2020 at Logitech. Previously, Ms. Tita-Reid served as Global Chief Marketing Officer and Executive Board Member for Hero-AG, a family-run healthy food company, from August 2017 to January 2020. In this role, she developed the organization's marketing function and brand strategy and built the first global innovation pipeline while overseeing the R&D, innovation, sustainability and quality functions. Prior to Hero-AG, Ms. Tita-Reid held leadership positions at Bayer PLC, where she served as Vice President-Country Division Head for Consumer Care in the UK and Ireland from 2014 to 2017 and Merck & Co, Inc., where she served in various roles from 2011 to 2014, including General Manager for Western Europe from 2013 to 2014. Earlier in her career, Ms. Tita-Reid spent 19 years at Procter & Gamble, where she managed a number of consumer brands in the baby and feminine care categories, spearheaded multi-cultural marketing strategy across 15 billion-dollar brands, and led the multi-brand business unit for Hispanic and African American consumers. During her tenure, Ms. Tita-Reid trained and developed the P&G marketing function on ethnic marketing, and created breakthrough marketing strategies. Ms. Tita-Reid graduated with a Bachelor of Arts from Spelman College and holds an M.B.A. from Fuqua School of Business at Duke University. Ms. Tita-Reid has extensive experience as a multi-faceted executive with global marketing expertise, she has a record of strategic and operational ingenuity and transformation across complex organizations and a breadth of experience across the US and Europe.

Family Relationships

There are no family relationships among any of our executive officers or directors.

Code of Business Conduct and Ethics

We have adopted a Code of Business Conduct and Ethics for our directors, officers and employees, including our President and Chief Executive Officer, Principal Financial Officer, Principal Accounting Officer, and Controller (or persons performing an equivalent function). We provide mandatory online training for our employees with respect to the Code of Business Conduct and Ethics on an annual basis to ensure understanding and the importance of adhering to such guidelines. A copy of our Code of Business Conduct and Ethics may be accessed free of charge by visiting the Company's website at www.bluebirdbio.com and going to the Investors & Media—Corporate Governance section or by requesting a copy in writing from Joseph Vittiglio, Secretary, at our Somerville, Massachusetts office. We intend to post on our website any amendment to, or waiver under, a provision of the Code of Business Conduct and Ethics that applies to our President and Chief Executive Officer, Principal Financial Officer, Principal Accounting Officer, or Controller (or persons performing an equivalent function) within four business days following the date of such amendment or waiver.

Audit Committee and Audit Committee Financial Expert

We have a separately designated standing audit committee ("Audit Committee"). The members of the Audit Committee are Elisabeth Leiderman, M.D., John O. Agwunobi, M.D. and Mark Vachon. Dr. Leiderman serves as the Chairperson of the Audit Committee. Each member of the Audit Committee meets the independence requirements under applicable Nasdaq and Securities and Exchange Commission rules, including for purposes of serving on an audit committee. The members of our Audit Committee meet the requirements for financial literacy under the applicable Nasdaq rules. In addition, our Board of Directors has determined that Dr. Leiderman and Mr. Vachon each qualify as an "audit committee financial expert," as such term is defined in Item 407(d)(5) of Regulation S-K.

Item 11. Executive Compensation

This section discusses the material components of the executive compensation program for our current and former executive officers who are named in the 2023 Summary Compensation Table below. In 2023, our "named executive officers" and their positions were as follows:

- Andrew Obenshain, President and Chief Executive Officer;
- Thomas J. Klima, Chief Commercial and Operating Officer; and
- Richard A. Colvin, Chief Medical Officer

Summary Compensation Table

The following table sets forth the total compensation awarded to, earned by and paid during the fiscal years ended December 31, 2023 and December 31, 2022 for each of our named executive officers.

Name and Principal Position	Year	Salary ($)	Option Awards ($) (1)	Stock Awards ($) (2)	Non-equity incentive plan compensation ($) (2)	All other compensation ($) (3)	Total ($)
Andrew Obenshain	2023	681,657	1,142,440	863,590	348,024	13,470	3,049,181
Chief Executive Officer	2022	643,390	421,830	351,000	386,280	12,200	1,814,700
Thomas J. Klima	2023	479,615	371,800	281,050	183,600	13,470	1,329,535
Chief Commercial and Operating Officer	2022	435,769	325,232	267,050	207,000	12,200	1,247,251
Richard A. Colvin	2023	489,808	371,800	281,050	187,425	14,170	1,344,253
Chief Medical Officer	2022	467,639	374,297	294,240	216,000	12,600	1,364,776

(1) The amounts reported in the *Option awards* and *Stock awards* columns above represent the aggregate grant date fair value of the stock options and restricted stock units granted to such named executive officers during 2022 and 2023 as computed in accordance with FASB ASC 718, not including any estimates of forfeitures related to service-based vesting conditions. See note 16 of *Notes to Consolidated Financial Statements* elsewhere in this Annual Report for a discussion of assumptions made by the Company in determining the aggregate grant date fair value of our stock option and restricted stock unit awards. Note that the amounts reported in these columns reflect the accounting cost for these stock options and restricted stock units, and do not correspond to the actual economic value that may be received by the named executive officers from the stock options and restricted stock units. We also granted performance-based restricted stock unit awards to Mr. Obenshain in 2022 and 2023. The grant date fair value computed in accordance with FASB ASC 718 of these awards based on probable outcome as of the grant date is equal to their maximum grant date fair values.

(2) Amounts represent cash payment under our annual cash incentive program earned in respect of 2022 and 2023, based on achievement of performance goals. Please see the description of the annual cash incentive program under which bonuses were paid to the named executive officers in the "Annual Incentive Program" section below.

(3) Amounts represent employer matching contributions to the executive's 401(k) plan account and certain other employee fringe benefits.

Narrative Disclosure to Summary Compensation Table

Base Salary

We provide base salaries to our named executive officers to compensate them with a fair and competitive base level of compensation for services rendered during the year. Our Compensation Committee typically determines the base salary for each executive based on the executive's responsibilities, experience and, if applicable, the base salary level of the executive prior to joining bluebird. In addition, our Compensation Committee reviews and considers the level of base salary paid by companies in our peer group for similar positions. Our Compensation Committee's assessment of our named executive officers' base salary takes into account our compensation objectives and philosophy to retain highly qualified executives, to motivate them to achieve our business goals, and to reward them for superior short- and long-term performance.

At the beginning of 2023, our Compensation Committee reviewed the compensation for our Chief Executive Officer and each of our other named executive officers. With respect to Mr. Obenshain, our Compensation Committee reviewed his overall compensation and determined to increase his annual base salary from $643,750 to $682,400. This determination was based on his critical role at the Company, market conditions, company performance throughout 2022 in meeting key milestones and objectives, as well as consideration of the critical upcoming execution and risk inflection points throughout 2023. The Compensation Committee also approved merit increases in base salary for each of our other named executive officers serving at that time based on several factors including the Company's performance against the 2022 company goals, and each named executive officer's achievement of individual goals in 2022, as well as a consideration of the market conditions and a comparison of the executives' base salaries to similarly situated executives in our peer group. The table below sets forth the adjustments to the base salary, in dollars and as a percentage, for each of our named executive officers:

Name	2022 Base Salary ($)	2023 Base Salary ($)	Aggregate Increase (%)
Andrew Obenshain	643,750	682,400	6.0
Thomas J. Klima	460,000	480,000	4.4
Richard A. Colvin	480,000	490,000	2.1

Annual Incentive Program

At the end of 2022, our Board approved the Company's corporate goals for 2023. Consistent with past practice, the Company's annual incentive program for 2023 was structured based on Company-wide achievement of the Company's corporate goals and our employees' achievement of their individual goals during 2023. For 2023, our employees, including our named executive officers, had the opportunity to earn cash incentive awards calculated as a percentage of pre-established bonus targets based on the Company's performance against the Company's corporate goals and such employee's individual performance against their own pre-established individual goals.

For fiscal year 2023, the incentive award for our Chief Executive Officer and each of our other named executive officers was based 100% on the Company's performance relative to the pre-established company goals, with an individual multiplier of 0 - 1.5x based on individual performance. Our Compensation Committee, however, reserves the discretion to adjust upward or downward any cash incentive award as it deems appropriate, provided that bonuses are capped at 150% of target amounts.

The table below summarizes the pre-established 2023 Company goals, their relative weighting, and level of achievement for each Company goal as approved by the Board in December 2023.

2023 Company goals	Weighting	2023 Company performance assessment (weighted assessment)
Deliver for Patients	**75%**	**80% (60)**
– Complete target number of patient starts across ZYNTEGLO and SKYSONA programs – Expand QTC network – Achieve timely disposition of drug product – Submit lovo-cel BLA by target date and begin commercial preparation, including marketing plans and manufacturing capacity		The Board recognized the Company's achievements in receiving FDA approval of LYFGENIA, while simultaneously executing on the commercial launches of ZYNTEGLO and SKYSONA. Additionally, the Company made significant progress in expanding its QTC network and completing patient starts for ZYNTEGLO and SKYSONA in 2023. However, the Board also acknowledged certain regulatory delays and delays in drug product disposition.
People & Business	**25%**	**100% (25)**
– Meet targets for cash runway, employee engagement, and employee diversity		The Board recognized that the Company achieved its goals with respect to employee diversity. The Company ended the year with positive employee engagement, as evidenced by a comprehensive employee engagement survey, and achieved a turnover rate lower than the industry average. The Board also acknowledged challenges with respect to the Company's cash runway, including because the Company did not receive a priority review voucher in connection with the approval of LYFGENIA, but noted progress as the Company worked toward a year-end equity raise.
Total	**100%**	**85%**

The pre-established individual goals and individual performance against those goals in 2023 applicable to our named executive officers were as follows:

Name	2023 individual performance assessment (multiplier)	2023 individual goals
Andrew Obenshain	100% (1x)	Cross-functional leadership in guiding the organization to accomplish each of the corporate goals
Thomas J. Klima	100% (1x)	Leadership in preparing the organization for commercial launches in the United States and ongoing operational efficiency
Richard A. Colvin	100% (1x)	Leadership in guiding the organization through late-stage development programs in severe genetic disease and associated regulatory interactions as well as overseeing the continued efforts towards the filing and approval of the lovo-cel BLA

The table below shows each named executive officer's target incentive award under the 2023 annual incentive program as a percentage of the named executive officer's annual base salary in 2023, the target incentive award opportunity in dollars for 2023 and the actual incentive awards to our named executive officers for 2023 performance, which were paid in March 2024.

Name	2023 Target Incentive Award (% of 2023 Base Salary)(1)	2023 Target Incentive Award Opportunity ($)	Actual Total 2023 Incentive Award Amount ($)
Andrew Obenshain	60%	409,440	348,024
Thomas J. Klima	45%	216,000	183,600
Richard A. Colvin	45%	220,500	187,425

(1) Target Incentive Awards as a percentage of base salary did not change in 2023.

In 2024, our Compensation Committee approved changes to the 2024 annual incentive program, providing that the award for our Chief Executive Officer will be based 100% on the Company's performance against the corporate goals and the award

for our other named executive officers will be based 80% on the Company's performance against the corporate goals and 20% on such executive officer's performance against his or her individual goals.

Long-Term Incentive Awards

Our long-term incentive equity awards are generally in the form of stock options and restricted stock units, which deliver equivalent value while using fewer authorized shares. Beginning in 2021, we granted performance-based restricted stock units based on relative total shareholder return to our Chief Executive Officer, and beginning in 2024, we extended these grants to our other executive officers, in each case to further tie executive compensation to stock price performance and in response to stockholder feedback. We typically make equity award grants to each of our executive officers upon commencement of employment, and annually in conjunction with our review of their individual performance. Additional equity award grants may be made in connection with a promotion, or as a special incentive. Our executives benefit from stock options only if our stock price increases through the creation of stockholder value, and the value of restricted stock units increase as our stock price increases. Accordingly, we believe stock options and restricted stock units provide meaningful incentives to our executives to achieve increases in the value of our stock over time, while performance-based restricted stock units further align our executive officers' interests with long-term stockholders' interests and company performance. In addition, the vesting feature of our long-term incentive grants contributes to executive retention by providing an incentive to our executives to remain employed by us during the vesting period.

All equity awards to our executive officers are approved by our Compensation Committee. The size of equity awards varies among our executive officers based on their positions, competitive market data, and annual performance assessments. All stock options granted by bluebird have exercise prices equal to the fair market value of our common stock on the date of grant, so that the recipient will not realize any value from his or her options unless our share price increases above the stock price on the date of grant. Accordingly, this portion of our executive officers' compensation is at risk and is directly aligned with stockholder value creation.

As part of the ongoing review of our compensation strategy and practices, the Compensation Committee determines the appropriate mix of the type of equity awards, based in part on recommendations from Aon, its independent compensation consultant. Because of the volatility of our stock price in relation to when equity grants are made, our equity compensation guidelines set forth aggregate grant targets reflecting stock options plus restricted stock units based on number of shares (rather than value of the equity grants), and these guidelines are developed based on and in reference to our equity grant data for our peer companies. The target mix for annual long-term incentive equity grants to our executive officers is generally split approximately one-half in stock options and one-half in restricted stock units (including performance-based restricted stock units). The Compensation Committee believes that this deliberate mix of equity ensures that compensation remains tied to stock performance and promotes retention. The Compensation Committee may adjust the mix of award types or approve different award types as part of the overall compensation strategy. Awards made in connection with a new, extended or expanded employment relationship may involve a different mix of equity awards, depending on the Compensation Committee's assessment of the total compensation package being offered.

In addition, long-term equity incentive grants of stock options and restricted stock units to our executive officers typically vest over four years, which we believe provides an incentive to our executives to add value to the Company over the long-term and to remain with bluebird. Typically, the stock options we grant to our executives have a ten-year term and vest as to 25% of the shares on the first anniversary of the date of grant and then the remaining shares vest in equal monthly installments thereafter until the fourth anniversary of such date. Vesting of option grants to employees ceases upon termination of employment and exercise rights typically cease three months following termination of employment, except in the case of death or disability. Prior to the exercise of an option, the stock option holder does not have any rights as a stockholder with respect to the shares subject to such option, including voting rights or the right to receive dividends or dividend equivalents. Annual restricted stock units granted to our executives generally vest in equal annual installments beginning on or about the first anniversary of the day of grant, until the fourth anniversary of such date.

2023 Equity Awards

In 2023, we granted our Chief Executive Officer a performance-based restricted stock unit award, to further align our Chief Executive Officer's compensation with stockholder experience (the "2023 Performance-Based RSU Award"). This 2023 Performance-Based RSU Award is earned based on total shareholder return compared to a selected group of companies that are considered relevant peers across business segment and size metrics, with a focus on industry classifications, revenue, and market capitalization, and which was developed in consultation with Aon (our "Named Peer Group"). The multiplier used to determine the number of earned restricted stock units could range between 0% and 200%, with a threshold achievement level at -25th percentile (as compared to the peer median) required to earn any restricted stock units, target achievement level equal to the peer median, and a ceiling achievement level at the +50th percentile (as compared to the peer median). The 2023

Performance-Based RSU Award, to the extent earned, vest in full on approximately the third anniversary of the grant date on the date performance achievement is certified by our Compensation Committee, subject to Mr. Obenshain's continued service. For 2023, the target number of shares subject to this 2023 Performance-Based RSU Award is 101,400 shares. Under the terms of this award, the number of performance-based restricted stock units earned is calculated by multiplying the target number of performance-based restricted stock units by a performance multiplier.

In its design of the performance-based restricted stock units, the Compensation Committee considered various design elements and alternative approaches including milestone-based approaches. Given the stage of the Company, early in its commercial product launches, the Compensation Committee felt that an overall relative stock performance metric best captures the value inflections that would potentially be unlocked through exceptional company performance, and is fully aligned with stockholder interests. Furthermore, a three-year measurement period provides an incentive for our executives to take a long-term view with respect to company performance.

In connection with the annual review of our named executive officers' performance during 2022 and consistent with our compensation philosophy, in 2023 our Compensation Committee approved the annual long-term equity incentive awards granted to our named executive officers serving at that time as set forth in the table below:

	2023 Option Award		2023 RSU Award		2023 Performance-Based RSU Award	
Name	# Shares	Grant date fair value	# Shares	Grant date fair value	Target # Shares	Grant Date Fair value
Andrew Obenshain	338,000	$1,142,440	67,600	$345,436	101,400	$518,154
Thomas J. Klima	110,000	$371,800	55,000	$281,050	—	$—
Richard A. Colvin	110,000	$371,800	55,000	$281,050	—	$—

The grant date fair values of the awards presented above and shown in the Summary Compensation Table below were determined in accordance with Financial Accounting Standards Board (FASB), Accounting Standards Codification (ASC), Topic 718.

Benefits and Other Compensation

Other compensation to our executives consists primarily of the broad-based benefits we provide to all full-time employees in the United States, including medical, dental and vision insurance, group life and disability insurance, an employee stock purchase plan and a 401(k) plan. Pursuant to our employee stock purchase plan, employees, including our named executive officers, have an opportunity to purchase our common stock at a discount on a tax-qualified basis through payroll deductions. The employee stock purchase plan is designed to qualify as an "employee stock purchase plan" under Section 423 of the Internal Revenue Code. The purpose of the employee stock purchase plan is to encourage our employees, including our named executive officers, to become our stockholders and better align their interests with those of our other stockholders. Pursuant to our 401(k) plan, employees, including our named executive officers, may elect to defer a portion of their current compensation up to the statutorily prescribed annual limit (which was $22,500 in 2023), with additional salary deferrals not to exceed $30,000 available to those employees 50 years of age or older, and to have the amount of this deferral contributed to our 401(k) plan. We make discretionary matching contributions and other employer contributions on behalf of eligible employees under our 401(k) plan. For fiscal year 2023, we matched a portion of eligible employee contributions equal to 100% of the first 4% of eligible contributions pursuant to our 401(k) plan's matching formula.

Currently, we do not view perquisites or other personal benefits as a significant component of our executive compensation program. Accordingly, we do not provide perquisites to our named executive officers, except in situations where we believe it is appropriate to assist an individual in the performance of his or her duties, to make him or her more efficient and effective, and for recruitment and retention purposes. All future practices with respect to perquisites or other personal benefits will be approved and subject to periodic review by our Compensation Committee.

Certain executives, including our named executive officers, may be entitled to certain severance and/or change in control protections pursuant to their employment agreements, which are described below under *—Employment Arrangements with Our Named Executive Officers.* Our goal in providing severance and change in control benefits is to offer sufficient cash continuity protection such that our executives will focus their full time and attention on the requirements of the business rather than the potential implications for their respective position. We prefer to have certainty regarding the potential severance amounts payable to the named executive officers, rather than negotiating severance at the time that a named executive officer's employment terminates.

Outstanding Equity Awards at Fiscal Year End

The following table sets forth information concerning the outstanding equity awards held by each of the named executive officers as of December 31, 2023.

		Option Awards				Stock Awards			
Name	Award Grant Date	Number of securities underlying unexercised options ((#) exercisable)	Number of securities underlying unexercised options (#) unexercisable)	Option exercise price ($/ share)	Option expiration date	Number of shares or units of stock that have not vested (#)	Market value of shares or units of stock that have not vested ($)[1]	Equity incentive plan awards: number of unearned shares, units or other rights that have not vested (#)	Equity incentive plan awards: market or payout value of unearned shares, units or other rights that have not vested ($)[1]
Andrew Obenshain	12/1/2016	17,721	—	$34.20	12/1/2026				
	2/1/2018	9,353	—	$102.24	2/1/2028				
	2/1/2019	7,827	—	$67.06	2/1/2029				
	3/2/2020	6,705	186 (2)	$36.78	3/2/2030				
	3/2/2020					862 (3)	1,190		
	11/2/2020	15,918	4,756 (4)	$26.45	11/2/2030				
	11/2/2020					2,585 (5)	3,567		
	2/16/2021	43,889	16,331 (6)	$14.17	2/16/2031				
	2/16/2021					15,059 (7)	20,781		
	8/2/2021	240,655	160,888 (8)	$12.76	8/2/2031				
	8/2/2021					100,387 (9)	138,534		
	2/1/2022	41,250	48,750 (4)	$7.80	2/1/2032				
	2/1/2022					13,500 (5)	18,630		
	2/1/2022							27,000 (10)	37,260
	3/1/2023	—	338,000 (4)	$5.11	3/1/2033				
	3/1/2023					67,600 (5)	93,288		
	3/1/2023							101,400 (10)	139,932
Thomas J. Klima	6/1/2021	64,812	35,573 (11)	$15.50	6/1/2031				
	6/1/2021					25,096 (5)	34,632		
	2/1/2022	22,910	27,090 (4)	$7.80	2/1/2032				
	2/1/2022					18,750 (5)	25,875		
	9/1/2022	6,874	15,126 (4)	$6.55	9/1/2032				
	9/1/2022					8,250 (5)	11,385		
	3/1/2023	—	110,000 (4)	$5.11	3/1/2033				
	3/1/2023					55,000 (5)	75,900		
Richard A. Colvin	11/1/2018	11,134	—	$64.83	11/1/2028				
	3/2/2020	4,305	124 (2)	$36.78	3/2/2030				
	3/2/2020					555 (3)	766		
	2/16/2021	5,835	2,195 (6)	$14.17	2/16/2031				
	2/16/2021					2,009 (7)	2,772		
	3/1/2021	27,573	12,580 (4)	$14.99	3/1/2031				
	3/1/2021					10,039 (5)	13,854		
	2/1/2022	12,100	14,300 (4)	$7.80	2/1/2032				
	2/1/2022					9,975 (5)	13,766		
	11/1/2022	16,250	43,750 (4)	$6.35	11/1/2032				
	11/1/2022					22,500 (5)	31,050		
	3/1/2023	—	110,000 (4)	$5.11	3/1/2033				
	3/1/2023					55,000 (5)	75,900		

(1) All unvested stock options and restricted stock awards were granted under our 2013 Stock Option and Incentive Plan, 2023 Incentive Award Plan, or our 2021 Inducement Plan. The market value of restricted stock units that have not vested is based on the closing price of $1.38 per share for our common stock on December 31, 2023, as reported on the NASDAQ Global Select Market.

(2) The shares underlying these options vest as follows: 25% vested on January 4, 2021, with the remainder of the shares vesting in equal monthly installments over the following three years through January 4, 2024, subject to the grantee's continued service with us through each applicable vesting date.

(3) These restricted stock unit awards vest in four equal annual installments of 25% starting on January 4, 2021 through January 4, 2024, subject to the grantee's continued service with us through each applicable vesting date.

(4) The shares underlying these options vest as follows: 25% vest on the one-year anniversary of the grant date, with the remainder of the shares vesting in equal monthly installments over the following three years, subject to the grantee's continued service with us through each applicable vesting date.

(5) These restricted stock unit awards vest in four equal annual installments of 25% starting on the one-year anniversary of the award grant date, subject to the grantee's continued service with us through each applicable vesting date.

(6) The shares underlying these options vest as follows: 25% vested on January 4, 2022, with the remainder of the shares vesting in equal monthly installments over the following three years through January 4, 2025, subject to the grantee's continued service with us through each applicable vesting date.

(7) These restricted stock unit awards vest in four equal annual installments of 25% starting on January 4, 2022 through January 4, 2025, subject to the grantee's continued service with us through each applicable vesting date.

(8) This performance-based option was earned based on performance criteria related to the separation of 2seventy bio. Subject to achievement of the performance criteria, the shares underlying this option vest as follows: 25% vested on August 2, 2022, with the remainder of the shares vesting in equal monthly installments over the following three years through August 2, 2025, subject to the grantee's continued service with us through each applicable vesting date.

(9) This performance-based restricted stock unit award was earned based on performance criteria related to the separation of 2seventy bio. Subject to achievement of the performance criteria, the PSU vests in four equal annual installments of 25% starting on August 2, 2022 through August 2, 2025, subject to the grantee's continued service with us through each applicable vesting date.

(10) These performance-based restricted stock unit awards are earned based on relative total shareholder return compared to a peer group of companies in the Standard & Poor Biotechnology Index and, to the extent earned, will vest approximately three years from the grant date, subject to the grantee's continued service with us on the applicable vesting date.

(11) The shares underlying this option vest as follows: 25% vested on May 10, 2022, with the remainder of the shares vesting in equal monthly installments over the following three years through May 10, 2025, subject to the grantee's continued service with us through each applicable vesting date.

Employment Arrangements with our Named Executive Officers

Andrew Obenshain. We have entered into an employment agreement, effective as of January 7, 2021, with Mr. Obenshain providing for his employment as the Company's President, Severe Genetic Disease. Mr. Obenshain currently serves as our Chief Executive Officer and Principal Executive Officer. Under his agreement Mr. Obenshain was initially entitled to receive a base salary of $435,000 (which has been subsequently increased as described above), subject to adjustment at the discretion of the Compensation Committee. Mr. Obenshain is also currently eligible for an annual cash incentive award targeted at 60% of his annual base salary. Mr. Obenshain is eligible to participate in our employee benefit plans, subject to the terms of those plans.

Thomas J. Klima. We have entered into an employment agreement, effective as of April 20, 2021, with Mr. Klima providing for his employment as the Company's Chief Commercial Officer. Mr. Klima currently serves as our Chief Commercial and Operating Officer. Under his agreement Mr. Klima is entitled to receive a base salary of $400,000 (which has been subsequently increased as described above), subject to adjustment at the discretion of the Compensation Committee. Mr. Klima is also eligible for an annual cash incentive award targeted at 45% of his annual base salary. Mr. Klima's employment agreement also provides for an initial grant of stock options and restricted stock units and his eligibility to participate in our employee benefit plans, subject to the terms of those plans.

Richard A. Colvin. We have entered into an employment agreement, effective as of October 31, 2022, with Dr. Colvin providing for his employment as the Company's Chief Medical Officer. Under his agreement Dr. Colvin is entitled to receive a

base salary of $480,000 (which has been subsequently increased as described above), subject to adjustment at the discretion of the Compensation Committee. Dr. Colvin is also eligible for an annual cash incentive award targeted at 45% of his annual base salary. Dr. Colvin is eligible to participate in our employee benefit plans, subject to the terms of those plans.

These employment agreements also contain provisions that provide for certain payments and benefits in the event of an involuntary termination of employment. In addition, the named executive officers may be entitled to accelerated vesting of their outstanding and unvested awards in certain circumstances as described below.

Involuntary termination of employment

Pursuant to their employment agreements, each named executive officer is eligible to receive certain payments and benefits in the event his employment is terminated by us without "cause" (as defined in the employment agreements) or in the event he terminates his employment with "good reason" (as defined in the employment agreements). Upon the timely execution of a severance agreement, including a general release of claims, each named executive officer is eligible to receive the following payments and benefits:

- 12 months of base salary continuation; and
- if he elects to continue his or her group healthcare benefits, to the extent authorized by and consistent with COBRA, we will pay the named executive officer a monthly cash payment equal to the monthly employer contribution we would have made to provide him health insurance if he had remained employed by us until the earlier of (1) 12 months following the date of termination, or (2) the end of the named executive officer's COBRA health continuation period.

Involuntary termination of employment in connection with a sale event

In addition, in the event that any of our named executive officers terminates his or her employment with us for good reason or his or her employment with us is terminated by us without "cause", in either case within 12 months following a "sale event" (as defined in the 2013 Stock Option and Incentive Plan (now referred to as a "change in control" under the 2023 Incentive Award Plan)), he or she will be entitled to receive the following payments and benefits (in lieu of the payments and benefits described above) upon the timely execution of a severance agreement, including a general release of claims:

- a lump sum cash payment equal to one times the sum of (1) the named executive officer's then-current base salary (or base salary in effect immediately prior to the sale event, if higher) and (2) the named executive officer's target annual cash incentive compensation; and
- if he or she elects to continue his or her group healthcare benefits, to the extent authorized by and consistent with COBRA, we will pay the named executive officer a monthly cash payment equal to the monthly employer contribution we would have made to provide him or her health insurance if he or she had remained employed by us until the earlier of (1) 12 months following the date of termination or (2) the end of the named executive officer's COBRA health continuation period; and
- all stock options and other stock-based awards granted to the named executive officer after the date of his or her employment agreement will become fully exercisable and non-forfeitable as of the date of the named executive officer's termination.

Non-Employee Director Compensation

The Compensation Committee of our Board is responsible for making recommendations to our Board on appropriate compensation levels and arrangements for our non-employee directors, ensuring they are consistent with our compensation policy and remain competitive with our peer companies. The Compensation Committee reviews our non-employee director compensation on an annual basis. In making recommendations, the Compensation Committee takes various factors into consideration, including:

- Non-employee directors' responsibilities and the form and amount of compensation paid to directors at our peer companies;
- Ability to retain and attract the most qualified and experienced non-employee directors to oversee the management of our business operations; and
- Advice of an independent compensation consultant to review our non-employee director compensation program and promote alignment with market practice and stockholder interests.

Our goal is to appropriately compensate non-employee directors for their leadership and expertise while aligning non-employee director interests with those of our stockholders. In line with this goal, our non-employee director compensation

policy is underpinned by the same philosophy and principles that govern our executive compensation program. The Compensation Committee generally targeted non-employee director compensation near the 50th percentile of compensation paid non-employee directors with the companies in our peer group.

Our non-employee director compensation program is designed to:

- ✓ Align non-employee director and stockholder interests through grants of non-statutory stock option awards and restricted stock units;
- ✓ Encourage a vested interest in our long-term business performance through stock ownership requirements;
- ✓ Align non-employee director compensation with our peer companies of comparable stage of development, market capitalization and size;
- ✓ Ensure a robust non-employee director compensation governance framework is in place; and
- ✓ Help us attract and retain talent for Board service to support the long-term value of the Company.

Based on these considerations, our Board has adopted a non-employee director compensation policy, which provides for annual cash retainers. The non-executive chair of our Board and the chair of each of our committees is entitled to greater compensation for his or her services than other members of our Board, which we believe is commensurate with the additional time commitment and additional responsibility required by the position held and is consistent with the compensation practices of our peer group companies. On April 18, 2023, our Board amended the non-employee director compensation policy to increase the cash retainers payable for service on our Audit Committee. The table below sets forth the cash retainer applicable to our directors in 2023 following such amendment.

Annual cash retainers under the Non-Employee Director Compensation Policy	2023 Cash Retainer ($)
Board:	
All non-employee members of the Board	$ 45,000
Additional retainer for non-executive chair of the Board	$ 35,000
Audit Committee:	
Additional retainer for chair of the Audit Committee	$ 20,000
Additional retainer for other members of the Audit Committee	$ 10,000
Compensation Committee:	
Additional retainer for chair of the Compensation Committee	$ 15,000
Additional retainer for other members of the Compensation Committee	$ 7,500
Nominating and Corporate Governance Committee:	
Additional retainer for chair of the Nominating and Corporate Governance Committee	$ 10,000
Additional retainer for other members of the Nominating and Corporate Governance Committee	$ 5,000

In addition, under our non-employee director compensation policy, new members of our Board are eligible for an initial equity grant under our stock option plan. The initial equity grant takes the form of a grant of stock options and restricted stock units that vest in equal annual installments over a three-year period, subject to the non-employee director's continued service. In addition, on the date of the annual meeting of stockholders, each continuing non-employee director who has served on the Board for the previous six months will be eligible to receive an annual equity grant in the form of stock options and restricted stock units. These annual equity grants will vest in full upon the earlier of the first anniversary of the date of grant or the date of the following annual meeting of stockholders, subject to the non-employee director's continued service. The number of shares subject to the initial equity grants and the annual equity grants are summarized in the table below. In the case of each initial equity grant and annual equity grant, the Board or Compensation Committee may exercise their discretion to provide for a different number of shares subject to equity awards in the event they determine a variation from the stated amount is warranted. All of the foregoing options are granted with an exercise price equal to the fair market value of our common stock on the date of grant. On April 18, 2023, our Board amended the non-employee director compensation policy to also increase the size of the annual and initial equity grant retainers payable thereunder as well as remove the additional equity grant retainer payable for service as non-executive chair of the Board. The equity grant policy applicable to our directors in 2023 is summarized below.

Equity grants under the Non-Employee Director Compensation Policy	Stock options (# of shares)	Restricted Stock Units (# of shares)
Initial equity grant	32,400	16,185
Annual equity grant	21,600	10,790

In March 2024, the Board, upon recommendation from the Compensation Committee and to further align certain director compensation to be within the 50th percentile of director compensation of the Company's peer group, approved the following changes to director equity compensation:

- adjusted initial equity grants for new Board members to 74,775 stock options and 37,350 restricted stock units; and
- adjusted annual equity grants for continuing Board members to 49,850 stock options and 24,900 restricted stock units.

The following table sets forth the compensation we paid to our non-employee directors during the year ended December 31, 2023. Other than as set forth in the table we did not pay any compensation, reimburse any expense of (other than reasonable out-of-pocket expenses to attend meetings of the Board or any committee), make any equity awards or non-equity awards to, or pay any other compensation to any of the other non-employee members of our Board in the year ended December 31, 2023. Mr. Obenshain, our current Chief Executive Officer, received no compensation for his service as a director, and, consequently, is not included in this table. The compensation received by Mr. Obenshain as an employee during the year ended December 31, 2023 is presented in the *Summary Compensation Table* above.

Name(1)	Fees earned or paid in cash($)(2)	Stock awards($)(2)	Option awards($)(2)	Total($)
John O. Agwunobi, M.D.	64,500	41,110	55,296	160,906
Mark Vachon	104,500	41,110	55,296	200,906
Elisabeth Leiderman, M.D.	71,500	41,110	55,296	167,906
Najoh Tita-Reid	57,500	41,110	55,296	153,906
Charlotte Jones-Burton, M.D., M.S.	50,000	41,110	55,296	146,406
Richard Paulson (3)	33,500	15,388	16,350	65,238
Nick Leschly	— (4)	41,110	55,296	96,406

(1) The aggregate number of shares of our common stock underlying stock options outstanding as of December 31, 2023 for the non-employee directors were: Dr. Agwunobi: 58,001, Mr. Vachon: 60,789, Dr. Leiderman: 41,657, Ms. Tita-Reid: 41,657, Dr. Jones-Burton: 34,100, Mr. Paulson: 7,500, and Mr. Leschly: 829,003. The aggregate number of restricted stock units outstanding as of December 31, 2023 for the non-employee directors was: Dr. Agwunobi: 10,790, Mr. Vachon: 10,790, Dr. Leiderman: 13,913, Ms. Tita-Reid: 13,899, Dr. Jones-Burton: 13,899, Mr. Paulson: 4,663, and Mr. Leschly: 18,790. Mr. Leschly's stock option and restricted stock unit amounts include equity awards granted to him from his time as the former CEO of bluebird.

(2) The amounts reported represent the aggregate grant date fair value of the stock options and restricted stock units granted to our non-employee directors during 2023 as computed in accordance with FASB ASC Topic 718, not including any estimates of forfeitures. See note 16 of *Notes to Consolidated Financial Statements* in elsewhere in this Annual Report for a discussion of assumptions made by the Company in determining the aggregate grant date fair value of our stock option and restricted stock unit awards for the fiscal year ended December 31, 2023. Note that the amounts reported in this column reflect the accounting cost for these grants, and do not correspond to the actual economic value that may be received by the non-employee directors from the exercise of the options or vesting of the restricted stock units.

(3) Mr. Paulson joined our Board in April 2023.

(4) Mr. Leschly elected to not receive the cash portion of his compensation.

Compensation Recovery Policy

Effective October 2023, our Board adopted a Policy for Recovery of Erroneously Awarded Compensation (the "Clawback Policy") to implement final clawback rules issued by the SEC. The Clawback Policy applies to our current and former

executive officers and subjects their incentive-based compensation received on or after October 2, 2023 to clawback in the event our company is required to prepare an accounting restatement to correct its material noncompliance with any financial reporting requirement under U.S. securities laws. In these circumstances, the Clawback Policy requires the Company to recover, reasonably promptly, the portion of incentive-based compensation that is deemed to have been erroneously awarded, unless the Compensation Committee (which administers the policy) determines that recovery would be impracticable and that one or more of the allowable impracticability conditions under SEC rules has been met.

In connection with the filing of this Annual Report on Form 10-K, the Company restated its consolidated financial statements as of and for the year ended December 31, 2022, and for each of the first three quarters of 2022 and 2023. The Company determined that this restatement would not result in the recoupment of any compensation because the restatement did not affect any incentive compensation approved, awarded or granted after October 2, 2023.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

Equity compensation plan information

The following table presents aggregate summary information as of December 31, 2023, regarding our existing equity compensation plans.

Plan Category	Number of Securities to be Issued Upon Exercise of Outstanding Options, Restricted Stock Units and Other Rights (a)		Weighted Average Exercise Price of Outstanding Options (b)		Number of Securities Remaining Available for Future Issuance Under Equity Compensation Plans (excluding securities reflected in column (a)) (c)	
Equity Compensation Plans Approved by Stockholders (1)	8,434,643	(2)	$ 14.16	(3)	6,214,812	(4)
Equity Compensation Plans Not Approved by Stockholders (5)	150,577		$ 15.50		1,099,423	
Total	8,585,220		$ 14.18		7,314,235	

(1) Consists of shares of common stock underlying equity awards under the 2013 Stock Option and Incentive Plan, the 2013 Employee Stock Purchase Plan (the "ESPP") and the 2023 Incentive Award Plan. Our 2013 Stock Option and Incentive Plan expired in June 2023 and no further shares were available for issuance thereunder after such date.

(2) Includes 4,207,682 shares subject to restricted stock units and 4,226,961 shares to be issued upon the exercise of outstanding stock options.

(3) The calculation does not take into account the 4,207,682 shares of common stock subject to outstanding restricted stock units. Such shares will be issued at the time the restricted stock units vest, without any cash consideration payable for those shares.

(4) Consists of shares available for future issuance under the ESPP and the 2023 Incentive Award Plan. As of December 31, 2023, 1,144,236 shares of common stock were available for issuance under the ESPP, and 5,070,576 shares of common stock were available for issuance under the 2023 Incentive Award Plan.

(5) We established an Inducement Plan in May 2021 (the "Inducement Plan") to be used exclusively for the grant of equity awards to prospective officers and employees who were not previously an employee or non-employee director as an inducement material to each such individual entering into employment with us. The Inducement Plan initially reserved 600,000 shares, which was later increased to an aggregate of 1,250,000 shares in January, 2022, for the issuance of awards to such individuals of non-qualified stock options, stock appreciation rights, restricted stock units, restricted stock awards, stock awards and/or dividend equivalent rights in the discretion of the plan administrator and in accordance with Nasdaq rules. Our Compensation Committee currently administers the Inducement Plan.

Security Ownership of Certain Beneficial Owners and Management

The following table sets forth the amount of common stock of bluebird beneficially owned, directly or indirectly, as of September 11, 2024, by (i) each current director of bluebird, (ii) each named executive officer of bluebird, (iii) all directors and executive officers of bluebird as a group, and (iv) each person who is known to bluebird to beneficially own more than five percent (5%) of the outstanding shares of common stock of bluebird, as determined through SEC filings, and the percentage of the common stock outstanding represented by each such amount. All shares of common stock shown in the table reflect sole voting and investment power except as otherwise noted.

Beneficial ownership is determined by the rules of the SEC and includes voting or investment power of the securities. As of September 11, 2024, bluebird had 193,913,585 shares of common stock outstanding. Shares of common stock subject to options to purchase, which are now exercisable or are exercisable within 60 days after September 11, 2024, or restricted stock units vesting within 60 days after September 11, 2024 are to be considered outstanding for purposes of computing the percentage ownership of the persons holding these options or other rights but are not to be considered outstanding for the purpose of computing the percentage ownership of any other person. Unless otherwise indicated, the address for each person listed below is c/o bluebird bio, Inc., 455 Grand Union Boulevard, Somerville, Massachusetts 02145.

Name and Address of Beneficial Owner	Number of Shares Beneficially Owned	Percentage of Shares Beneficially Owned
5% Stockholders		
The Goldman Sachs Group, Inc. (1)	12,180,740	6.3%
Directors and Named Executive Officers		
Nick Leschly (2)	1,123,597	*
John Agwunobi, M.D. (3)	84,465	*
Charlotte Jones-Burton, M.D., M.S. (4)	48,607	*
Elisabeth Leiderman, M.D. (5)	63,913	*
Richard Paulson (6)	4,054	*
Najoh Tita-Reid (7)	64,917	*
Mark Vachon (8)	85,830	*
Michael Cloonan	—	*
Richard A. Colvin (9)	205,558	*
Thomas J. Klima (10)	229,114	*
Andrew Obenshain (11)	818,093	*
All executive officers and directors as a group (13 persons) (12)	**2,779,178**	1.4%

* Represents holdings of less than 1%.

(1) Based solely on a Schedule 13G reporting beneficial ownership as of December 29, 2023, filed with the SEC on February 6, 2024, each of The Goldman Sachs Group, Inc. and The Goldman Sachs Group, LLC has shared voting power with respect to 12,180,235 shares and shared dispositive power with respect to 12,180,740 shares. The address of The Goldman Sachs Group, Inc. is 200 West Street, New York, New York 10282.

(2) Consists of 457,044 shares of common stock and 666,553 shares of common stock underlying options exercisable within 60 days of September 11, 2024. Such shares include 45,699 shares of common stock held in the Nick Leschly 2001 Trust for which Mr. Leschly is co-trustee with his spouse, and with whom he shares voting and dispositive power, and 123,000 shares of common stock held in the Nick Leschly Irrevocable GST Trust of 2019 for which Mr. Leschly is co-trustee with his spouse, and with whom he shares voting and dispositive power.

(3) Consists of 26,464 shares of common stock and 58,001 shares of common stock underlying options exercisable and restricted stock units vesting within 60 days of September 11, 2024.

(4) Consists of 17,007 shares of common stock and 31,600 shares of common stock underlying options exercisable and restricted stock units vesting within 60 days of September 11, 2024.

(5) Consists of 19,133 shares of common stock and 44,780 shares of common stock underlying options exercisable and restricted stock units vesting within 60 days of September 11, 2024.

(6) Consists of 1,554 shares of common stock and 2,500 shares of common stock underlying options exercisable and restricted stock units vesting within 60 days of September 11, 2024.

(7) Consists of 20,137 shares of common stock and 44,780 shares of common stock underlying options exercisable and restricted stock units vesting within 60 days of September 11, 2024.

(8) Consists of 25,041 shares of common stock and 60,789 shares of common stock underlying options exercisable and restricted stock units vesting within 60 days of September 11, 2024.

(9) Consists of 44,098 shares of common stock and 161,460 shares of common stock underlying options exercisable and restricted stock units vesting within 60 days of September 11, 2024.

(10) Consists of 46,458 shares of common stock and 182,656 shares of common stock underlying options exercisable and restricted stock units vesting within 60 days of September 11, 2024.

(11) Consists of 109,795 shares of common stock and 708,298 shares of common stock underlying options exercisable and restricted stock units vesting within 60 days of September 11, 2024.

(12) Consists of 774,014 shares of common stock and 2,005,164 shares of common stock underlying options and restricted stock units vesting within 60 days of September 11, 2024.

Item 13. Certain Relationships and Related Transactions and Director Independence

Procedures for related party transactions

We have adopted a related person transaction approval policy that governs the review of related person transactions at bluebird. Pursuant to this policy, if we want to enter into a transaction with a related person or an affiliate of a related person, our Chief Legal and Business Officer will review the proposed transaction to determine, based on applicable Nasdaq and SEC rules, if such transaction requires pre-approval by the Audit Committee and/or Board. If pre-approval is required, such matters will be reviewed at the next regular or special Audit Committee and/or Board meeting. In addition, our Compensation Committee charter requires that compensation arrangements with our executive officers be approved by our Compensation Committee. We may not enter into a related person transaction unless our Chief Legal and Business Officer has either specifically confirmed in writing that no further reviews are necessary or has confirmed that all requisite corporate reviews have been obtained.

Transactions with related persons

The following are certain transactions, arrangements and relationships with our directors, executive officers and stockholders owning 5% or more of our outstanding common stock, or any member of the immediate family of any of the foregoing persons, since January 1, 2022, other than equity and other compensation, termination, change in control and other arrangements, which are described under “Item 11. Executive Compensation.”

In connection with the 2021 separation of our severe genetic disease and oncology programs into two independent, publicly traded companies (the "Separation"), we entered into a separation agreement with 2seventy bio that, among other things, set forth bluebird’s agreements with 2seventy bio regarding the principal actions to be taken in connection with the Separation. Nick Leschly, our former Chief Executive Officer, is an executive officer of 2seventy bio, and Mr. Leschly is a member of our Board. The separation agreement identified assets transferred to, liabilities assumed by and contracts assigned to 2seventy bio as part of the Separation, and it provided for when and how these transfers, assumptions and assignments occurred. The purpose of the separation agreement was to provide 2seventy bio and bluebird with assets to operate their respective businesses and retain or assume liabilities related to those assets. Each of 2seventy bio and bluebird agreed to releases, with respect to pre-Separation claims, and cross indemnities, with respect to post-Separation claims, that were principally designed to place financial responsibility for the obligations and liabilities allocated to 2seventy bio under the separation agreement with 2seventy bio and financial responsibility for the obligations and liabilities allocated to bluebird under the separation agreement with bluebird. bluebird and 2seventy bio are also each subject to mutual 12-month employee non-solicit and non-hire restrictions, subject to certain customary exceptions.

In connection with the Separation, we also entered into two transition services agreements with 2seventy bio. Pursuant to the transition services agreements, we are obligated to provide and are entitled to receive certain transition services related to corporate functions, such as finance, human resources, internal audit, research and development, financial reporting, and information technology. Services provided by us to 2seventy bio will continue for an initial term of up to two years, unless earlier terminated or extended according to the terms of the transition services agreement. Services received and performed are paid at a mutually agreed upon rate. During the years ended December 31, 2023 and 2022, we incurred $5.0 million and $8.8 million, respectively, of net expense for services provided by 2seventy bio. In April 2022, we and 2seventy bio agreed to amend

certain terms of the transition services agreement to reduce our occupancy of a shared facility with 2seventybio and reduce the related fees.

We also entered into a tax matters agreement with 2seventy bio governing bluebird's and 2seventy bio's respective rights, responsibilities and obligations with respect to taxes (including taxes arising in the ordinary course of business and taxes, if any, incurred as a result of any failure of the distribution and certain related transactions to qualify as tax-free for U.S. federal income tax purposes), tax attributes, the preparation and filing of tax returns, the control of audits and other tax proceedings, and assistance and cooperation in respect of tax matters.

In addition, we entered into an employee matters agreement with 2seventy bio. The employee matters agreement allocates assets, liabilities and responsibilities relating to the employment, compensation and employee benefits of bluebird and 2seventy bio employees, and other related matters, in connection with the Separation, including the treatment of outstanding bluebird incentive equity awards and certain retirement and welfare benefit obligations. The employee matters agreement generally provides that, unless otherwise specified, 2seventy bio is responsible for liabilities associated with employees who transfer to 2seventy bio and employees whose employment terminated prior to the distribution but who primarily supported the 2seventy bio business, and bluebird is responsible for liabilities associated with other employees, including employees retained by bluebird. Pursuant to the employee matters agreement, the outstanding bluebird equity awards held by 2seventy bio and bluebird employees were adjusted immediately prior to the distribution, with the intent to maintain, immediately following the distribution, the economic value of the awards immediately before the distribution date.

We additionally entered into an intellectual property license agreement with 2seventy bio, pursuant to which each party granted a license to certain intellectual property and technology to the other. bluebird granted 2seventy bio a perpetual, worldwide, non-exclusive, royalty-free, fully paid-up license (or, as the case may be, sublicense) to certain intellectual property to allow 2seventy bio to use such intellectual property in connection with 2seventy bio's ongoing and future research and development activities and product candidates. 2seventy bio granted bluebird a perpetual, worldwide, non-exclusive, royalty-free, fully paid-up license (or, as the case may be, sublicense) to certain intellectual property for use in bluebird's existing products and product candidates. Such licenses between the parties generally allow current or future uses of the intellectual property in connection with each party's respective fields.

Board Independence

Our Board has determined, upon the recommendation of our Nominating and Corporate Governance Committee, that each of our directors, other than Andrew Obenshain who serves as our President and Chief Executive Officer, and Nick Leschly, who served as our President and Chief Executive Officer from October 2010 to November 2021, has no relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director and is independent within the meaning of the director independence standards of the Nasdaq rules. In addition, all members of the Audit Committee, Compensation Committee, and Nominating and Corporate Governance Committee satisfy the applicable independence criteria of the SEC and Nasdaq rules.

Item 14. Principal Accountant Fees and Services

Independent Registered Public Accounting Firm Fees

The following is a summary and description of fees billed by Ernst & Young LLP for the fiscal years ended December 31, 2023 and 2022.

	Fiscal Year 2023	Percentage of 2023 Services Approved by Audit Committee	Fiscal Year 2022	Percentage of 2022 Services Approved by Audit Committee
Audit fees (1)	$ 4,105,605	100%	$ 1,179,300	100%
Audit-related fees (2)	$ —	100%	$ —	100%
Tax fees (3)	$ 104,022	100%	$ 282,116	100%
All other fees (4)	$ —	100%	$ 2,585	100%
Total fees	**$ 4,209,627**	**100%**	**$ 1,464,001**	**100%**

(1) Audit fees in 2023 and 2022 include fees for our annual audit, and quarterly review procedures. Additionally, audit fees in 2023 and 2022 include fees incurred in connection with our public equity offerings, including registration statements, comfort letters and consents.

(2) Audit-related fees are related to accounting consultations.

(3) Tax fees are related to tax return preparation, tax advisory services and international tax compliance.

(4) All other fees are related to licensing fees paid to Ernst & Young LLP for access to its proprietary accounting research database.

PART IV

Item 15. Exhibits and Financial Statement Schedules

(a)(1) Financial Statements.

The response to this portion of Item 15 is set forth under Item 8 above.

(a)(2) Financial Statement Schedules.

All schedules have been omitted because they are not required or because the required information is given in the Consolidated Financial Statements or Notes thereto set forth under Item 8 above.

(a)(3) Exhibits.

See the Exhibit Index immediately before the signature page of this Annual Report on Form 10-K. The exhibits listed in the Exhibit Index are filed or incorporated by reference as part of this Annual Report on Form 10-K.

Item 16. Form 10-K Summary

Not applicable.

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bluebird bio, Inc.

Index to Consolidated Financial Statements

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of bluebird bio, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of bluebird bio, Inc. (the Company) as of December 31, 2023 and 2022, the related consolidated statements of operations and comprehensive loss, stockholders' equity and cash flows for each of the two years in the period ended December 31, 2023, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2023 and 2022, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2023, in conformity with U.S. generally accepted accounting principles.

The Company's Ability to Continue as a Going Concern

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the financial statements, the Company has suffered recurring operating losses and negative operating cash flows and has stated that substantial doubt exists about the Company's ability to continue as a going concern. Management's evaluation of the events and conditions and management's plans regarding these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Restatement of 2022 Consolidated Financial Statements

As discussed in Note 2 to the consolidated financial statements, the 2022 consolidated financial statements have been restated to correct misstatements associated with the Company's accounting for leases and other areas. See below for discussion of our related critical audit matter.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matters

The critical audit matters communicated below are matters arising from the current period audit of the financial statements that were communicated or required to be communicated to the audit committee and that: (1) relate to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matters below, providing separate opinions on the critical audit matters or on the accounts or disclosures to which they relate.

Accrued Clinical and Contract Research Organization Costs

Description of the Matter

As of December 31, 2023, the Company has recognized $73.2 million of Accrued expenses and other current liabilities. Included within this amount are accrued costs for clinical and contract research organization activities. As discussed in Note 3 to the consolidated financial statements, the Company records costs for research and development and contract research organization activities based upon estimates of costs incurred through the balance sheet date that have yet to be invoiced by the contract research organizations, clinical study sites, laboratories, consultants, or other vendors.

Auditing the Company's accrued clinical and contract research organization costs was especially challenging due to the fact that information necessary to estimate the accruals is accumulated from multiple sources. In addition, in certain circumstances, the determination of the nature and level of services that have been received during the reporting period requires judgment because the timing and pattern of vendor invoicing does not correspond to the level of services provided and there may be delays in invoicing from clinical study sites and other vendors.

How We Addressed the Matter in Our Audit

To evaluate the accrued clinical and contract research organization costs, our audit procedures included, among others, testing the completeness and accuracy of the underlying data used in the estimates and evaluating the significant judgements and estimates made by management to determine the accruals. We read a sample of contracts with the Company's contract research organization and clinical study sites to evaluate financial and other contractual terms. We discussed the progress of a sample of the clinical trials through the balance sheet date with the Company's operations personnel that oversee the clinical trials. We obtained third party confirmation from the clinical research organization to further test the underlying data used in management's estimate. We also analyzed fluctuations in accruals by trial throughout the period subject to audit and tested a sample of invoices from the third-party contract research organization, clinical study sites, laboratories, consultants, or other vendors. Finally, we compared subsequent invoices received from third parties to amounts accrued as of the balance sheet date.

Embedded Leases in Contract Manufacturing Agreements

Description of the Matter

As discussed in Notes 3 and 11 to the consolidated financial statements, the Company has elected to combine lease and non-lease components of agreements containing a lease, including certain contract manufacturing agreements, and allocate all of the contract consideration to the lease components. The Company has recognized right-of-use assets and lease liabilities associated with leases embedded within contract manufacturing agreements as finance leases. Right-of-use assets, which are classified within Property, plant and equipment, net on the consolidated balance sheet, and lease liabilities associated with finance leases were $84.5 million and $122.4 million as of December 31, 2023.

Auditing the Company's accounting for embedded leases was complex due to the judgment required in determining whether the agreements with contract manufacturing organizations contain an embedded lease and was also complex and challenging due to the volume of amendments to the agreements. Given the volume of amendments to these agreements, auditing the subsequent measurement of these embedded leases required additional audit effort to analyze whether they represented lease modifications and, if so, to test the remeasurement of the right-of-use assets and lease liabilities. Auditing the accounting for embedded leases was also especially challenging due to the material weakness identified by the Company over the accounting for leases. The material weakness required an increased extent of audit effort to test the identification of embedded leases and the accounting for agreements, including amendments, with contract manufacturing organizations.

How We Addressed the Matter in Our Audit

Our audit procedures included, among others, testing the completeness of the Company's identification of agreements with embedded leases and amendments by reading agreements with contract manufacturing organizations and evaluating the nature of the Company's relationship with vendors to which significant recurring payments were made. We read the underlying agreements and evaluated the Company's conclusion about whether there was an embedded lease and whether there was a modification to the lease agreement. To test the accuracy of the Company's measurement of right-of-use assets and lease liabilities, we read a sample of agreements, compared the contractual information to the inputs included in the Company's lease calculations and recalculated the Company's measurements. Because of the material weakness, we performed incremental audit procedures by increasing our sample sizes for the aforementioned procedures.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2012.

Boston, Massachusetts

September 13, 2024

bluebird bio, Inc.
Consolidated Balance Sheets
(in thousands, except per share amounts)

	As of December 31,	
	2023	2022
		(As Restated)
Assets		
Current assets:		
Cash and cash equivalents	$ 221,755	$ 113,006
Marketable securities	—	67,321
Prepaid expenses	14,800	8,618
Inventory	22,919	—
Receivables and other current assets	22,211	14,627
Total current assets	281,685	203,572
Marketable securities	—	1,414
Property, plant and equipment, net	65,936	67,636
Intangible assets, net	10,438	4,868
Goodwill	5,646	5,646
Operating lease right-of-use assets	201,113	230,885
Restricted cash and other non-current assets	54,343	55,793
Total assets	$ 619,161	$ 569,814
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 18,498	$ 14,867
Due to factor	2,520	—
Accrued expenses and other current liabilities	73,188	53,464
Operating lease liability, current portion	21,202	27,594
Finance lease liability, current portion	84,705	60,654
Total current liabilities	200,113	156,579
Operating lease liability, net of current portion	186,687	209,128
Finance lease liability, net of current portion	37,732	69,682
Other non-current liabilities	92	92
Total liabilities	424,624	435,481
Commitments and contingencies *Note 12*		
Stockholders' equity:		
Preferred stock, $0.01 par value, 5,000 shares authorized; 0 shares issued and outstanding at December 31, 2023 and December 31, 2022	$ —	$ —
Common stock, $0.01 par value, 250,000 and 125,000 shares authorized; 192,772 and 82,923 shares issued and outstanding at December 31, 2023 and December 31, 2022, respectively	1,905	830
Additional paid-in capital	4,454,756	4,185,988
Accumulated other comprehensive loss	(1,796)	(4,070)
Accumulated deficit	(4,260,328)	(4,048,415)
Total stockholders' equity	194,537	134,333
Total liabilities and stockholders' equity	$ 619,161	$ 569,814

See accompanying notes to consolidated financial statements.

bluebird bio, Inc.
Consolidated Statements of Operations and Comprehensive Loss
(in thousands, except per share amounts)

	Year ended December 31,	
	2023	2022
		(As Restated)
Revenue:		
Product revenue, net	$ 29,065	$ 2,739
Other revenue	432	858
Total revenues	29,497	3,597
Cost of product revenue	33,527	10,077
Gross margin	(4,030)	(6,480)
Operating expenses:		
Selling, general and administrative	165,510	140,326
Research and development	167,652	200,439
Restructuring expense	—	4,940
Total operating expenses	333,162	345,705
Gain from sale of priority review voucher, net	92,930	102,000
Loss from operations	(244,262)	(250,185)
Interest income	9,869	1,032
Interest expense	(16,353)	(6,322)
Other income, net	38,707	25,250
Loss before income taxes	(212,039)	(230,225)
Income tax benefit (expense)	126	(117)
Net loss	(211,913)	(230,342)
Net loss per share—basic	$ (1.93)	$ (2.93)
Net loss per share—diluted	$ (1.93)	$ (2.93)
Weighted-average number of common shares used in computing net loss per share—basic	109,825	78,585
Weighted-average number of common shares used in computing net loss per share—diluted	109,825	78,585
Other comprehensive income (loss):		
Other comprehensive income (loss), net of tax benefit (expense) of $0.0 million and $0.0 million for the years ended December 31, 2023 and 2022, respectively	2,274	(1,159)
Total other comprehensive income (loss)	2,274	(1,159)
Comprehensive loss	$ (209,639)	$ (231,501)

See accompanying notes to consolidated financial statements.

bluebird bio, Inc.
Consolidated Statements of Stockholders' Equity
(in thousands)

	Common stock		Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balances at December 31, 2021 (As Restated)	71,115	$ 711	$ 4,096,402	$ (2,911)	$ (3,818,073)	$ 276,129
Vesting of restricted stock units	979	10	(10)	—	—	—
Exercise of stock options	7	—	16	—	—	16
Purchase of shares under ESPP	68	1	238	—	—	239
Issuance of common stock	10,742	107	53,960	—	—	54,067
Issuance of unrestricted stock awards to settle accrued employee compensation	12	1	—	—	—	1
Stock-based compensation expense	—	—	35,382	—	—	35,382
Other comprehensive loss	—	—	—	(1,159)	—	(1,159)
Net loss	—	—	—	—	(230,342)	(230,342)
Balances at December 31, 2022 (As Restated)	82,923	830	4,185,988	(4,070)	(4,048,415)	134,333
Vesting of restricted stock	1,092	10	(206)	—	—	(196)
Exercise of stock options	24	—	92	—	—	92
Purchase of shares under ESPP	135	2	427	—	—	429
Issuance of common stock	106,333	1,063	247,304	—	—	248,367
Exercise of warrants	2,265	—	—	—	—	—
Stock-based compensation expense	—	—	21,151	—	—	21,151
Other comprehensive income	—	—	—	2,274	—	2,274
Net loss	—	—	—	—	(211,913)	(211,913)
Balances at December 31, 2023	192,772	$ 1,905	$ 4,454,756	$ (1,796)	$ (4,260,328)	$ 194,537

See accompanying notes to consolidated financial statements.

bluebird bio, Inc
Consolidated Statements of Cash Flows
(in thousands)

	Year ended December 31,	
	2023	2022
		(As Restated)
Cash flows from operating activities:		
Net loss	$ (211,913)	$ (230,342)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	28,525	8,344
Stock-based compensation expense	19,429	35,090
Noncash research and development expense (finance lease)	21,198	11,180
Noncash operating lease expense	29,772	23,562
Gain from sale of priority review voucher	(92,930)	(102,000)
Unrealized loss on equity securities	—	3,135
Excess inventory reserve	15,363	7,519
Other non-cash items	24	4,138
Gain on foreign currency exchange rates	167	(1,801)
Proceeds from sale of accounts receivable	5,040	—
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(3,894)	(4,820)
Inventory	(36,560)	(7,227)
Accounts payable	2,841	(7,709)
Operating lease liabilities	(28,887)	(14,072)
Accrued expenses and other liabilities	13,353	(45,974)
Accrued interest payable under finance lease	3,426	4,758
Net cash used in operating activities	(235,046)	(316,219)
Cash flows from investing activities:		
Purchase of property, plant and equipment	(4,189)	(8,208)
Purchases of marketable securities	(43,297)	—
Proceeds from maturities of marketable securities	108,521	131,445
Proceeds from sales of marketable securities	5,853	30,216
Purchase of intangible assets	(4,868)	(5,000)
Proceeds from sale of priority review voucher	92,930	102,000
Net cash provided by investing activities	154,950	250,453
Cash flows from financing activities:		
Proceeds from public offering of common stock, net of issuance costs	248,198	54,237
Proceeds from the transfer of invoices	2,520	—
Principal payments on finance leases	(54,367)	(36,734)
Other financing activities	(103)	16
Net cash provided by financing activities	196,248	17,519
Increase (decrease) in cash, cash equivalents and restricted cash	116,152	(48,247)
Cash, cash equivalents and restricted cash at beginning of year	158,445	206,692
Cash, cash equivalents and restricted cash at end of year	$ 274,597	$ 158,445
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 221,755	$ 113,006
Restricted cash included in receivables and other current assets	9,202	1,502
Restricted cash included in restricted cash and other non-current assets	43,640	43,937
Total cash, cash equivalents and restricted cash	$ 274,597	$ 158,445
Supplemental cash flow disclosures:		
Right-of-use assets obtained in exchange for operating lease liabilities	(708)	221,036
Purchases of property, plant and equipment included in accounts payable and accrued expenses	884	(3)
Purchases of intangible assets included in accrued expenses	1,221	—

Right-of-use assets obtained in exchange for finance lease liabilities	21,929	73,443
Offering expenses included in accounts payable and accrued expenses	964	170
Beneficiary interest obtained in transferred invoices	560	—
Cash paid (refunded) during the period for income taxes	(42)	253

See accompanying notes to consolidated financial statements.

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 April 2022, the Board of Directors of the Company approved a comprehensive restructuring plan intended to reduce operating expenses and enhance the Company's focus on achieving FDA approval for its programs in the U.S. As part of the restructuring, the Company reduced its workforce by approximately 30% across the second and third quarters of 2022. Refer to Note 19, *Reduction in workforce*, for more information on this restructuring.

In June 2022, the Company entered into an Equity Distribution Agreement (the "Equity Distribution Agreement") with Goldman Sachs & Co. LLC ("Goldman") to sell shares of the Company's common stock up to $75.0 million, from time to time, through an "at the market" equity offering program under which Goldman would act as manager. Under the Equity Distribution Agreement, the Company paid to Goldman a commission equal to up to 3.0% of the gross proceeds of any Common Stock sold through Goldman under the Equity Distribution Agreement. The Company terminated the Equity Distribution Agreement in August 2023.

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 December 31, 2023, 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 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 million, available in four tranches.

Since its inception, the Company has incurred significant operating losses and negative operating cash flows. As of December 31, 2023, the Company had an accumulated deficit of $4.3 billion. During the twelve months ended December 31, 2023, the Company incurred a net loss of $211.9 million and used $235.0 million of cash in operations. As of December 31, 2023, the Company had cash and cash equivalents of $221.8 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. Restatement of Previously Issued Financial Statements

The Company has restated its previously issued consolidated financial statements as of and for the year ended December 31, 2022, and its previously issued unaudited condensed consolidated financial information for each of the first three quarters of the years ended December 31, 2023 and 2022 in this Form 10-K. The restated unaudited condensed consolidated financial information is disclosed in Note 21, *Quarterly Financial Information (Unaudited)*. The Company has also restated impacted amounts within the notes to the consolidated financial statements, as applicable.

Leases

The Company identified errors pertaining to 1) the application of its accounting policy for the treatment of combining lease and non-lease components in arrangements that contain a lease, 2) the missed identification of embedded leases and improper accounting for its lease arrangements, including embedded leases and lease modifications, and 3) the misclassification of such embedded leases. The Company's accounting policy is to combine lease and non-lease components in all agreements. The Company determined that it did not consistently combine such components in its arrangements with contract manufacturing organizations ("CMOs") and a contract testing organization ("CTO") and certain office and laboratory space agreements that contain a lease. Correctly combining lease and non-lease components on a consistent basis, correctly identifying embedded leases and lease modifications associated with a change in scope of lease or non-lease components, and correcting other accounting errors for all lease arrangements resulted in the following adjustments as of and for the year ended December 31, 2022:

- Three embedded leases with CMOs that were incorrectly classified as operating leases were determined to be finance leases. As a result, property, plant and equipment (right-of-use finance lease assets), finance lease liabilities, and interest expense were understated and operating lease right-of-use assets, accounts payable and accrued expenses, operating lease liabilities and research and development expense were overstated. The impact to prepaid expenses, other non-current assets, and other income varied from period to period.

- Two additional embedded finance leases were identified in existing CMO agreements and one additional embedded finance lease was identified in an existing CTO agreement. As a result, property, plant and equipment (right-of-use finance lease assets), finance lease liabilities, and interest expense were understated, accounts payable and accrued expenses were overstated, and on a net basis, research and development expense was understated. The impact to prepaid expenses and other income varied from period to period.

- Minimum fixed payments for certain operating leases related to office and laboratory space were understated and sublease income was improperly classified within selling, general and administrative expense, which resulted in an understatement of operating lease right-of-use assets, operating lease liabilities, selling, general and administrative expense and other income. The impact to other current assets varied from period to period.

- Right-of-use assets associated with finance leases used in performing research and development activities without alternative future use at lease commencement or upon lease modification were immediately expensed to research and development expense which is the primary driver of the $95.6 million adjusted to accumulated deficit as of December 31, 2021. Subsequent finance lease amortization costs are expensed as research and development until achievement of regulatory approval and a subsequent modification to the embedded lease occurs.

- Reclassification of the principal portion of embedded finance lease payments as financing activities as well as other reclassifications of cash and non-cash activity within operating activities on the consolidated statement of cash flows.

Other Adjustments

In addition to the misstatements identified above, the Company has corrected other immaterial errors. These other errors are quantitatively and qualitatively immaterial, individually and in the aggregate. However, the Company has corrected these other errors as part of the correction for the material errors related to embedded leases.

Impact of Restatement

The following tables represent the as-restated consolidated balance sheet as of December 31, 2022, and the related consolidated statements of operations and comprehensive loss, stockholders' equity and cash flows for the year ended December 31, 2022. These tables also present a reconciliation from the prior period as previously reported to the as-restated amounts. The amounts as previously reported for fiscal year 2022 were derived from the Annual Report on Form 10-K for the fiscal year ended December 31, 2022, filed on March 29, 2023.

Consolidated Balance Sheet:

(in thousands)	As of December 31, 2022			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Assets				
Current assets:				
Cash and cash equivalents	$ 113,006	$ —	$ —	$ 113,006
Marketable securities	67,321	—	—	67,321
Prepaid expenses	8,374	244	—	8,618
Receivables and other current assets	10,787	3,840	—	14,627
Total current assets	199,488	4,084	—	203,572
Marketable securities	1,414	—	—	1,414
Property, plant and equipment, net	9,362	58,585	(311)	67,636
Goodwill	5,646	—	—	5,646
Intangible assets, net	4,868	—	—	4,868
Operating lease right-of-use assets	281,996	(51,111)		230,885
Restricted cash and other non-current assets	52,128	3,665	—	55,793
Total assets	$ 554,902	$ 15,223	$ (311)	$ 569,814
Liabilities and Stockholders' Equity				
Current Liabilities:				
Accounts payable	$ 25,092	$ (10,225)	$ —	$ 14,867
Accrued expenses and other current liabilities	51,985	(520)	1,999	53,464
Operating lease liability, current portion	51,160	(23,566)	—	27,594
Finance lease liability, current portion	—	60,654	—	60,654
Total current liabilities	128,237	26,343	1,999	156,579
Operating lease liability, net of current portion	230,230	(21,102)	—	209,128
Financing lease liability, net of current portion	—	69,682	—	69,682
Other non-current liabilities	92	—	—	92
Total liabilities	358,559	74,923	1,999	435,481
Commitments and contingencies (Note 12)				
Stockholders' Equity:				
Preferred stock, $0.01 par value, 5,000 shares authorized; 0 shares issued and outstanding at December 31, 2022	$ —	$ —	$ —	$ —
Common stock, $0.01 par value, 125,000 shares authorized; 82,923 shares issued and outstanding at December 31, 2022	830	—	—	830
Additional paid-in capital	4,186,086	—	(98)	4,185,988
Accumulated other comprehensive loss	(4,070)	—	—	(4,070)
Accumulated deficit	(3,986,503)	(59,700)	(2,212)	(4,048,415)
Total stockholders' equity	196,343	(59,700)	(2,310)	134,333
Total liabilities and stockholders' equity	$ 554,902	$ 15,223	$ (311)	$ 569,814

Consolidated Statement of Operations and Comprehensive Loss

(in thousands, except per share amounts)	Year ended December 31, 2022			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Revenue:				
Product revenue, net	$ 2,739	$ —	$ —	$ 2,739
Other revenue	858	—	—	858
Total revenues	3,597	—	—	3,597
Cost of product revenue	10,077	—	—	10,077
Gross margin	(6,480)	—	—	(6,480)
Operating expenses:				
Research and development	240,764	(40,121)	(204)	200,439
Selling, general and administrative	136,908	3,516	(98)	140,326
Restructuring expenses	4,940	—	—	4,940
Total operating expenses	382,612	(36,605)	(302)	345,705
Gain from sale of priority review voucher, net	102,000	—	—	102,000
(Loss) Income from operations	(287,092)	36,605	302	(250,185)
Interest income	1,032	—	—	1,032
Interest expense	—	(6,322)	—	(6,322)
Other (expense) income, net	19,599	5,651	—	25,250
(Loss) income before income taxes	(266,461)	35,934	302	(230,225)
Income tax (expense) benefit	(117)	—	—	(117)
Net (loss) income	(266,578)	35,934	302	(230,342)
Net (loss) income per share - basic	$ (3.39)	$ 0.46	$ —	$ (2.93)
Net (loss) income per share - diluted	$ (3.39)	$ 0.46	$ —	$ (2.93)
Weighted-average number of common shares used in computing net (loss) income per share - basic:	78,585	—	—	78,585
Weighted-average number of common shares used in computing net (loss) income per share - diluted:	78,585	—	—	78,585
Other comprehensive (loss) income:				
Other comprehensive (loss) income, net of tax (benefit) expense of $0.0 million for the year ended December 31, 2022	(1,159)	—	—	(1,159)
Total other comprehensive (loss) income	(1,159)	—	—	(1,159)
Comprehensive (loss) income	$ (267,737)	$ 35,934	$ 302	$ (231,501)

Consolidated Statement of Changes in Stockholders' Equity:

(in thousands)	Common stock Shares	Common stock Amount	Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total stockholders' equity
As Previously Reported						
Balances at December 31, 2021	71,115	$ 711	$ 4,096,402	$ (2,911)	$ (3,719,925)	$ 374,277
Vesting of restricted stock	979	10	(10)	—	—	—
Exercise of stock options	7	—	16	—	—	16
Purchase of shares under ESPP	68	1	238	—	—	239
Issuance of common stock	10,742	107	53,960	—	—	54,067
Issuance of unrestricted stock awards to settle accrued employee compensation	12	1	—	—	—	1
Stock-based compensation expense	—	—	35,480	—	—	35,480
Other comprehensive loss	—	—	—	(1,159)	—	(1,159)
Net (loss) income	—	—	—	—	(266,578)	(266,578)
Balances at December 31, 2022	82,923	$ 830	$ 4,186,086	$ (4,070)	$ (3,986,503)	$ 196,343
Adjustments to Leases						
Balances at December 31, 2021	—	$ —	$ —	$ —	$ (95,634)	$ (95,634)
Vesting of restricted stock	—	—	—	—	—	—
Exercise of stock options	—	—	—	—	—	—
Purchase of shares under ESPP	—	—	—	—	—	—
Issuance of common stock	—	—	—	—	—	—
Issuance of unrestricted stock awards to settle accrued employee compensation	—	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—
Other comprehensive loss	—	—	—	—	—	—
Net (loss) income	—	—	—	—	35,934	35,934
Balances at December 31, 2022	—	$ —	$ —	$ —	$ (59,700)	$ (59,700)
Other Adjustments						
Balances at December 31, 2021	—	—	—	—	(2,514)	(2,514)
Vesting of restricted stock	—	—	—	—	—	—
Exercise of stock options	—	—	—	—	—	—
Purchase of shares under ESPP	—	—	—	—	—	—
Issuance of common stock	—	—	—	—	—	—
Issuance of unrestricted stock awards to settle accrued employee compensation	—	—	—	—	—	—
Stock-based compensation expense	—	—	(98)	—	—	(98)
Other comprehensive loss	—	—	—	—	—	—
Net (loss) income	—	—	—	—	302	302
Balances at December 31, 2022	—	$ —	$ (98)	$ —	$ (2,212)	$ (2,310)
As Restated						
Balances at December 31, 2021	71,115	711	4,096,402	(2,911)	(3,818,073)	276,129
Vesting of restricted stock	979	10	(10)	—	—	—
Exercise of stock options	7	—	16	—	—	16
Purchase of shares under ESPP	68	1	238	—	—	239
Issuance of common stock	10,742	107	53,960	—	—	54,067

Issuance of unrestricted stock awards to settle accrued employee compensation	12	1	—	—	—	1
Stock-based compensation expense	—	—	35,382	—	—	35,382
Other comprehensive loss	—	—	—	(1,159)	—	(1,159)
Net (loss) income	—	—	—	—	(230,342)	(230,342)
Balances at December 31, 2022	82,923	$ 830	$ 4,185,988	$ (4,070)	$ (4,048,415)	$ 134,333

Consolidated Statement of Cash Flows

(in thousands)	Year Ended December 31, 2022			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Cash flows from operating activities:				
Net (loss) income	$ (266,578)	$ 35,934	$ 302	$ (230,342)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation and amortization	5,001	3,547	(204)	8,344
Stock-based compensation expense	35,188	—	(98)	35,090
Noncash research and development expense (finance lease)	—	11,180	—	11,180
Noncash operating lease expense	—	23,562	—	23,562
Gain from sale of priority review voucher	(102,000)	—	—	(102,000)
Unrealized loss on equity securities	3,135	—	—	3,135
Excess inventory reserve	7,519	—	—	7,519
Other non-cash items	3,904	234	—	4,138
(Gain) on foreign currency exchange rates	—	(1,801)	—	(1,801)
Changes in operating assets and liabilities:				
Prepaid expenses and other assets	3,260	(8,080)	—	(4,820)
Inventory	(7,227)	—	—	(7,227)
Operating right of use assets	42,706	(42,706)	—	—
Accounts payable	(920)	(6,789)	—	(7,709)
Accrued expenses and other liabilities	(51,228)	5,254	—	(45,974)
Accrued interest payable under finance lease	—	4,758	—	4,758
Operating lease liabilities	(25,713)	11,641	—	(14,072)
Net cash used in operating activities	(352,953)	36,734	—	(316,219)
Cash flows from investing activities:				
Purchase of property, plant and equipment	(8,208)	—	—	(8,208)
Proceeds from maturities of marketable securities	131,445	—	—	131,445
Proceeds from sales of marketable securities	30,216	—	—	30,216
Purchase of intangible assets	(5,000)	—	—	(5,000)
Proceeds from sale of priority review voucher	102,000	—	—	102,000
Net cash provided by investing activities	250,453	—	—	250,453
Cash flows from financing activities:				
Proceeds from exercise of stock options and ESPP contributions	16	—	—	16
Principal payments on finance lease	—	(36,734)	—	(36,734)
Proceeds from public offering of common stock, net of issuance costs	54,237	—	—	54,237
Net cash provided by financing activities	54,253	(36,734)	—	17,519
(Decrease) increase in cash, cash equivalents and restricted cash	(48,247)	—	—	(48,247)
Cash, cash equivalents and restricted cash at beginning of year	206,692	—	—	206,692
Cash, cash equivalents and restricted cash at end of year	$ 158,445	$ —	$ —	$ 158,445
Reconciliation of cash, cash equivalents and restricted cash:				
Cash and cash equivalents	$ 113,006	$ —	$ —	$ 113,006
Restricted cash included in receivables and other current assets	1,502	—	—	1,502
Restricted cash included in restricted cash and other non-current assets	43,937	—	—	43,937
Total cash, cash equivalents and restricted cash	$ 158,445	$ —	$ —	$ 158,445
Supplemental cash flow disclosures:				
Right-of-use assets obtained in exchange for operating lease liabilities	236,003	(14,967)	—	221,036
Increase (Reduction) of right of use asset and associated operating lease liability due to lease reassessment	(2,833)	2,833	—	—
Purchases of property, plant and equipment included in accounts payable and accrued expenses	(3)	—	—	(3)
Offering expenses included in accounts payable and accrued expenses	170	—	—	170
Right-of-use assets obtained in exchange for finance lease liabilities	—	73,443	—	73,443
Cash paid during the period for income taxes	253	—	—	253

3. Summary of significant accounting policies and basis of presentation

Basis of presentation

The Company's consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States ("GAAP"). The accompanying consolidated financial statements include the accounts of the Company and its wholly owned or controlled subsidiaries. All intercompany accounts and transactions have been eliminated. 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 the Accounting Standards Updates ("ASUs") of the Financial Accounting Standards Board ("FASB").

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.

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.

Principles of consolidation

The accompanying consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries.

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.

Foreign currency translation

The financial statements of the Company's subsidiaries with functional currencies other than the U.S. dollar are translated into U.S. dollars using period-end exchange rates for assets and liabilities, historical exchange rates for stockholders' equity and weighted average exchange rates for operating results. Translation gains and losses are included in other comprehensive income (loss) in stockholders' equity. Foreign currency transaction gains and losses are included in other income, net in the results of operations.

Segment information

The Company operates in a single segment, focusing on researching, developing and commercializing potentially transformative gene therapies for severe genetic diseases. Consistent with its operational structure, its chief operating decision maker manages and allocates resources at a global, consolidated level. Therefore, results of the Company's operations are

reported on a consolidated basis for purposes of segment reporting. All material long-lived assets of the Company reside in the United States.

Cash and cash equivalents

The Company considers all highly liquid investments purchased with original final maturities of 90 days or less from the date of purchase to be cash equivalents. Cash equivalents comprise marketable securities with maturities of less than 90 days when purchased. Cash equivalents are reported at fair value.

Marketable securities

The Company's marketable securities are maintained by investment managers and consist of U.S. government agency securities and treasuries, equity securities, corporate bonds and commercial paper. Debt securities are carried at fair value with the unrealized gains and losses included in other comprehensive income (loss) as a component of stockholders' equity until realized. Any premium arising at purchase is amortized to the earliest call date and any discount arising at purchase is accreted to maturity. Amortization and accretion of premiums and discounts are recorded in interest income, net. Equity securities with readily determinable fair values are also carried at fair value with unrealized gains and losses included in other income, net. Realized gains and losses on both debt and equity securities are determined using the specific identification method and are included in other income, net.

The Company classifies equity securities with readily determinable fair values, which would be available for use in its current operations, as current assets even though the Company may not dispose of such marketable securities within the next 12 months. Equity securities are included in the balance of marketable securities on the Company's consolidated balance sheets. The Company classifies marketable securities with a remaining maturity when purchased of greater than three months as available-for-sale. Marketable securities with a remaining maturity date greater than one year are classified as non-current assets.

Effective January 1, 2020, the Company adopted ASU 2016-13, *Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses on Financial Statements* ("ASU 2016-13" or "ASC 326"), using the effective date method. As the Company had never recorded any other-than-temporary-impairment adjustments to its available-for-sale debt securities prior to the effective date, no transition provisions are applicable to the Company.

The Company assesses its available-for-sale debt securities under the available-for-sale debt security impairment model in ASC 326 as of each reporting date in order to determine if a portion of any decline in fair value below carrying value recognized on its available-for-sale debt securities is the result of a credit loss. The Company records credit losses in the consolidated statements of operations and comprehensive loss as credit loss expense within other income, net, which is limited to the difference between the fair value and the amortized cost of the security. To date, the Company has not recorded any credit losses on its available-for-sale debt securities.

Accrued interest receivable related to the Company's available-for-sale debt securities is presented within receivables and other current assets on the Company's consolidated balance sheets. The Company has elected the practical expedient available to exclude accrued interest receivable from both the fair value and the amortized cost basis of available-for-sale debt securities for the purposes of identifying and measuring any impairment. The Company writes off accrued interest receivable once it has determined that the asset is not realizable. Any write-offs of accrued interest receivable are recorded by reversing interest income, recognizing credit loss expense, or a combination of both. To date, the Company has not written off any accrued interest receivables associated with its marketable securities.

Concentrations of counter-party and liquidity risk

To reduce exposure to counter-party and liquidity risk, the Company maintains concentration limits across all of its overnight bank deposits. As of December 31, 2023, the Company's restricted cash balances were held in bank deposits. The remaining balance of cash and cash equivalents are invested in money market funds, U.S. government agency securities and treasuries, and investment grade commercial paper in line with the company's board approved investment policy. The Company has not experienced any credit losses on its deposits of cash and cash equivalents.

Periodically, the Company reviews the credit risk of its deposits based on public information including credit agency reports and public financial reporting.

Fair value of financial instruments

The Company has certain financial assets and liabilities recorded at fair value which have been classified as Level 1, 2 or 3 within the fair value hierarchy as described in the accounting standards for fair value measurements:

Level 1—Fair values are determined utilizing quoted prices (unadjusted) in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.

Level 2—Fair values are determined utilizing quoted prices for identical or similar assets or liabilities in active markets or other market observable inputs such as interest rates, yield curves and foreign currency spot rates.

Level 3—Prices or valuations that require inputs that are both significant to the fair value measurement and unobservable.

To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

Items measured at fair value on a recurring basis include marketable securities (see Note 5, *Marketable securities,* and Note 6, *Fair value measurements*). The carrying amounts of accounts payable and accrued expenses approximate their fair values due to their short-term nature.

Goodwill

Goodwill represents the excess of the purchase price over the fair value of the net assets acquired when accounted for using the acquisition method of accounting for business combinations. Goodwill is not amortized but is evaluated for impairment within the Company's single reporting unit on an annual basis, during the fourth quarter, or more frequently if an event occurs or circumstances change that would more-likely-than-not reduce the fair value of the Company's reporting unit below its carrying amount. The Company performs a one-step quantitative test and records the amount of goodwill impairment, if any, as the excess of a reporting unit's carrying amount over its fair value, not to exceed the total amount of goodwill allocated to the reporting unit. The Company has not recognized any impairment charges related to goodwill to date.

A significant sustained decrease in the Company's common stock, may in the future be a potential indicator that all or a portion of our goodwill is impaired and may require a quantitative impairment assessment of the Company's goodwill at an interim date, which may result in an impairment charge in future periods. While management cannot predict if or when future goodwill impairments may occur, an impairment could have material adverse effects on the Company's financial condition, operating income, net assets, and/or the Company's cost of, or access to, capital.

Intangible assets, net

Intangible assets, net consist of in-licensed rights with finite lives, net of accumulated amortization. The Company amortizes its intangible assets using the straight-line method over their estimated economic lives and periodically reviews for impairment.

Property, plant and equipment

Property, plant and equipment is stated at cost. Maintenance and repairs that do not improve or extend the lives of the respective assets are expensed to operations as incurred. Depreciation and amortization is calculated using the straight-line method over the estimated useful lives of the assets, which are as follows:

Asset	Estimated useful life
Building	40 years
Computer equipment and software	3 years
Furniture and fixtures	5 years
Laboratory equipment	3-5 years
Finance lease right-of-use asset	Shorter of the useful life or remaining lease term
Leasehold improvements	Shorter of the useful life or remaining lease term

Impairment of long-lived assets

The Company reviews long-lived assets when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. Recoverability is measured by comparison of the book values of the assets to future net undiscounted cash flows that the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the book value of the assets exceed their fair value, which is measured based on the projected discounted future net cash flows arising from the assets.

Leases

At the inception of an arrangement, the Company determines whether the arrangement is or contains a lease and its classification based on the unique facts and circumstances present in the arrangement. Leases with a term greater than one year are recognized on the balance sheet as right-of-use assets and current and non-current lease liabilities, as applicable. Right-of-use assets associated with finance leases used in the performance of research and development activities without alternative future use at lease commencement or at lease modification are immediately expensed to research and development expense pursuant to the guidance under ASC 730, Research and Development Costs.

Lease liabilities and their corresponding right-of-use assets are initially recorded based on the present value of lease payments over the expected remaining lease term. Certain adjustments to the right-of-use asset may be required for items such as incentives received. The interest rate implicit in lease contracts is typically not readily determinable. As a result, the Company utilizes its incremental borrowing rate to discount lease payments, which reflects the fixed rate at which the Company could borrow on a collateralized basis the amount of the lease payments in the same currency, for a similar term, in a similar economic environment. To estimate its incremental borrowing rate, a credit rating applicable to the Company is estimated using a synthetic credit rating analysis since the Company does not currently have a rating agency-based credit rating. Subsequent to the initial measurement, the Company will continue to account for:

- the lease liability at the net present value using the incremental borrowing rate that was in effect as of the lease commencement, lease modification, or transition date, and;
- the finance lease right-of-use asset and operating lease expense on a straight line basis.

The Company has elected not to recognize leases with an original term of one year or less on the balance sheet. The Company typically only includes an initial lease term in its assessment of a lease arrangement. Options to renew a lease are not included in the Company's assessment unless there is reasonable certainty that the Company will renew.

Assumptions made by the Company at the commencement date are re-evaluated upon occurrence of certain events, including a lease modification. A lease modification results in a separate contract when the modification changes the scope of or consideration for a lease or non-lease component under the agreement, such as when the lessor grants the lessee an additional right-of-use not included in the original lease and when lease payments increase commensurate with the standalone price for the additional right-of-use. When a lease modification results in a separate contract, it is accounted for in the same manner as a new lease.

In accordance with ASC 842, components of a lease should be split into three categories: lease components, non-lease components, and non-components. The fixed and in-substance fixed contract consideration (including any consideration related to non-components) must be allocated based on the respective relative fair values to the lease components and non-lease components.

Entities may elect not to separate lease and non-lease components. Rather, entities would account for each lease component and related non-lease component together as a single lease component. The Company has elected to account for lease and non-

lease components together as a single lease component for real-estate leases and embedded CMO and CTO agreements and to allocate all the contract consideration to the lease component only, or to individual lease components on a relative standalone selling price basis when the lease terms associated with the individual lease components are not otherwise co-terminus. Lease modifications associated with embedded leases in contract manufacturing and testing arrangements arise with frequency based on the complexity of the gene therapy manufacturing processes and the related services provided by contract manufacturing and testing organizations, which are combined with lease components. The Company applies judgment in determining whether the contractual terms for the delivery of services represent a change in the scope or consideration of the arrangements, which are accounted for as lease modifications, or represent variable lease payments, which are recognized as incurred.

ASC 842 allows for the use of judgment in determining whether the assumed lease term is for a major part of the remaining economic life of the underlying asset and whether the present value of lease payments represents substantially all of the fair value of the underlying asset. The Company applies the guidance in ASC 842-10-55-2 to assist in determining lease classification across its portfolio of leases.

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.

Factoring agreement

In December 2023, the Company entered into a factoring agreement with a non-bank financial institution. The proceeds from the factoring agreement will be used to fund general working capital needs. The factoring agreement provides the Company access to up to $100.0 million on a revolving basis, measured by the outstanding balance of purchased accounts ("Purchased Accounts" as defined in the Invoice Purchase and Sales Agreement) sold on the purchase dates ("Purchase Dates" as defined in the Invoice Purchase and Sales Agreement) from time to time. The Company sells 100% of the invoice to Alterna Capital Solutions LLC, and the upfront purchase price for a Purchased Account is 90% of the invoice amount. The remaining 10% less applicable fees is payable only if and when Alterna Capital Solutions LLC receives full payment. The upfront payments are treated as a short-term liability, labeled as due to factor on our consolidated balance sheets. The short-term liability will be released upon infusion of the drug product. As of December 31, 2023, the balance in the due to factor account was $2.5 million.

During December of 2023, the Company sold Purchased Accounts on the Purchase Dates to Alterna Capital Solutions LLC. The Purchased Assets are not available to our creditors to satisfy any of our obligations.

Selling, general and administrative expenses

Selling, general and administrative expenses are expensed as incurred. Selling, general and administrative expenses consist primarily of salaries and related costs for personnel, including stock-based compensation and travel expenses for the Company's 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.

The Company expenses the costs of advertising, which relates to advertising agency expenses, as incurred. Advertising expenses for the years ended December 31, 2023 and 2022 were immaterial.

Research and development expenses

Research and development costs are charged to expense as costs are incurred in performing research and development activities, including salaries and benefits, facilities costs, overhead costs, clinical study and related clinical manufacturing costs, CMO and CTO costs, contract research organization ("CRO") costs, license and milestone fees, contract services, manufacturing costs for pre-launch inventory that did not qualify for capitalization, and other related costs. Up-front fees and milestones paid to third parties in connection with technologies which have not reached technological feasibility and do not have an alternative future use are expensed as research and development expense as incurred. In circumstances where amounts have been paid in excess of costs incurred, the Company records a prepaid expense. The Company accrues costs for clinical trial activities based upon estimates of the services received and related expenses incurred that have yet to be invoiced by the contract research organizations, clinical study sites, laboratories, consultants, or other clinical trial vendors that perform the activities.

Cost of product revenue

All costs directly related to the manufacturing and delivery of the product to our qualified treatment centers are included in cost of product revenue which are associated with the sale of ZYNTEGLO and SKYSONA in the United States. Reserves for excess inventories are also included in cost of product revenue.

Stock-based compensation

The Company's share-based compensation programs grant awards that have included stock options, restricted stock units, restricted stock awards, unrestricted stock awards and shares issued under its employee stock purchase plan. Grants are awarded to employees and non-employees, including the Company's board of directors.

The Company accounts for its stock-based compensation awards in accordance with FASB ASC Topic 718, *Compensation —Stock Compensation* ("ASC 718"). ASC 718 requires all stock-based payments, including grants of stock options and restricted stock units and modifications to existing stock options, to be recognized in the consolidated statements of operations and comprehensive loss based on their fair values.

The Company's stock-based awards are subject to either service or performance-based vesting conditions. Compensation expense related to awards with service-based vesting conditions is recognized on a straight-line basis based on the grant date fair value over the associated service period of the award, which is generally the vesting term. Compensation expense related to awards with performance-based vesting conditions is recognized based on the grant date fair value over the requisite service period using the accelerated attribution method to the extent achievement of the performance condition is probable.

The Company estimates the fair value of its option awards using the Black-Scholes option pricing model, which requires the input of subjective assumptions, including (i) the expected stock price volatility, (ii) the calculation of expected term of the award, (iii) the risk-free interest rate, and (iv) expected dividends. Effective January 1, 2020, the Company eliminated the use of a representative peer group and uses only its own historical volatility data in its estimate of expected volatility given that there is now a sufficient amount of historical information regarding the volatility of its own stock price. For both employee and non-employee awards, the measurement date is the date of grant. The Company has estimated the expected term of its employee stock options using the "simplified" method, whereby, the expected term equals the arithmetic average of the vesting term and the original contractual term of the option due to its lack of sufficient historical data. The risk-free interest rates for periods within the expected term of the option are based on the U.S. Treasury securities with a maturity date commensurate with the expected term of the associated award. The Company has never paid, and does not expect to pay, dividends in the foreseeable future.

The Company accounts for forfeitures as they occur. Stock-based compensation expense recognized in the financial statements is based on awards for which performance or service conditions are expected to be satisfied.

Conversion and modification of equity awards outstanding at date of separation of 2seventy bio

In connection with the separation of 2seventy bio on November 4, 2021 (the "Separation"), under the provisions of the existing plans, the Company adjusted its outstanding equity awards in accordance with the terms of the Employee Matters Agreement (an equitable adjustment) to preserve the intrinsic value of the awards immediately before and after the Separation. These modified awards otherwise retained substantially the same terms and conditions, including term and vesting provisions. The Company will recognize future expense for awards denominated in bluebird stock and 2seventy bio stock granted to the Company's employees as a result of the separation of 2seventy bio. Expense related to awards denominated in bluebird stock granted to 2seventy bio employees will be incurred by 2seventy bio.

Stock-based compensation expense related to modified awards is measured based on the fair value for the awards as of the modification date. Any incremental stock-based compensation expense arising from the excess of the fair value of the awards on the modification date compared to the fair value of the awards immediately before the modification date is recognized at the modification date or ratably over the requisite remaining service period, as appropriate.

Interest income

Interest income consists primarily of interest income earned on investments.

Interest expense

Interest expense consists primarily of the interest expense on finance lease arrangements.

Other income, net

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

Net loss per share

Basic net loss per share is calculated by dividing net loss attributable to common stockholders by the weighted average number of common shares outstanding during the period, including the shares of common stock issuable upon the exercise of warrants that are exercisable for little or no consideration. Diluted net loss per share is calculated by dividing the net loss attributable to common stockholders by the weighted-average number of common equivalent shares outstanding for the period, including the shares of common stock issuable upon the exercise of warrants that are exercisable for little or no consideration as well as any dilutive effect from outstanding stock options, unvested restricted stock, restricted stock units, and employee stock purchase plan stock using the treasury stock method. Given that the Company recorded a net loss for each of the periods

presented, there is no difference between basic and diluted net loss per share since the effect of common stock equivalents would be anti-dilutive and are, therefore, excluded from the diluted net loss per share calculation.

The Company follows the two-class method when computing net loss per share in periods when issued shares that meet the definition of participating securities are outstanding. The two-class method determines net loss per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to common stockholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed. Accordingly, in periods in which the Company reports a net loss attributable to common stockholders when participating securities are outstanding, losses are not allocated to the participating securities.

Income taxes

Income taxes are recorded in accordance with FASB ASC Topic 740, *Income Taxes* ("ASC 740"), which provides for deferred taxes using an asset and liability approach. The Company recognizes deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Valuation allowances are provided, if based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. The Company accrues for potential interest and penalties related to unrecognized tax benefits in income tax expense.

Comprehensive loss

Comprehensive loss is composed of net loss and other comprehensive income (loss). Other comprehensive income (loss) consists of unrealized gains and losses on debt securities, foreign currency translation adjustments and other items.

Restructuring expenses

The Company records costs and liabilities associated with exit and disposal activities in accordance with FASB ASC Topic 420, *Exit or Disposal Cost Obligations* ("ASC 420") and Topic 712, *Compensation - Nonretirement Postemployment Benefits* ("ASC 712"). Such costs are based on estimates of fair value in the period liabilities are incurred. The Company evaluates and adjusts these costs as appropriate for changes in circumstances as additional information becomes available. Refer to Note 19, *Reduction in workforce,* for more information.

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.

4. Product revenue and reserves

For the years ended December 31, 2023 and 2022, the Company recorded $29.1 million and $2.7 million, respectively, of product revenue. Product revenue by therapy represents:

	For the year ended December 31,	
	2023	2022
ZYNTEGLO	$ 16,692	$ 2,739
SKYSONA	12,373	—
Total product revenue	$ 29,065	$ 2,739

Two individual customers accounted for 49% and 35% of product revenue for the year ended December 31, 2023, and one individual customer accounted for 100% of product revenue for the year ended December 31, 2022.

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 with 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 2023 and 2022 were within the United States and Europe, respectively.

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, 2022	$ —
Provision for rebates	5,365
Payments/credits	—
Balance at December 31, 2023	$ 5,365

5. Marketable securities

The following table summarizes the marketable securities held at December 31, 2023 and 2022 (in thousands):

	Amortized cost / cost	Unrealized gains	Unrealized losses	Fair value
December 31, 2023				
U.S. government agency securities and treasuries	$ —	$ —	$ —	$ —
Total	$ —	$ —	$ —	$ —
December 31, 2022				
U.S. government agency securities and treasuries	$ 67,970	$ —	$ (1,733)	$ 66,237
Corporate bonds	2,524	—	(26)	2,498
Total	$ 70,494	$ —	$ (1,759)	$ 68,735

No available-for-sale debt securities held as of December 31, 2022 had remaining maturities greater than five years.

6. 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 December 31, 2023 and 2022 (in thousands):

	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
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	—	—	560
Total assets	$ 222,315	$ 221,755	$ —	$ 560
December 31, 2022				
Assets:				
Cash and cash equivalents	$ 113,006	$ 113,006	$ —	$ —
Marketable securities:				
U.S. government agency securities and treasuries	66,237	—	66,237	—
Corporate bonds	2,498	—	2,498	—
Total assets	$ 181,741	$ 113,006	$ 68,735	$ —

Cash and cash equivalents

As of December 31, 2023, cash and cash equivalents comprise funds in cash, money market accounts, and commercial paper.

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 value 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. At December 31, 2022, the balance in the Company's accumulated other comprehensive loss was composed primarily of activity related to the Company's available-for-sale debt securities. There were $0.1 million, and no material realized losses recognized on the sale or maturity of available-for-sale securities during the years ended December 31, 2023 and 2022, respectively.

Accrued interest receivable on the Company's available-for-sale debt securities totaled $0.3 million and $0.1 million as of December 31, 2023 and 2022, respectively. No accrued interest receivable was written off during the twelve months ended December 31, 2023 or 2022.

The following table summarizes available-for-sale debt securities in a continuous unrealized loss position for less than and greater than twelve months, and for which an allowance for credit losses has not been recorded at December 31, 2023 and 2022 (in thousands):

Description	Less than 12 months		12 months or greater		Total	
	Fair value	Unrealized losses	Fair value	Unrealized losses	Fair value	Unrealized losses
December 31, 2023						
U.S. government agency securities and treasuries	$ —	$ —	$ —	$ —	$ —	$ —
Total	$ —	$ —	$ —	$ —	$ —	$ —
December 31, 2022						
U.S. government agency securities and treasuries	$ —	$ —	$ 66,237	$ (1,733)	$ 66,237	$ (1,733)
Corporate bonds	—	—	2,498	(26)	2,498	(26)
Total	$ —	$ —	$ 68,735	$ (1,759)	$ 68,735	$ (1,759)

The Company determined that there was no material change in the credit risk of the above investments during the twelve months ended December 31, 2023. As such, an allowance for credit losses was not recognized.

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 December 31, 2023, 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 three months ended December 31, 2023:

	For the year ended December 31,
	2023
Level 3 financial assets, beginning of period	$ —
Beneficiary interest obtained in transferred invoices	560
Proceeds from previously transferred invoices	—
Level 3 financial assets, end of period	$ 560

7. Inventory

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

	As of December 31,	
	2023	2022
Raw materials	$ 2,329	$ —
Work in progress	17,375	—
Finished goods	3,215	—
Inventory	$ 22,919	$ —

Raw materials inventory consists of completed materials purchased directly from third party suppliers. Work in progress inventory consists of materials manufactured at 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.

As of December 31, 2022, the Company did not have any manufactured inventory that was in progress or had received final quality acceptance after the FDA approval of ZYNTEGLO, SKYSONA, or LYFGENIA. Prior to FDA approval, the existing inventory was expensed to research and development expense.

8. Property, plant and equipment, net

Property, plant and equipment, net, consists of the following (in thousands):

	As of December 31,	
	2023	2022
		(As Restated)
Finance lease right of use assets	$ 84,524	$ 71,179
Laboratory equipment	14,636	21,048
Office equipment	4,767	4,323
Computer equipment and software	2,005	1,597
Leasehold improvements	1,251	—
Construction-in-progress	563	—
Total property, plant and equipment	107,746	98,147
Less accumulated depreciation and amortization	(41,810)	(30,511)
Property, plant and equipment, net	$ 65,936	$ 67,636

Depreciation and amortization expense related to property, plant and equipment was $28.0 million and $8.2 million for the years ended December 31, 2023 and 2022, respectively.

9. Restricted cash

As of December 31, 2023 and 2022, the Company maintained letters of credit of $51.2 million and $43.9 million, respectively, which are collateralized with bank accounts at financial institutions in accordance with the agreements. Total restricted cash as of December 31, 2023 and 2022 consisted of the following (in thousands):

	As of December 31,	
	2023	2022
50 Binney Street lease	$ 40,072	$ 40,072
Assembly Row lease	2,753	2,753
Embedded lease	7,632	—
Other	2,385	2,614
Total restricted cash	$ 52,842	$ 45,439

Refer to Note 11, *Leases,* for further information on the Company's letters of credit. The Company presents the current portion of restricted cash in receivables and other current assets.

10. Accrued expenses and other current liabilities

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

	As of December 31,	
	2023	2022
		(As Restated)
Accrued CMO and CRO costs	$ 24,824	$ 17,369
Accrued employee compensation	19,972	20,095
Accrued goods and services	8,391	7,635
Accrued rebates	5,365	—
Accrued refund liability	5,600	—
Other	9,036	8,365
Total accrued expenses and other current liabilities	$ 73,188	$ 53,464

11. Leases

The Company leases certain office and laboratory space. Additionally, the Company entered into CMO and CTO agreements which have been determined to contain an embedded lease.

Operating lease commitments

60 Binney Street lease & sublease

In October 2021, the Company entered into a consent to assignment and amendment to its lease agreement for its 60 Binney Street lease (the "Assignment"). The Assignment transfers the Company's interest in the lease to 2seventy bio and releases the Company from its obligation to maintain the $13.8 million collateralized letter of credit required under the original 60 Binney Street lease. Although the lease was legally transferred to 2seventy bio, the lessor required that the Company remain secondarily liable as a guarantor for payment obligations accruing under the 60 Binney Street lease from the date of Assignment until the later of (i) the date that the Company has completely vacated the premises and (ii) the date that 2seventy bio has reached a market capitalization of $900 million. Upon Separation, the lease assignment was accounted for as the termination of the original lease and the Company de-recognized the right-of-use asset and lease liability related to the 60 Binney Street lease and recognized the fair value of the guarantee pursuant to ASC 405, *Liabilities*. The fair value of the guarantee was not material.

Concurrent with the Assignment of the 60 Binney Street lease, the Company entered into a sublease agreement with 2seventy bio for office, laboratory and storage space located at 60 Binney Street (the "60 Binney Street Sublease") while it constructs and outfits its new office and laboratory space. The lease was modified in July 2022 to reflect the decreased use of the office and lab space. Under the terms of the 60 Binney Street Sublease, the Company leased 72,988 square feet for $1.0 million per month in base rent for the period beginning from the Assignment through March 2022 and 58,004 square feet for $0.8 million per month in base rent for the period from April 2022 through June 2022. Under the terms of the modification, beginning in July 2022, the Company is required to pay $0.6 million monthly until no later than December 2023. The Company also pays monthly fees for use of the facilities and support personnel, calculated based on its pro-rata share of operating costs during the term of the 60 Binney Street Sublease. The Company accounted for the 60 Binney Street Sublease as a new lease under ASC 842, and in November 2021, recognized a right-of-use asset and lease liability related to the 60 Binney Street Sublease. The Company terminated the 60 Binney Street Sublease in August 2023 and derecognized the right-of-use asset and lease liability.

50 Binney Street sublease & sub-sublease

In April 2019, the Company entered into a sublease agreement for office space located at 50 Binney Street in Cambridge, Massachusetts (the “50 Binney Street Sublease”) to supplement the Company’s then-current corporate headquarters located at 60 Binney Street in Cambridge, Massachusetts. Under the terms of the 50 Binney Street Sublease, the Company leases 267,278 square feet of office space for $99.95 per square foot, or $26.7 million per year in base rent subject to certain operating expenses, taxes and annual rent increases of approximately 3%. The lease commenced in April 2022 and lease payments began in July 2022. The lease term will end on December 31, 2030, unless other specific circumstances specified in the 50 Binney Street Sublease occur. Upon signing the 50 Binney Street Sublease, the Company executed a $40.1 million cash-collateralized letter of credit, which may be reduced in the future subject to the terms of the 50 Binney Street Sublease and certain reduction

requirements specified therein. The $40.1 million of cash collateralizing the letter of credit is classified as restricted cash and other non-current assets on the Company's consolidated balance sheets.

In December 2021, the Company entered into a sub-sublease agreement (the "Sub-Sublease") with Meta Platforms, Inc. ("Meta"). Under the terms of the Sub-Sublease, the Company is subleasing the entirety of the 50 Binney Street premises which it has rights to under the 50 Binney Street Sublease. The Company is sub-subleasing the premises for $29.4 million in the first year (inclusive of parking costs) with 3% annual increases in each subsequent year. Meta received access to 50 Binney Street at the lease commencement date, which is the same point that the Company received access under the 50 Binney Street Sublease. The Company remains liable under the 50 Binney Street Sublease, including for the maintenance of the $40.1 million collateralized letter of credit. The Company recognizes monthly sublease income of $2.6 million in other income, net for the sub-subleased space.

Assembly Row lease

In November 2021, the Company entered into a lease agreement with Assembly Row 5B, LLC ("Landlord") for office space located at 455 Grand Union Boulevard in Somerville, Massachusetts to serve as the Company's future corporate headquarters. Under the terms of the arrangement, the Company leases approximately 61,180 square feet starting at an annual rate of $45 per square foot, subject to annual increases of 2.5%, plus operating expenses and taxes. In addition, the Company is eligible to receive a tenant work allowance of $160 per rentable square foot of the premises. The lease commenced on March 1, 2022, the date on which the Landlord tendered possession of the premises to the Company with any tenant work required to be performed by the Landlord substantially completed. Since the lease commencement date, the Company has recognized rent expense of $0.3 million monthly.

Hood Park lease

In October 2022, the Company entered into a sublease with Finch Therapeutics, Inc. ("Finch") for office and laboratory space at Finch's corporate headquarters located at 100 Hood Park Drive, Charlestown, Massachusetts. Under the terms of the arrangement, the Company leases 42,261 square feet for $55 per square foot, subject to annual increases of 3.0%, plus operating expenses and taxes. This sublease commenced December 15, 2022, the date on which the landlord tendered possession of the premises to the Company and is expected to terminate on December 14, 2025. Since the lease commencement date, the Company has recognized substantially all of the monthly $0.2 million costs as rent expense.

Finance lease commitments - Embedded leases

The Company has six embedded CMO and CTO arrangements. The details are noted below.

Drug Product Manufacturing

Embedded Lease No. 1:

In June 2016, the Company entered into a manufacturing agreement for the future commercial production of the Company's ZYNTEGLO and SKYSONA drug products with a CMO. Under this 12-year agreement, the CMO will complete the design, construction, validation and process validation of the leased suites prior to anticipated commercial launch of the product candidates. From 2016 through March 2018, while the suites were under construction, the Company paid a total of $12.0 million in contractual milestone payments. Construction was completed in March 2018, and beginning in April 2018, the Company paid $5.1 million per year, in fixed suite reservation fees as well as certain fixed labor, raw materials, testing and shipping costs for manufacturing services. The Company may terminate this agreement at any time upon payment of a one-time termination fee and up to 24 months of slot fees and 12 months of labor fees. The Company concluded that this agreement contains an embedded lease as the suites are designated for the Company's exclusive use during the agreement's term, that it was not the deemed owner during construction, and the lease was not a capital lease under ASC 840-10, Leases - Overall. As a result, the Company initially accounted for the agreement as an operating lease under ASC 840 and recognized straight-line rent expense over the non-cancellable term of the embedded lease. As part of the Company's adoption of ASC 842, effective January 1, 2019, the Company carried forward the existing lease classification under ASC 840. The lease was modified and reclassified as a finance lease as of March 2019. In September 2023, the agreement was amended to change the fee structure in the arrangement from a fixed suite reservation fee to a fixed slot manufacturing fee. In addition, separate from the suite and existing equipment, the Company also has a forward starting embedded equipment lease that has not yet commenced. As the CMO is required to provide operational equipment throughout the contract term, the forward starting lease was established to account for the equipment that the CMO is obligated to lease to the Company in the future once the current equipment reaches its useful life and the lease expires. This forward starting lease is expected to commence in 2025 with an initial lease term of

three years and has fixed commitments of approximately $54.5 million. The Company has prepaid approximately $3.7 million, which is accounted for as prepaid asset on the Company's balance sheet that would adjust the right-of-use asset when the forward starting embedded equipment lease commences in 2025.

Embedded Lease No. 2:

In November 2016, the Company entered into an agreement for clinical and commercial production of the Company's ZYNTEGLO, SKYSONA, and LYFGENIA drug products with a CMO at an existing facility. The Company concluded that this agreement contains an embedded operating lease as the clean rooms are designated for the Company's exclusive use during the agreement's term. The term of the agreement is five years with subsequent three-year renewals at the mutual option of each party. The Company recognized a right-of-use asset and lease liability and were recognizing rent expense on a straight-line basis throughout the estimated remaining term of the embedded lease upon effective date of ASC 842. In January 2019, the Company amended this agreement along with the execution of new work orders and additional contracts for the delivery of non-lease services from the CMO, resulting in a lease modification under ASC 842. Under the terms of the amended agreement, the Company is required to pay annual maintenance and production fees of up to €16.5 million, depending on its production needs, and may terminate this agreement with twelve months' notice and a one-time termination fee. The amendment also provides for an option to reserve an additional clean room for a one-time option fee and annual maintenance fees. In connection with the lease modification, in April 2019, the Company reconsidered the lease classification and accounted for this embedded lease as a finance lease under ASC 842. In September 2021, the Company reassessed the term of this lease due to the planned orderly wind down of its operations in Europe, resulting in a reduction to the right-of-use asset and related lease liability to reflect the shortened expected term of the agreement. In November 2021, the Company exercised its right to terminate the lease agreement, effective in November 2022 and was required to pay a one-time termination fee of €1.0 million upon termination. That termination payment was made in December 2021. The Company also paid for services rendered through the date of termination based on work completed and expenses incurred prior to the date of termination.

Embedded Lease No. 3:

During July 2020, the Company amended an existing CMO arrangement to reserve additional manufacturing capacity for LYFGENIA. The Company concluded that this amendment contains an embedded lease as a controlled environment room at the facility is designated for the Company's exclusive use during the term of the agreement, with the option to sublease the space if the Company provides notice that it will not utilize it for a specified duration of time. Under the amended agreement, the Company is required to pay up to $5.4 million per year in maintenance fees in addition to the cost of any services provided and may terminate this agreement with eighteen months' notice. The term of the agreement is five years with the option to extend. when the additional manufacturing capacity became available that served as lease commencement in March 2021, the Company classified the embedded lease as a finance lease.

In February 2021, the Company amended the CMO arrangement to reserve additional manufacturing capacity. The Company concluded that this amended agreement contains an embedded lease as a controlled environment room at the facility is designated for the Company's exclusive use during the agreement's term. Under the amended agreement, the Company must pay $4.2 million per year in maintenance fees and per-batch fees for the cost of any services provided. Either party may terminate this agreement with eighteen months' notice at any time or with eight months' notice after defined milestones in the agreement have been met. The term of the agreement is five years, with the option to extend. The lease commenced in November 2021 when the suites became available, and upon commencement the Company classified the embedded lease as a finance lease. The lease has been subsequently modified based on changes in the delivery of non-lease component services, which have been combined with lease components under our accounting policy.

In August 2022, the Company amended another arrangement with this CMO to reserve additional controlled environmental rooms. The Company concluded that this amended agreement contains an embedded finance lease as the controlled environment room at the facility is designated for the Company's exclusive use during the agreement's term. Under the amended agreement, an existing deposit of $10.8 million was applied towards the monthly lease payments through September 2023. The term of the agreement was 14 months with an option to extend. The Company exercised the option to extend through December 31, 2023.

Drug Substance Manufacturing

Embedded Lease No. 4:

In November 2017, the Company entered a commercial manufacturing services agreement with a CMO to establish commercial production of the Company's suspension vector. The Company concluded this agreement contained an embedded

finance lease when production commenced in the new facility in 2019 as the Company has dedicated suite space and reserved production capacity at a rate that allows the Company to take more than substantially all the capacity of certain manufacturing space and equipment within the facility. Under the agreement, the Company must pay capacity reservation fees, minimum purchase commitment fees, and milestone fees for achievement of certain activities. In addition, the Company prepaid approximately €13.5 million between 2018 and 2019 towards certain milestone payments and in exchange for production credits. As of December 31, 2021, those credits have been fully used by the Company. The Company modified the lease to extend its term in October 2022, April 2023, and finally in October 2023, which extended the lease term to end in March 2024 in addition to changes in the delivery of non-lease component services, which have been combined with lease components under our accounting policy.

Embedded Lease No. 5:

In January 2018, the Company entered a clinical and commercial supply agreement with a CMO to manufacture ZYNTEGLO and SKYSONA vector products. The Company concluded this agreement contained an embedded finance lease. The Company and the CMO originally agreed to wind down manufacturing activities in December 2021. In December 2022, the Company reestablished this manufacturing agreement and concluded the revised arrangement contains an embedded finance lease as the Company is using the entire capacity of a manufacturing suite at the facility. The term of the agreement is three years and requires the Company to pay suite reservation fees of $13.5 million in 2023 and $18.0 million per year in 2024 and 2025, in addition to the cost of any services provided.

Quality Testing

Embedded Lease No. 6:

In 2018, the Company entered a 2-year master contract services agreement (MCSA) with a CTO to provide clinical development services (other than manufacturing services). The original MCSA expired in June 2020 but was reinstated through December 2024 under the amendment of the MCSA effective April 2021. The Company concluded this agreement contained an embedded finance lease as the Company had certain lab suites implicitly dedicated to it for various clinical testing procedures. The Company is required to pay the fixed price outlined in each of the various service contracts covering quality, stability, and other services. The lease has been subsequently modified based on changes in the delivery of non-lease component services, which have been combined with lease components under our accounting policy.

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 years ended December 31, 2023 and 2022 (in thousands):

	For the year ended December 31,	
	2023	2022
		(As Restated)
Finance leases		
Interest expense	$ 16,350	$ 6,324
Amortization expense	24,653	3,886
Total fixed finance lease cost	$ 41,003	$ 10,210
Operating leases		
Fixed lease cost	43,272	36,122
Total fixed operating lease cost	43,272	36,122
Variable lease cost	22,960	28,161
Short-term lease cost	238	143
Total lease cost	$ 107,473	$ 74,636
Operating sublease income	$ 41,165	$ 29,369
Cash paid in the measurement of lease liabilities		
Operating cash flows used for operating leases	$ 28,887	$ 14,072
Operating cash flows used for finance leases	12,924	1,551
Financing cash flows for finance leases	54,367	36,734
Other information:		
Weighted average remaining lease term - finance leases (in years)	1.6 years	2.4 years
Weighted average discount rate - finance leases	13.93 %	12.52 %
Weighted average remaining lease term - operating leases (in years)	7.0 years	7.8 years
Weighted average discount rate - operating leases	7.01 %	7.05 %

For finance leases embedded in CMO arrangements, interest expense is recognized using the effective interest method, applying the Company's incremental borrowing rate as required by ASC 842, and amortization expense is recognized on a straight-line basis over the shorter of the life of the asset or the term of the lease. Rather than amortizing the finance lease right-of-use assets that did not have alternative future use, those amounts were recognized as research and development expense at lease commencement or lease modification and were $21.2 million and $11.2 million for the years ended December 31, 2023 and 2022, respectively.

As of December 31, 2023, future minimum commitments under ASC 842 under the Company's leases were as follows (in thousands):

	Operating Leases	Financing Leases
Remaining Lease Payments		
2024	34,918	93,531
2025	37,084	34,995
2026	35,719	2,519
2027	36,742	2,519
2028	37,795	1,031
2029 and thereafter	81,986	—
Total	264,244	134,595
Less: Imputed Interest	(56,355)	(12,158)
Present Value of Lease Liabilities	$ 207,889	$ 122,437

12. Commitments and contingencies

Lease commitments

The Company leases certain office and laboratory space and has embedded leases at CMOs and a CTO. As of December 31, 2023, the Company has commitments arising from a forward starting lease that has 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 $54.5 million. The following table presents the non-cancelable contractual obligations arising from this arrangement (in thousands):

Years ended December 31,	Future commitment
2024	$ —
2025	11,959
2026	17,655
2027	17,655
2028	7,225
2029 and thereafter	—
Total purchase commitments	$ 54,494

Refer to Note 11, *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 described below as a loss is not probable and a loss, or a range of loss, is not reasonably estimable.

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 the Company 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 the Company's 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.

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 the Company's 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. Parties must submit a scheduling order to the Court by September 23, 2024.

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 the Company's 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. Parties must submit a scheduling order to the Court by September 23, 2024.

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.

13. Equity

The Company is authorized to issue 250.0 million shares of common stock. Holders of common stock are entitled to one vote per share. Holders of common stock are entitled to receive dividends, if and when declared by the Company's board of directors, and to share ratably in the Company's assets legally available for distribution to the Company's shareholders in the event of liquidation. Holders of common stock have no preemptive, subscription, redemption or conversion rights. As of December 31, 2023 and 2022, the Company had 192.8 million and 82.9 million shares of common stock issued and outstanding, respectively.

In September 2021, the Company entered into an equity purchase agreement with certain investors, pursuant to which the Company agreed to sell and issue, in a private placement offering of securities, an aggregate of (i) 2.3 million shares of the Company's common stock at a purchase price per share of $16.50 and (ii) pre-funded warrants to purchase up to 2.3 million shares of common stock (the "Pre-Funded Warrants") at an effective price of $16.49 per share ($16.49 paid to the Company upon the closing of the offering and $0.01 to be paid upon exercise of such Pre-Funded Warrants). This resulted in aggregate gross proceeds to the Company of approximately $75.0 million. In October 2023, the investors opted to exercise their warrant. Pursuant to the terms of the warrant agreement, the company issued 2.3 million shares of common stock to the investor. As of December 31, 2023, there are no outstanding Pre-Funded Warrants.

In June 2022, the Company entered into the Equity Distribution Agreement with Goldman to sell shares of the Company's common stock, with aggregate gross sales proceeds of up to $75.0 million, from time to time, through an "at the market" equity offering program under which Goldman will act as manager. The Company terminated the Equity Distribution Agreement in August 2023.

For the year ended December 31, 2022, the Company sold 10.7 million shares of common stock under the Equity Distribution Agreement for gross proceeds of $56.2 million ($54.2 million net of offering costs).

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 option 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 December 31, 2023, 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.

The Company is authorized to issue 5.0 million shares of preferred stock in one or more series and to fix the powers, designations, preferences and relative participating option or other rights thereof, including dividend rights, conversion rights, voting rights, redemption terms, liquidation preferences and the number of shares constituting any series, without any further vote or action by the Company's shareholders. As of December 31, 2023 and 2022, the Company had no shares of preferred stock issued or outstanding.

14. Sale of Priority Review Vouchers

On November 29, 2022, the Company entered into an asset purchase agreement with argenx BV ("argenx"), pursuant to which the Company agreed to sell a Rare Pediatric Disease Priority Review Voucher ("PRV") to argenx. The Company was awarded the voucher under an FDA program intended to encourage the development of certain rare pediatric disease product applications. The Company received the PRV when SKYSONA received accelerated approval by the FDA for the treatment of early, active CALD. Pursuant to the agreement, argenx agreed to pay the Company $102.0 million, payable in cash, upon the closing of the sale. The Company received a cash payment of $102.0 million upon closing on December 29, 2022, and there were no transaction costs associated with the sale.

On January 5, 2023, the Company entered into an asset purchase agreement with Bristol-Myers Squibb Company ("BMS"), pursuant to which the Company agreed to sell a PRV to BMS. The Company was awarded the voucher under the FDA program described above. The Company received the PRV when ZYNTEGLO was approved by the FDA for the treatment of ß-thalassemia in adult and pediatric patients who require regular red blood cell transfusions. Pursuant to the agreement, BMS agreed to pay the Company $95.0 million, payable in cash, upon the closing of the sale, which occurred

simultaneously with the parties entering into the agreement. The Company received cash of $95.0 million and recognized $2.1 million in transaction costs.

15. Intangible assets

Intangible assets, net of accumulated amortization, are summarized as follows (in thousands):

	As of December 31, 2023			
	Cost	Accumulated amortization	Impairment	Net
In-licensed rights	11,089	(651)	—	10,438
Total	$ 11,089	$ (651)	$ —	$ 10,438

	As of December 31, 2022			
	Cost	Accumulated amortization	Impairment	Net
In-licensed rights	5,000	(132)	—	4,868
Total	$ 5,000	$ (132)	$ —	$ 4,868

Amortization expense for intangible assets was $0.5 million and $0.1 million for the years ended December 31, 2023 and 2022, respectively.

In-licensed rights

In-licensed rights consist of capitalized milestone payments made to third parties upon receiving regulatory approval of ZYNTEGLO, SKYSONA, and LYFGENIA in the U.S. The in-licensed rights are being amortized on a straight-line basis over the remaining life of the product exclusivity period in the U.S. of approximately twelve years per product, as the life of the product exclusivity reflects the expected time period that the Company will benefit from the in-licensed rights.

The following table summarizes the estimated future amortization for intangible assets for the next five years and thereafter (in thousands):

	As of December 31, 2023
2024	$ 926
2025	926
2026	926
2027	926
2028	926
2029 and thereafter	$ 5,808
Total	$ 10,438

16. Stock-based compensation

2023 Incentive Award Plan

In June 2023, the Company's stockholders approved the bluebird bio, Inc. 2023 Incentive Award Plan ("2023 Plan"), which became effective on June 16, 2023. The 2023 Plan replaced the 2013 Stock Option and Incentive Plan ("2013 Plan").

The 2023 Plan allows for the granting of incentive stock options, non-qualified stock options, restricted stock units, and restricted stock awards to the Company's employees, members of the board of directors, and consultants of the Company. The 2023 Plan initially authorized the issuance of up to 5.2 million shares of common stock.

Any awards outstanding under the Company's 2013 Plan at the time of adoption of the 2023 Plan remained outstanding and effective. The shares of common stock underlying any awards that are forfeited, canceled, repurchased, expired or are

otherwise terminated (other than by exercise) under the 2013 Plan are added to the shares of common stock available for issuance under the 2023 Plan. The 2023 Plan will expire in 2033. As of December 31, 2023, the total number of shares of common stock that may be issued under the 2023 Plan is 5.1 million.

2021 Inducement Plan

On May 18, 2021, the Company's board of directors adopted the bluebird bio, Inc. 2021 Inducement Plan (the "Inducement Plan") pursuant to the Nasdaq Stock Market LLC listing rules ("Rule 5635(c)(4)"). In accordance with Rule 5635(c)(4), equity-based incentive awards under the Inducement Plan may only be made to a newly hired employee who has not previously been a member of the Company's board of directors, or an employee who is being rehired following a bona fide period of non-employment by the Company as a material inducement to the employee's entering into employment with the Company. The Inducement Plan initially reserved 600,000 shares, which was later increased to an aggregate of 1,250,000 shares in January 2022.

The exercise price of stock options granted under the Inducement Plan will not be less than the fair market value of a share of the Company's common stock on the grant date. Other terms of awards, including vesting requirements, are determined by the Company's board of directors or compensation committee and are subject to the provisions of the Inducement Plan. Stock options granted to employees under the Inducement Plan generally vest over a four-year period but may be granted with different vesting terms. Certain options may provide for accelerated vesting in the event of a change in control. Stock options granted under the Inducement Plan expire no more than 10 years from the date of grant. As of December 31, 2023, 1.1 million shares of common stock are available for future grant under the Inducement Plan.

2013 Stock Option and Incentive Plan

In June 2013, the Company's board of directors adopted the 2013 Plan, which was subsequently approved by the Company's stockholders and became effective upon the closing of the Company's IPO. The 2013 Plan expired in June 2023.

The 2013 Plan allowed for the granting of incentive stock options, non-qualified stock options, restricted stock units and restricted stock awards to the Company's employees, members of the board of directors, and consultants of the Company. The Company initially reserved approximately 1.0 million shares of its common stock for the issuance of awards under the 2013 Plan. The 2013 Plan provided that the number of shares reserved and available for issuance would automatically increase each January 1, beginning on January 1, 2014, by four percent of the outstanding number of shares of common stock on the immediately preceding December 31 or such lesser number of shares as determined by the Company's compensation committee. In January 2023 and January 2022, the number of common stock available for issuance under the 2013 Plan was increased by approximately 2.8 million and 3.3 million shares, respectively, as a result of this automatic increase provision.

Conversion and modification of equity awards outstanding at the Separation

In connection with the Separation on November 4, 2021, under the provisions of the existing plans, the Company adjusted its outstanding equity awards in accordance with the terms of the Employee Matters Agreement (an equitable adjustment) to preserve the intrinsic value of the awards immediately before and after the distribution of all of the outstanding shares of 2seventy bio common stock to our stockholders. Upon the distribution, employees holding stock options, restricted stock units ("RSUs") and performance restricted stock units ("PRSUs") denominated in pre-distribution bluebird stock received a number of otherwise-similar awards either in post-Distribution bluebird stock or in a combination of post-distribution bluebird stock and 2seventy bio stock based on conversion ratios outlined for each group of employees in the Employee Matters Agreement that the Company entered into in connection with the distribution. The equity awards that were granted prior to 2021 were converted under the shareholder method, wherein employees holding outstanding equity awards received equity awards in both bluebird and 2seventy bio. The conversion ratio for the shareholder method took into consideration a distribution ratio of one share of 2seventy bio common stock for every three shares of bluebird common stock. For equity awards granted in 2021, the number of awards that were outstanding at the Separation were proportionately adjusted to maintain the aggregate intrinsic value of the awards at the date of the Separation. The conversion ratio was determined based on the volume weighted-average trading price for bluebird common stock for the five trading days before and after the Separation. These modified awards otherwise retained substantially the same terms and conditions, including term and vesting provisions.

Additionally, bluebird will not incur any future compensation cost related to equity awards held by 2seventy bio employees and directors. The Company will incur future compensation cost related to 2seventy bio equity awards held by bluebird employees.

Stock-based compensation expense

The Company recognized stock-based compensation expense totaling $19.4 million and $35.1 million during the years ended December 31, 2023 and 2022, respectively. Stock-based compensation expense recognized by award type is as follows (in thousands):

	Year ended December 31,	
	2023	2022
		(As Restated)
Stock options	$ 6,478	$ 14,168
Restricted stock units	12,619	20,138
Employee stock purchase plan and other	332	784
	$ 19,429	$ 35,090

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

	Year ended December 31,	
	2023	2022
		(As Restated)
Cost of product revenue	$ 881	$ —
Selling, general and administrative	9,548	15,831
Research and development	9,000	19,259
	$ 19,429	$ 35,090

During the years ended December 31, 2023 and 2022, the Company had $1.7 million and $0.3 million of stock-based compensation expense that was capitalized into inventory, respectively.

As of December 31, 2023, the Company had $8.7 million and $18.8 million of unrecognized compensation expense related to unvested stock options and restricted stock units (exclusive of those with both service and performance conditions that have not yet been achieved), respectively, that is expected to be recognized over a weighted-average period of 1.96 years and 2.92 years, respectively.

Stock options

The fair value of each option issued to employees was estimated at the date of grant using the Black-Scholes option pricing model with the following weighted-average assumptions:

	Year ended December 31,	
	2023	2022
Expected volatility	70.9 %	66.4 %
Expected term (in years)	6.10	6.0
Risk-free interest rate	4.1 %	1.9 %
Expected dividend yield	0.0 %	0.0 %

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

	Shares (in thousands)	Weighted-average exercise price per share	Weighted-average contractual life (in years)	Aggregate intrinsic value (a) (in thousands)
Outstanding at December 31, 2022	2,668	$ 24.38		
Granted	2,077	$ 4.89		
Exercised	(3)	$ 2.74		
Canceled or forfeited	(515)	$ 29.55		
Outstanding at December 31, 2023	4,227	$ 14.16	8.06	$ —
Exercisable at December 31, 2023	1,469	$ 28.80	6.54	$ —
Vested and expected to vest at December 31, 2023	4,227	$ 14.16	8.06	$ —

(a) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the fair value of the common stock for the options that were in the money at December 31, 2023.

The weighted-average fair values of options granted during the years ended December 31, 2023 and 2022 was $3.24 and $4.46, respectively. There was no intrinsic value of options exercised during the years ended December 31, 2023 and 2022, respectively.

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 balance at December 31, 2022	2,415	$ 11.44
Granted	3,369	4.76
Vested	(1,057)	14.35
Forfeited	(520)	8.09
Unvested balance at December 31, 2023	4,207	$ 6.08

The intrinsic value of restricted stock units, including shares held by employees of 2seventy bio, vested during the years ended December 31, 2023 and 2022 was $5.4 million and $6.0 million, respectively. The total grant date fair value of restricted stock units vested during the years ended December 31, 2023 and December 31, 2022 was $15.2 million and $14.0 million, respectively.

Employee Stock Purchase Plan

In June 2013, the Company's board of directors adopted its 2013 Employee Stock Purchase Plan ("2013 ESPP"), which was subsequently approved by its stockholders and became effective upon the closing of the Company's IPO. The 2013 ESPP authorizes 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 approximately 1.4 million shares of the Company's common stock available to participating employees. During each of the years ended December 31, 2023 and 2022, approximately 0.1 million shares of common stock were issued under the 2013 ESPP.

17. 401(k) Savings plan

In 1997, the Company established a defined-contribution savings plan under Section 401(k) of the Internal Revenue Code (the "401(k) Plan"). The 401(k) Plan covers all employees who meet defined minimum age and service requirements, and allows participants to defer a portion of their annual compensation on a pretax basis. Expense related to the 401(k) Plan from continuing operations totaled $3.1 million and $2.7 million for the years ended December 31, 2023 and 2022, respectively.

18. Income taxes

The components of loss before income taxes were as follows (in thousands):

	Year ended December 31,	
	2023	2022
		(As Restated)
U.S.	$ (213,306)	$ (230,160)
Foreign	1,267	(65)
Total	$ (212,039)	$ (230,225)

The provision for (benefit from) income taxes were as follows (in thousands):

	Year ended December 31,	
	2023	2022
		(As Restated)
Current:		
Federal	$ —	$ —
State	—	—
Foreign	(126)	117
Deferred:		
Federal	—	—
State	—	—
Foreign	—	—
Total income tax expense (benefit)	$ (126)	$ 117

A reconciliation of income tax expense (benefit) computed at the statutory federal income tax rate to the Company's effective income tax rate as reflected in the financial statements is as follows:

	Year ended December 31,	
	2023	2022
		(As Restated)
Federal income tax expense at statutory rate	21.0 %	21.0 %
State income tax, net of federal benefit	7.3 %	5.2 %
Permanent differences	0.6 %	(0.1)%
Stock-based compensation	(3.6)%	(9.3)%
Research and development credit	5.8 %	8.3 %
Leases	5.4 %	34.4 %
Right-of-use assets	— %	(26.1)%
Fixed assets	(2.7)%	(7.2)%
Foreign differential	— %	0.3 %
Other	0.9 %	(0.6)%
Change in valuation allowance	(34.6)%	(26.0)%
Effective income tax rate (expense) benefit	0.1 %	(0.1)%

For the years ended December 31, 2023 and 2022, the Company recognized an income tax expense (benefit) of $(0.1) million or 0.1% and $0.1 million or (0.1)%, respectively. The Company did not recognize any significant tax expense for the years ended December 31, 2023 or 2022 as the Company was subject to a full valuation allowance. Deferred taxes are recognized for temporary differences between the basis of assets and liabilities for financial statement and income tax purposes.

The significant components of the Company's deferred tax assets and liabilities are composed of the following (in thousands):

	Year ended December 31,	
	2023	2022
		(As Restated)
Deferred tax assets:		
U.S. net operating loss carryforwards (federal and state)	$ 726,021	$ 713,557
Tax credit carryforwards (federal and state)	313,963	300,236
Capitalized license fees and research and development expenses	2,079	1,831
Capitalized research and development expenses under Section 174	91,306	54,885
Stock-based compensation	9,847	11,686
Lease liabilities	100,288	98,296
Accruals and other	20,336	17,325
Total deferred tax assets	1,263,840	1,197,816
Right-of-use assets	(54,976)	(61,388)
Fixed assets	(16,701)	(17,536)
Less valuation allowance	(1,192,163)	(1,118,892)
Net deferred taxes	$ —	$ —

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 valuation allowance increased on a net basis by approximately $73.3 million during the year ended December 31, 2023 due primarily to the capitalization of research and development credits that became effective in the 2022 tax year under the Tax Cuts and Jobs Act, net operating losses, and tax credit carryforwards. Effective January 1, 2021, the Company adopted ASU 2019-12, which simplifies accounting for income taxes. There was no material impact on the Company's financial position or results of operations upon adoption.

As of December 31, 2023 and 2022, the Company had U.S. federal net operating loss carryforwards of approximately $2.7 billion and $2.7 billion, respectively, which may be available to offset future income tax liabilities. Of the amount as of December 31, 2023, $2.0 billion will carryforward indefinitely while $0.7 billion will expire at various dates through 2037. As of December 31, 2023 and 2022, the Company also had U.S. state net operating loss carryforwards of approximately $2.5 billion and $2.5 billion, respectively, which may be available to offset future income tax liabilities and expire at various dates through 2043.

As of December 31, 2023 and 2022, the Company had federal research and development and orphan drug tax credit carryforwards of approximately $299.5 million and $285.6 million, respectively, available to reduce future tax liabilities which expire at various dates through 2043. As of December 31, 2023 and 2022, the Company had state credit carryforwards of approximately $18.3 million and $18.5 million, respectively, available to reduce future tax liabilities which expire at various dates through 2037. During the fourth quarter of 2018, the Company completed an analysis of prior year estimates of U.S. research and development and orphan drug tax credits for the years 2013 through 2017. The analysis resulted in an immaterial adjustment to the Company's income tax benefit, which was offset by an adjustment to the valuation allowance. An analysis of the U.S. research and development and orphan drug credits has not yet been completed for years 2018 through 2023. As of December 31, 2023 and 2022, the Company had capital loss carryforwards of approximately $4.2 million and $4.2 million, respectively, which may be available to offset future capital gains and will begin to expire in 2027.

In March 2021, the American Rescue Plan Act ("ARPA") was enacted and contained extenders to the refundable employee retention credit and provided further limitations to executive compensation effective for tax years beginning after 2026. In August 2022, the Inflation Reduction Act ("IRA") was enacted and introduced a 15% corporate alternative minimum tax ("CAMT") for corporations with average annual adjusted financial statement income for any three-year tax period ending after December 31, 2021 and preceding tax year exceeding $1 billion, effective for tax years beginning after December 31, 2021, as well as a 1% excise tax on stock repurchases made by public companies, climate and energy provisions, and extensions to the Affordable Care Act subsidies. The Company has concluded that the provisions in the ARPA and IRA have an immaterial impact on the Company's income tax expense due to its cumulative losses and full valuation allowance position.

Under the provisions of the Internal Revenue Code, the net operating loss and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. Net operating loss and tax credit carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50 percent, as defined under Sections 382 and 383 of the Internal Revenue Code, respectively, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of the Company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. The Company has completed several financings since its inception and prior to its initial public offering in 2013, which it believes has resulted in a change in control as defined by Sections 382 and 383 of the Internal Revenue Code. The Company completed a study through December 2023 confirming no ownership changes have occurred since the Company's initial public offering in 2013; any ownership shifts occurring after December 2023 could result in an ownership change under Section 382. There is a significant likelihood that the Company may experience an ownership change as a result of future equity offerings, including any sales under the ATM program, although whether the Company experiences an ownership change will depend on the specific facts that apply at the time of any offering.

The Company files Federal income tax returns in the United States, and various state and foreign jurisdictions. The federal, state and foreign income tax returns are generally subject to tax examinations for the tax years ended December 31, 2020 through December 31, 2022. To the extent the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service, or state or foreign tax authorities to the extent utilized in a future period.

A reconciliation of the beginning and ending amount of unrecognized tax benefits is as follows (in thousands):

	Unrecognized tax benefits
Balance as of December 31, 2021 **(As Restated)**	$ 21,956
Increases (decreases) for tax positions related to current period	1,582
Increases (decreases) for tax positions related to prior periods	(45)
Balance as of December 31, 2022 **(As Restated)**	23,493
Increases (decreases) for tax positions related to current period	944
Increases (decreases) for tax positions related to prior periods	101
Balance as of December 31, 2023	24,538

The unrecognized tax benefits at December 31, 2023, if recognized, would not affect the Company’s effective tax rate due to its full valuation allowance position. The Company does not anticipate that the amount of existing unrecognized tax benefits will significantly increase or decrease within the next 12 months. The Company has elected to include interest and penalties related to uncertain tax positions as a component of its provision for income taxes. For the years ended December 31, 2023 and 2022, the Company’s accrued interest and penalties related to uncertain tax positions were not material.

19. Reduction in workforce

In April 2022, the Board of Directors of the Company approved a comprehensive restructuring plan intended to reduce operating expenses. As part of the restructuring, the Company reduced its workforce by approximately 30% across the second and third quarters of 2022. The Company incurred approximately $4.9 million in costs to implement the restructuring, comprised primarily of severance payments and continuing health care coverage over the severance period. The restructuring actions associated with these charges commenced in April 2022, and were completed by September 30, 2022.

The following tables summarizes the accrued liabilities activity recorded in connection with the reduction in workforce for the year ended December 31, 2022 (in thousands):

	Restructuring Expenses in 2022	Amounts paid in 2022
April 2022 reduction	4,940	(4,940)
Total	4,940	(4,940)

The Company recorded approximately $4.9 million in restructuring expenses as of December 31, 2022. There were no restructuring expenses recorded as of December 31, 2023.

20. 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):

	Year ended December 31,	
	2023	2022
Outstanding stock options [(1)]	5,860	4,429
Restricted stock units [(1)]	4,239	2,524
Warrants	12,450	—
ESPP shares and other	90	63
	22,639	7,016

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

Net loss per share for the years ended December 31, 2023 and 2022 were $1.93 and $2.93, respectively.

21. Quarterly Financial Information (Unaudited)

As further described in Note 2, the previously reported unaudited condensed consolidated financial information for the three months ended March 31, 2023 and 2022, the three and six months ended June 30, 2023 and 2022 and the three and nine months ended September 30, 2023 and 2022 are required to be restated. The as-restated interim financial information for each relevant period is included in the tables that follow. As part of the restatement, the Company recorded adjustments to correct the misstatements in the impacted interim periods. Descriptions of the lease entries which drove the restatement and other immaterial entries also recorded can be found in Note 2. For the interim periods in the nine months ended September 30, 2023, following the commercialization of ZYNTEGLO and SKYSONA, the embedded lease adjustments for certain CMOs and the CTO also impact inventory and cost of product revenue. The unaudited condensed consolidated financial information reflect all adjustments which are, in the opinion of management, necessary for a fair statement of the results for the interim periods presented. Restated amounts are computed independently each quarter; therefore, the sum of the quarterly amounts may not equal the total amount for the respective year due to rounding.

Condensed Consolidated Balance Sheets

The following unaudited condensed consolidated balance sheet tables present the impacts of the restatement adjustments as of the periods ended March 31, 2023 and 2022, June 30, 2023 and 2022, and September 30, 2023 and 2022. The period ended December 31, 2023 was not subject to restatement.

(in thousands)	As of March 31, 2023			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Assets				
Current assets:				
Cash and cash equivalents	$ 239,045	$ —	$ —	$ 239,045
Marketable securities	79,212	—	—	79,212
Prepaid expenses	13,466	(2,082)	—	11,384
Inventory	3,809	(610)	—	3,199
Receivables and other current assets	15,539	3,374	(2,800)	16,113
Total current assets	351,071	682	(2,800)	348,953
Property, plant and equipment, net	8,718	53,223	(270)	61,671
Goodwill	5,646	—	—	5,646
Intangible assets, net	5,613	—	—	5,613
Operating lease right-of-use assets	270,153	(46,690)	—	223,463
Restricted cash and other non-current assets	51,535	3,665	—	55,200
Total assets	$ 692,736	$ 10,880	$ (3,070)	$ 700,546
Liabilities and Stockholders' Equity				
Current Liabilities:				
Accounts payable	$ 19,235	$ (2,522)	$ —	$ 16,713
Accrued expenses and other current liabilities	45,294	1,017	(801)	45,510
Operating lease liability, current portion	51,404	(24,784)		26,620
Financing lease liability, current portion	—	58,747	—	58,747
Total current liabilities	115,933	32,458	(801)	147,590
Operating lease liability, net of current portion	221,971	(18,176)		203,795
Financing lease liability, net of current portion	—	58,649	—	58,649
Other non-current liabilities	92	—	—	92
Total liabilities	337,996	72,931	(801)	410,126
Commitments and contingencies (Note 12)				
Stockholders' Equity:				
Preferred stock, $0.01 par value, 5,000 shares authorized; 0 shares issued and outstanding at March 31, 2023	$ —	$ —	$ —	$ —
Common stock, $0.01 par value, 125,000 shares authorized; 106,370 shares issued and outstanding at March 31, 2023	1,064	—	—	1,064
Additional paid-in capital	4,322,025	—	(98)	4,321,927
Accumulated other comprehensive loss	(3,086)	—	—	(3,086)
Accumulated deficit	(3,965,263)	(62,051)	(2,171)	(4,029,485)
Total stockholders' equity	354,740	(62,051)	(2,269)	290,420
Total liabilities and stockholders' equity	$ 692,736	$ 10,880	$ (3,070)	$ 700,546

(in thousands)	As of June 30, 2023			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Assets				
Current assets:				
Cash and cash equivalents	$ 172,872	$ —	$ —	$ 172,872
Marketable securities	72,431	—	—	72,431
Prepaid expenses	13,597	(2,162)	—	11,435
Inventory	13,642	(1,838)	602	12,406
Receivables and other current assets	15,435	3,659		19,094
Total current assets	287,977	(341)	602	288,238
Property, plant and equipment, net	10,227	51,722	(235)	61,714
Goodwill	5,646	—	—	5,646
Intangible assets, net	5,490	—	—	5,490
Operating lease right-of-use assets	302,849	(86,929)		215,920
Restricted cash and other non-current assets	51,204	3,665	—	54,869
Total assets	$ 663,393	$ (31,883)	$ 367	$ 631,877
Liabilities and Stockholders' Equity				
Current Liabilities:				
Accounts payable	$ 10,894	$ (1,081)	$ —	$ 9,813
Accrued expenses and other current liabilities	56,531	(599)	1,999	57,931
Operating lease liability, current portion	67,591	(42,427)	—	25,164
Financing lease liability, current portion	—	55,706	—	55,706
Total current liabilities	135,016	11,599	1,999	148,614
Operating lease liability, net of current portion	239,266	(40,930)		198,336
Financing lease liability, net of current portion	—	50,017	—	50,017
Other non-current liabilities	92	—	—	92
Total liabilities	374,374	20,686	1,999	397,059
Commitments and contingencies (Note 12)				
Stockholders' Equity:				
Preferred stock, $0.01 par value, 5,000 shares authorized; 0 shares issued and outstanding at June 30, 2023	$ —	$ —	$ —	$ —
Common stock, $0.01 par value, 250,000 shares authorized; 106,454 shares issued and outstanding at June 30, 2023	1,065	—	—	1,065
Additional paid-in capital	4,328,489	—	(98)	4,328,391
Accumulated other comprehensive loss	(2,364)	—	—	(2,364)
Accumulated deficit	(4,038,171)	(52,569)	(1,534)	(4,092,274)
Total stockholders' equity	289,019	(52,569)	(1,632)	234,818
Total liabilities and stockholders' equity	$ 663,393	$ (31,883)	$ 367	$ 631,877

(in thousands)	As of September 30, 2023			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Assets				
Current assets:				
Cash and cash equivalents	$ 165,347	$ —	$ —	$ 165,347
Marketable securities	8,946	—	—	8,946
Accounts Receivable	23,000	—	(8,400)	14,600
Prepaid expenses	11,431	(2,040)	—	9,391
Inventory	20,969	(3,205)	602	18,366
Other current assets	17,383	3,538		20,921
Total current assets	247,076	(1,707)	(7,798)	237,571
Property, plant and equipment, net	9,972	63,398	(280)	73,090
Goodwill	5,646	—	—	5,646
Intangible assets, net	5,368	—	—	5,368
Operating lease right-of-use assets	294,717	(87,796)		206,921
Restricted cash and other non-current assets	50,829	3,665	—	54,494
Total assets	$ 613,608	$ (22,440)	$ (8,078)	$ 583,090
Liabilities and Stockholders' Equity				
Current Liabilities:				
Accounts payable	$ 19,852	$ (3,042)	$ 316	$ 17,126
Deferred revenue	9,653	—	(8,400)	1,253
Accrued expenses and other current liabilities	57,768	572	—	58,340
Operating lease liability, current portion	71,684	(46,160)	—	25,524
Financing lease liability, current portion	—	85,208	—	85,208
Total current liabilities	158,957	36,578	(8,084)	187,451
Operating lease liability, net of current portion	232,023	(39,456)	—	192,567
Financing lease liability, net of current portion	—	50,146	—	50,146
Other non-current liabilities	92	—	—	92
Total liabilities	391,072	47,268	(8,084)	430,256
Commitments and contingencies (Note 12)				
Stockholders' Equity:				
Preferred stock, $0.01 par value, 5,000 shares authorized; 0 shares issued and outstanding at September 30, 2023	$ —	$ —	$ —	$ —
Common stock, $0.01 par value, 250,000 shares authorized; 107,022 shares issued and outstanding at September 30, 2023	1,071	—	—	1,071
Additional paid-in capital	4,333,594	—	(98)	4,333,496
Accumulated other comprehensive loss	(2,227)	—	—	(2,227)
Accumulated deficit	(4,109,902)	(69,708)	104	(4,179,506)
Total stockholders' equity	222,536	(69,708)	6	152,834
Total liabilities and stockholders' equity	$ 613,608	$ (22,440)	$ (8,078)	$ 583,090

(in thousands)	As of March 31, 2022			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Assets				
Current assets:				
Cash and cash equivalents	$ 106,260	$ —	$ —	$ 106,260
Marketable securities	105,328	—	—	105,328
Prepaid expenses	31,798	(1,549)	—	30,249
Receivables and other current assets	11,531	198	—	11,729
Total current assets	254,917	(1,351)	—	253,566
Marketable securities	55,049	—	—	55,049
Property, plant and equipment, net	11,234	(599)	(459)	10,176
Goodwill	5,646	—	—	5,646
Operating lease right-of-use assets	111,897	(61,082)	—	50,815
Restricted cash and other non-current assets	52,328	3,665	—	55,993
Total assets	$ 491,071	$ (59,367)	$ (459)	$ 431,245
Liabilities and Stockholders' Equity				
Current Liabilities:				
Accounts payable	$ 28,350	$ (4,937)	$ —	$ 23,413
Accrued expenses and other current liabilities	89,032	(15,597)	1,999	75,434
Operating lease liability, current portion	25,510	(9,262)	—	16,248
Financing lease liability, current portion	—	55,995	—	55,995
Total current liabilities	142,892	26,199	1,999	171,090
Operating lease liability, net of current portion	84,828	(46,407)	—	38,421
Financing lease liability, net of current portion	—	41,315	—	41,315
Other non-current liabilities	92	—	—	92
Total liabilities	227,812	21,107	1,999	250,918
Commitments and contingencies (Note 12)				
Stockholders' Equity:				
Preferred stock, $0.01 par value, 5,000 shares authorized; 0 shares issued and outstanding at March 31, 2022	$ —	$ —	$ —	$ —
Common stock, $0.01 par value, 125,000 shares authorized; 71,438 shares issued and outstanding at March 31, 2022	714	—	—	714
Additional paid-in capital	4,109,081	—	(1,384)	4,107,697
Accumulated other comprehensive loss	(4,459)	—	—	(4,459)
Accumulated deficit	(3,842,077)	(80,474)	(1,074)	(3,923,625)
Total stockholders' equity	263,259	(80,474)	(2,458)	180,327
Total liabilities and stockholders' equity	$ 491,071	$ (59,367)	$ (459)	$ 431,245

(in thousands)	As of June 30, 2022			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Assets				
Current assets:				
Cash and cash equivalents	$ 81,499	$ —	$ —	$ 81,499
Marketable securities	51,010	—	—	51,010
Prepaid expenses	24,473	(667)	—	23,806
Receivables and other current assets	10,476	2,997	—	13,473
Total current assets	167,458	2,330	—	169,788
Marketable securities	40,641	—	—	40,641
Property, plant and equipment, net	14,566	(688)	(406)	13,472
Goodwill	5,646	—	—	5,646
Operating lease right-of-use assets	292,731	(54,005)	—	238,726
Restricted cash and other non-current assets	52,550	3,665	—	56,215
Total assets	$ 573,592	$ (48,698)	$ (406)	$ 524,488
Liabilities and Stockholders' Equity				
Current Liabilities:				
Accounts payable	$ 24,865	$ (2,846)	$ —	$ 22,019
Accrued expenses and other current liabilities	75,550	(12,189)	1,999	65,360
Operating lease liability, current portion	48,446	(22,583)	—	25,863
Financing lease liability, current portion	—	47,219	—	47,219
Total current liabilities	148,861	9,601	1,999	160,461
Operating lease liability, net of current portion	244,522	(27,634)	—	216,888
Financing lease liability, net of current portion	—	36,550	—	36,550
Other non-current liabilities	93	—	—	93
Total liabilities	393,476	18,517	1,999	413,992
Commitments and contingencies (Note 12)				
Stockholders' Equity:				
Preferred stock, $0.01 par value, 5,000 shares authorized; 0 shares issued and outstanding at June 30, 2022	$ —	$ —	$ —	$ —
Common stock, $0.01 par value, 125,000 shares authorized; 73,551 shares issued and outstanding at June 30, 2022	735	—	—	735
Additional paid-in capital	4,126,012	—	(1,854)	4,124,158
Accumulated other comprehensive loss	(4,416)	—	—	(4,416)
Accumulated deficit	(3,942,215)	(67,215)	(551)	(4,009,981)
Total stockholders' equity	180,116	(67,215)	(2,405)	110,496
Total liabilities and stockholders' equity	$ 573,592	$ (48,698)	$ (406)	$ 524,488

(in thousands)	As of September 30, 2022			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Assets				
Current assets:				
Cash and cash equivalents	$ 66,478	$ —	$ —	$ 66,478
Marketable securities	73,155	—	—	73,155
Prepaid expenses	8,270	55	—	8,325
Receivables and other current assets	12,535	2,425	—	14,960
Total current assets	160,438	2,480	—	162,918
Marketable securities	1,407	—	—	1,407
Property, plant and equipment, net	11,535	(777)	(358)	10,400
Goodwill	5,646	—	—	5,646
Intangible assets, net	—	—	4,972	4,972
Operating lease right-of-use assets	288,684	(56,750)	—	231,934
Restricted cash and other non-current assets	52,388	3,665	—	56,053
Total assets	$ 520,098	$ (51,382)	$ 4,614	$ 473,330
Liabilities and Stockholders' Equity				
Current Liabilities:				
Accounts payable	$ 18,622	$ (2,898)	$ —	$ 15,724
Accrued expenses and other current liabilities	64,314	(13,432)	1,998	52,880
Operating lease liability, current portion	43,791	(20,322)	—	23,469
Financing lease liability, current portion	—	41,264	—	41,264
Total current liabilities	126,727	4,612	1,998	133,337
Operating lease liability, net of current portion	234,422	(24,069)	—	210,353
Financing lease liability, net of current portion	—	31,036	—	31,036
Other non-current liabilities	92	—	—	92
Total liabilities	361,241	11,579	1,998	374,818
Commitments and contingencies (Note 12)				
Stockholders' Equity:				
Preferred stock, $0.01 par value, 5,000 shares authorized; 0 shares issued and outstanding at September 30, 2022	$ —	$ —	$ —	$ —
Common stock, $0.01 par value, 125,000 shares authorized; 82,880 shares issued and outstanding at September 30, 2022	829	—	—	829
Additional paid-in capital	4,181,393	—	(2,043)	4,179,350
Accumulated other comprehensive loss	(4,630)	—	—	(4,630)
Accumulated deficit	(4,018,735)	(62,961)	4,659	(4,077,037)
Total stockholders' equity	158,857	(62,961)	2,616	98,512
Total liabilities and stockholders' equity	$ 520,098	$ (51,382)	$ 4,614	$ 473,330

Condensed Consolidated Statements of Operations and Comprehensive Loss

The following unaudited condensed consolidated statements of operations and comprehensive loss tables present the impacts of the restatement adjustments for the three months ended March 31, 2023 and 2022, the three and six months ended June 30, 2023 and 2022, and the three and nine months ended September 30, 2023 and 2022. The year ended December 31, 2023 was not subject to restatement.

	Three Months ended March 31, 2023			
(in thousands, except per share amounts)	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Revenue				
Product revenue	$ 2,296	$ —	$ —	$ 2,296
Other revenue	85	—	—	85
Total revenues	2,381	—	—	2,381
Cost of product revenue	3,376	2,136	—	5,512
Gross margin	(995)	(2,136)	—	(3,131)
Operating expenses:				
Selling, general and administrative	37,354	113	—	37,467
Research and development	46,144	(4,516)	(41)	41,587
Total operating expenses	83,498	(4,403)	(41)	79,054
Gain from sale of priority review voucher, net	92,930	—	—	92,930
Income (loss) from operations	8,437	2,267	41	10,745
Interest income	2,828	—	—	2,828
Interest expense	(3)	(4,267)	—	(4,270)
Other income, net	9,978	(351)	—	9,627
Income (loss) before income taxes	21,240	(2,351)	41	18,930
Income tax (expense) benefit	—	—	—	—
Net income (loss)	21,240	(2,351)	41	18,930
Net income (loss) per share - basic (1)	$ 0.21	$ (0.02)	$ —	$ 0.18
Net income (loss) per share - diluted (1)	$ 0.21	$ (0.02)	$ —	$ 0.18
Weighted-average number of common shares used in computing net income (loss) per share - basic:	102,920	—	—	102,920
Weighted-average number of common shares used in computing net income (loss) per share - diluted:	103,303	—	—	103,303
Other comprehensive income (loss):				
Other comprehensive income (loss), net of tax (benefit) expense of $0.0 million for the three months ended March 31, 2023	984	—	—	984
Total other comprehensive income (loss)	984	—	—	984
Comprehensive income (loss)	$ 22,224	$ (2,351)	$ 41	$ 19,914

(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.

(in thousands, except per share amounts)	Six Months ended June 30, 2023			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Revenue				
Product revenue	$ 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:				
Selling, general and administrative	77,703	226	—	77,929
Research and development	88,418	(14,705)	(678)	73,035
Total operating expenses	166,121	(14,479)	(678)	150,964
Gain from sale of priority review voucher, net	92,930	—	—	92,930
Income (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
Income (loss) before income taxes	(51,748)	7,131	678	(43,939)
Income tax (expense) benefit	80		—	80
Net income (loss)	(51,668)	7,131	678	(43,859)
Net income (loss) per share - basic	$ (0.49)	$ 0.07	$ 0.01	$ (0.41)
Net income (loss) per share - diluted	$ (0.49)	$ 0.07	$ 0.01	$ (0.41)
Weighted-average number of common shares used in computing net income (loss) per share - basic:	105,819	—	—	105,819
Weighted-average number of common shares used in computing net income (loss) per share - diluted:	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 income (loss)	$ (49,962)	$ 7,131	$ 678	$ (42,153)

(in thousands, except per share amounts)	Three Months ended June 30, 2023			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Revenue				
Product revenue	$ 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:				—
Selling, general and administrative	40,349	113	—	40,462
Research and development	42,274	(10,189)	(637)	31,448
Total operating expenses	82,623	(10,076)	(637)	71,910
Income (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
Income (loss) before income taxes	(72,988)	9,482	637	(62,869)
Income tax (expense) benefit	80	—	—	80
Net income (loss)	(72,908)	9,482	637	(62,789)
Net income (loss) per share - basic (1)	$ (0.67)	$ 0.09	$ 0.01	$ (0.58)
Net income (loss) per share - diluted (1)	$ (0.67)	$ 0.09	$ 0.01	$ (0.58)
Weighted-average number of common shares used in computing net income (loss) per share - basic:	108,685	—	—	108,685
Weighted-average number of common shares used in computing net income (loss) per share - diluted:	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 income (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.

(in thousands, except per share amounts)	Nine Months ended September 30, 2023			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Revenue				
Product revenue	$ 21,414	$ —	$ —	$ 21,414
Other revenue	249	—	—	249
Total revenues	21,663	—	—	21,663
Cost of product revenue	23,895	(2,560)	—	21,335
Gross margin	(2,232)	2,560	—	328
Operating expenses:				—
Selling, general and administrative	118,406	294	—	118,700
Research and development	133,881	52	(2,397)	131,536
Total operating expenses	252,287	346	(2,397)	250,236
Gain from sale of priority review voucher, net	92,930	—	—	92,930
Income (loss) from operations	(161,589)	2,214	2,397	(156,978)
Interest income	7,961	—	—	7,961
Interest expense	(3)	(12,328)	—	(12,331)
Other income (expense), net	30,152	106	(81)	30,177
Income (loss) before income taxes	(123,479)	(10,008)	2,316	(131,171)
Income tax (expense) benefit	80	—	—	80
Net income (loss)	(123,399)	(10,008)	2,316	(131,091)
Net income (loss) per share - basic (1)	$ (1.15)	$ (0.09)	$ 0.02	$ (1.23)
Net income (loss) per share - diluted (1)	$ (1.15)	$ (0.09)	$ 0.02	$ (1.23)
Weighted-average number of common shares used in computing net income (loss) per share - basic:	106,924	—	—	106,924
Weighted-average number of common shares used in computing net income (loss) per share - diluted:	106,924	—	—	106,924
Other comprehensive income (loss):				
Other comprehensive income (loss), net of tax (benefit) expense of $0.0 million for the nine months ended September 30, 2023	1,843	—	—	1,843
Total other comprehensive income (loss)	1,843	—	—	1,843
Comprehensive income (loss)	$ (121,556)	$ (10,008)	$ 2,316	$ (129,248)

(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.

(in thousands, except per share amounts)	Three Months ended September 30, 2023			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Revenue				
Product revenue	$ 12,281	$ —	$ —	$ 12,281
Other revenue	111	—	—	111
Total revenues	12,392	—	—	12,392
Cost of product revenue	10,955	(1,829)	—	9,126
Gross margin	1,437	1,829	—	3,266
Operating expenses:				
Selling, general and administrative	40,703	68	—	40,771
Research and development	45,463	14,757	(1,719)	58,501
Total operating expenses	86,166	14,825	(1,719)	99,272
Income (loss) from operations	(84,729)	(12,996)	1,719	(96,006)
Interest income	2,454	—	—	2,454
Interest expense	—	(4,311)	—	(4,311)
Other income (expense), net	10,544	168	(81)	10,631
Income (loss) before income taxes	(71,731)	(17,139)	1,638	(87,232)
Income tax (expense) benefit	—	—	—	—
Net income (loss)	(71,731)	(17,139)	1,638	(87,232)
Net income (loss) per share - basic	$ (0.66)	$ (0.16)	$ 0.02	$ (0.80)
Net income (loss) per share - diluted	$ (0.66)	$ (0.16)	$ 0.02	$ (0.80)
Weighted-average number of common shares used in computing net income (loss) per share - basic:	109,098	—	—	109,098
Weighted-average number of common shares used in computing net income (loss) per share - diluted:	109,098	—	—	109,098
Other comprehensive income (loss):				
Other comprehensive income (loss), net of tax (benefit) expense of $0.0 million for the three months ended September 30, 2023	137	—	—	137
Total other comprehensive income (loss)	137	—	—	137
Comprehensive income (loss)	$ (71,594)	$ (17,139)	$ 1,638	$ (87,095)

(in thousands, except per share amounts)	Three Months ended March 31, 2022			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Revenue:				
Product revenue	$ 1,408	$ —	$ —	$ 1,408
Other revenue	537	—	—	537
Total revenues	1,945	—	—	1,945
Cost of product revenue	8,310	—	—	8,310
Gross margin	(6,365)	—	—	(6,365)
Operating expenses:				
Research and development	77,875	(15,511)	(930)	61,434
Selling, general and administrative	36,106	(147)	(510)	35,449
Total operating expenses	113,981	(15,658)	(1,440)	96,883
Income (loss) from operations	(120,346)	15,658	1,440	(103,248)
Interest income	106	—	—	106
Interest expense	—	(1,028)	—	(1,028)
Other income (expense), net	(1,912)	530	—	(1,382)
Income (loss) before income taxes	(122,152)	15,160	1,440	(105,552)
Income tax (expense) benefit	—	—	—	—
Net income (loss)	(122,152)	15,160	1,440	(105,552)
Net income (loss) per share - basic	$ (1.66)	$ 0.21	$ 0.02	$ (1.43)
Net income (loss) per share - diluted	$ (1.66)	$ 0.21	$ 0.02	$ (1.43)
Weighted-average number of common shares used in computing net income (loss) per share - basic:	73,688	—	—	73,688
Weighted-average number of common shares used in computing net income (loss) per share - diluted:	73,688	—	—	73,688
Other comprehensive income (loss):				
Other comprehensive income (loss), net of tax (benefit) expense of $0.0 million for the three months ended March 31, 2022	(1,548)	—	—	(1,548)
Total other comprehensive income (loss)	(1,548)	—	—	(1,548)
Comprehensive income (loss)	$ (123,700)	$ 15,160	$ 1,440	$ (107,100)

(in thousands, except per share amounts)	Six Months ended June 30, 2022			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Revenue:				
Product revenue	$ 2,739	$ —	$ —	$ 2,739
Other revenue	725	—	—	725
Total revenues	3,464	—	—	3,464
Cost of product revenue	10,055	—	—	10,055
Gross margin	(6,591)	—	—	(6,591)
Operating expenses:				
Research and development	141,716	(27,866)	(1,192)	112,658
Selling, general and administrative	72,800	(461)	(771)	71,568
Restructuring expenses	6,639	—	—	6,639
Total operating expenses	221,155	(28,327)	(1,963)	190,865
Income (loss) from operations	(227,746)	28,327	1,963	(197,456)
Interest income	280	—	—	280
Interest expense	—	(1,950)	—	(1,950)
Other income, net	5,176	2,042	—	7,218
Income (loss) before income taxes	(222,290)	28,419	1,963	(191,908)
Income tax (expense) benefit	—	—	—	—
Net income (loss)	(222,290)	28,419	1,963	(191,908)
Net income (loss) per share - basic	$ (3.02)	$ 0.39	$ 0.03	$ (2.60)
Net income (loss) per share - diluted	$ (3.02)	$ 0.39	$ 0.03	$ (2.60)
Weighted-average number of common shares used in computing net income (loss) per share - basic:	73,727	—	—	73,727
Weighted-average number of common shares used in computing net income (loss) per share - diluted:	73,727	—	—	73,727
Other comprehensive income (loss):				
Other comprehensive income (loss), net of tax (benefit) expense of $0.0 million for the six months ended June 30, 2022	(1,505)	—	—	(1,505)
Total other comprehensive income (loss)	(1,505)	—	—	(1,505)
Comprehensive income (loss)	$ (223,795)	$ 28,419	$ 1,963	$ (193,413)

(in thousands, except per share amounts)	Three Months ended June 30, 2022			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Revenue:				
Product revenue	$ 1,331	$ —	$ —	$ 1,331
Other revenue	188	—	—	188
Total revenues	1,519	—	—	1,519
Cost of product revenue	1,745	—	—	1,745
Gross margin	(226)	—	—	(226)
Operating expenses:				
Research and development	63,841	(12,355)	(262)	51,224
Selling, general and administrative	36,694	(314)	(261)	36,119
Restructuring expenses	6,639	—	—	6,639
Total operating expenses	107,174	(12,669)	(523)	93,982
Income (loss) from operations	(107,400)	12,669	523	(94,208)
Interest income	174	—	—	174
Interest expense	—	(922)	—	(922)
Other income, net	7,088	1,512	—	8,600
Income (loss) before income taxes	(100,138)	13,259	523	(86,356)
Income tax (expense) benefit	—	—	—	—
Net income (loss)	(100,138)	13,259	523	(86,356)
Net income (loss) per share - basic	$ (1.36)	$ 0.18	$ 0.01	$ (1.17)
Net income (loss) per share - diluted	$ (1.36)	$ 0.18	$ 0.01	$ (1.17)
Weighted-average number of common shares used in computing net income (loss) per share - basic:	73,767	—	—	73,767
Weighted-average number of common shares used in computing net income (loss) per share - diluted:	73,767	—	—	73,767
Other comprehensive income (loss):				
Other comprehensive income (loss), net of tax (benefit) expense of $0.0 million for the three months ended June 30, 2022	43	—	—	43
Total other comprehensive income (loss)	43	—	—	43
Comprehensive income (loss)	$ (100,095)	$ 13,259	$ 523	$ (86,313)

(in thousands, except per share amounts)	Nine Months ended September 30, 2022			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Revenue:				
Product revenue	$ 2,739	$ —	$ —	$ 2,739
Other revenue	795	—	—	795
Total revenues	3,534	—	—	3,534
Cost of product revenue	10,056	—	—	10,056
Gross margin	(6,522)	—	—	(6,522)
Operating expenses:				
Research and development	194,864	(30,789)	(6,406)	157,669
Selling, general and administrative	106,201	(188)	(767)	105,246
Restructuring expenses	4,940	—	—	4,940
Total operating expenses	306,005	(30,977)	(7,173)	267,855
Income (loss) from operations	(312,527)	30,977	7,173	(274,377)
Interest income	663	—	—	663
Interest expense	—	(3,502)	—	(3,502)
Other income, net	13,061	5,198	—	18,259
Income (loss) before income taxes	(298,803)	32,673	7,173	(258,957)
Income tax (expense) benefit	(7)	—	—	(7)
Net income (loss)	(298,810)	32,673	7,173	(258,964)
Net income (loss) per share - basic	$ (3.91)	$ 0.43	$ 0.09	$ (3.39)
Net income (loss) per share - diluted	$ (3.91)	$ 0.43	$ 0.09	$ (3.39)
Weighted-average number of common shares used in computing net income (loss) per share - basic:	76,361	—	—	76,361
Weighted-average number of common shares used in computing net income (loss) per share - diluted:	76,361	—	—	76,361
Other comprehensive income (loss):				
Other comprehensive income (loss), net of tax (benefit) expense of $0.0 million for the nine months ended September 30, 2022	(1,719)	—	—	(1,719)
Total other comprehensive income (loss)	(1,719)	—	—	(1,719)
Comprehensive income (loss)	$ (300,529)	$ 32,673	$ 7,173	$ (260,683)

(in thousands, except per share amounts)	Three Months ended September 30, 2022			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Revenue:				
Product revenue	$ —	$ —	$ —	$ —
Other revenue	71	—	—	71
Total revenues	71	—	—	71
Cost of product revenue	—	—	—	—
Gross margin	71	—	—	71
Operating expenses:				
Research and development	53,149	(2,923)	(5,214)	45,012
Selling, general and administrative	33,402	273	4	33,679
Restructuring expenses	(1,699)	—	—	(1,699)
Total operating expenses	84,852	(2,650)	(5,210)	76,992
Income (loss) from operations	(84,781)	2,650	5,210	(76,921)
Interest income	383	—	—	383
Interest expense	—	(1,552)	—	(1,552)
Other income, net	7,885	3,156	—	11,041
Income (loss) before income taxes	(76,513)	4,254	5,210	(67,049)
Income tax (expense) benefit	(7)	—	—	(7)
Net income (loss)	(76,520)	4,254	5,210	(67,056)
Net income (loss) per share - basic (1)	$ (0.94)	$ 0.05	$ 0.06	$ (0.82)
Net income (loss) per share - diluted (1)	$ (0.94)	$ 0.05	$ 0.06	$ (0.82)
Weighted-average number of common shares used in computing net income (loss) per share - basic:	81,543	—	—	81,543
Weighted-average number of common shares used in computing net income (loss) per share - diluted:	81,543	—	—	81,543
Other comprehensive income (loss):				
Other comprehensive income (loss), net of tax (benefit) expense of $0.0 million for the three months ended September 30, 2022	(214)	—	—	(214)
Total other comprehensive income (loss)	(214)	—	—	(214)
Comprehensive income (loss)	$ (76,734)	$ 4,254	$ 5,210	$ (67,270)

(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.

Condensed Consolidated Statements of Stockholders' Equity

The following unaudited condensed consolidated statements of stockholders' equity tables present the impacts of the restatement adjustments for the three months ended March 31, 2023 and 2022, the three months ended June 30, 2023 and 2022, and the three months ended September 30, 2023 and 2022. The year ended December 31, 2023, was not subject to restatement.

(in thousands)	Common stock Shares	Common stock Amount	Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total stockholders' equity
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	23,000	230	130,061	—	—	130,291
Stock-based compensation expense	—	—	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 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	—	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—
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	—	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—
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	23,000	230	130,061	—	—	130,291
Stock-based compensation expense	—	—	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

(in thousands)	Common stock Shares	Common stock Amount	Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total stockholders' equity
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
Stock-based compensation expense	—	—	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 Leases						
Balances at March 31, 2023	—	—	—	—	(62,051)	(62,051)
Vesting of restricted stock	—	—	—	—	—	—
Exercise of stock options	—	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—
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	—	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—
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
Stock-based compensation expense	—	—	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

(in thousands)	Common stock Shares	Common stock Amount	Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total stockholders' equity
As previously reported						
Balances at June 30, 2023	106,454	$ 1,065	$ 4,328,489	$ (2,364)	$ (4,038,171)	$ 289,019
Vesting of restricted stock	566	6	(6)	—	—	—
Exercise of stock options	2	—	8	—	—	8
Issuance of common stock	—	—	(50)	—	—	(50)
Stock-based compensation expense	—	—	5,153	—	—	5,153
Other comprehensive income (loss)	—	—	—	137	—	137
Net income (loss)	—	—	—	—	(71,731)	(71,731)
Balances at September 30, 2023	107,022	$ 1,071	$ 4,333,594	$ (2,227)	$ (4,109,902)	$ 222,536
Adjustments to Leases						
Balances at June 30, 2023	—	—	—	—	(52,569)	(52,569)
Vesting of restricted stock	—	—	—	—	—	—
Exercise of stock options	—	—	—	—	—	—
Issuance of common stock	—	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—
Other comprehensive income (loss)	—	—	—	—	—	—
Net income (loss)	—	—	—	—	(17,139)	(17,139)
Balances at September 30, 2023	—	$ —	$ —	$ —	$ (69,708)	$ (69,708)
Other Adjustments						
Balances at June 30, 2023	—	—	(98)	—	(1,534)	(1,632)
Vesting of restricted stock	—	—	—	—	—	—
Exercise of stock options	—	—	—	—	—	—
Issuance of common stock	—	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—
Other comprehensive income (loss)	—	—	—	—	—	—
Net income (loss)	—	—	—	—	1,638	1,638
Balances at September 30, 2023	—	$ —	$ (98)	$ —	$ 104	$ 6
As Restated						
Balances at June 30, 2023	106,454	1,065	4,328,391	(2,364)	(4,092,274)	234,818
Vesting of restricted stock	566	6	(6)	—	—	—
Exercise of stock options	2	—	8	—	—	8
Issuance of common stock	—	—	(50)	—	—	(50)
Stock-based compensation expense	—	—	5,153	—	—	5,153
Other comprehensive income (loss)	—	—	—	137	—	137
Net income (loss)	—	—	—	—	(87,232)	(87,232)
Balances at September 30, 2023	107,022	$ 1,071	$ 4,333,496	$ (2,227)	$ (4,179,506)	$ 152,834

(in thousands)	Common stock Shares	Common stock Amount	Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total stockholders' equity
As Previously Reported						
Balances at December 31, 2021	71,115	$ 711	$ 4,096,402	$ (2,911)	$ (3,719,925)	$ 374,277
Vesting of restricted stock	310	3	(3)	—	—	—
Exercise of stock options	1	—	1	—	—	1
Issuance of unrestricted stock awards to settle accrued employee compensation	12	—	—	—	—	—
Stock-based compensation expense	—	—	12,681	—	—	12,681
Other comprehensive income (loss)	—	—	—	(1,548)	—	(1,548)
Net income (loss)	—	—	—	—	(122,152)	(122,152)
Balances at March 31, 2022	71,438	$ 714	$ 4,109,081	$ (4,459)	$ (3,842,077)	$ 263,259
Adjustments to Leases						
Balances at December 31, 2021	—	—	—	—	(95,634)	(95,634)
Vesting of restricted stock	—	—	—	—	—	—
Exercise of stock options	—	—	—	—	—	—
Issuance of unrestricted stock awards to settle accrued employee compensation	—	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—
Other comprehensive income (loss)	—	—	—	—	—	—
Net income (loss)	—	—	—	—	15,160	15,160
Balances at March 31, 2022	—	$ —	$ —	$ —	$ (80,474)	$ (80,474)
Other Adjustments						
Balances at December 31, 2021	—	—	—	—	(2,514)	(2,514)
Vesting of restricted stock	—	—	—	—	—	—
Exercise of stock options	—	—	—	—	—	—
Issuance of unrestricted stock awards to settle accrued employee compensation	—	—	—	—	—	—
Stock-based compensation expense	—	—	(1,384)	—	—	(1,384)
Other comprehensive income (loss)	—	—	—	—	—	—
Net income (loss)	—	—	—	—	1,440	1,440
Balances at March 31, 2022	—	$ —	$ (1,384)	$ —	$ (1,074)	$ (2,458)
As Restated						
Balances at December 31, 2021	71,115	711	4,096,402	(2,911)	(3,818,073)	276,129
Vesting of restricted stock	310	3	(3)	—	—	—
Exercise of stock options	1	—	1	—	—	1
Issuance of unrestricted stock awards to settle accrued employee compensation	12	—	—	—	—	—
Stock-based compensation expense	—	—	11,297	—	—	11,297
Other comprehensive income (loss)	—	—	—	(1,548)	—	(1,548)
Net income (loss)	—	—	—	—	(105,552)	(105,552)
Balances at March 31, 2022	71,438	$ 714	$ 4,107,697	$ (4,459)	$ (3,923,625)	$ 180,327

(in thousands)	Common stock Shares	Common stock Amount	Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total stockholders' equity
As Reported						
Balances at March 31, 2022	71,438	714	4,109,081	(4,459)	(3,842,077)	263,259
Vesting of restricted stock	60	1	(1)	—	—	—
Exercise of stock options	1	—	1	—	—	1
Issuance of common stock	2,052	20	8,023	—	—	8,043
Stock-based compensation expense	—	—	8,908	—	—	8,908
Other comprehensive income (loss)	—	—	—	43	—	43
Net income (loss)	—	—	—	—	(100,138)	(100,138)
Balances at June 30, 2022	73,551	$ 735	$ 4,126,012	$ (4,416)	$ (3,942,215)	$ 180,116
Adjustments to Leases						
Balances at March 31, 2022	—	—	—	—	(80,474)	(80,474)
Vesting of restricted stock	—	—	—	—	—	—
Exercise of stock options	—	—	—	—	—	—
Issuance of common stock	—	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—
Other comprehensive income (loss)	—	—	—	—	—	—
Net income (loss)	—	—	—	—	13,259	13,259
Balances at June 30, 2022	—	$ —	$ —	$ —	$ (67,215)	$ (67,215)
Other Adjustments						
Balances at March 31, 2022	—	—	(1,384)	—	(1,074)	(2,458)
Vesting of restricted stock	—	—	—	—	—	—
Exercise of stock options	—	—	—	—	—	—
Issuance of common stock	—	—	—	—	—	—
Stock-based compensation expense	—	—	(470)	—	—	(470)
Other comprehensive income (loss)	—	—	—	—	—	—
Net income (loss)	—	—	—	—	523	523
Balances at June 30, 2022	—	$ —	$ (1,854)	$ —	$ (551)	$ (2,405)
As Restated						
Balances at March 31, 2022	71,438	714	4,107,697	(4,459)	(3,923,625)	180,327
Vesting of restricted stock	60	1	(1)	—	—	—
Exercise of stock options	1	—	1	—	—	1
Issuance of common stock	2,052	20	8,023	—	—	8,043
Stock-based compensation expense	—	—	8,438	—	—	8,438
Other comprehensive income (loss)	—	—	—	43	—	43
Net income (loss)	—	—	—	—	(86,356)	(86,356)
Balances at June 30, 2022	73,551	$ 735	$ 4,124,158	$ (4,416)	$ (4,009,981)	$ 110,496

(in thousands)	Common stock Shares	Common stock Amount	Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total stockholders' equity
As Previously Reported						
Balances at June 30, 2022	73,551	735	4,126,012	(4,416)	(3,942,215)	180,116
Vesting of restricted stock	572	6	(6)	—	—	—
Purchase of shares under ESPP	67	1	238	—	—	239
Issuance of common stock	8,690	87	45,937	—	—	46,024
Stock-based compensation expense	—	—	9,212	—	—	9,212
Other comprehensive income (loss)	—	—	—	(214)	—	(214)
Net income (loss)	—	—	—	—	(76,520)	(76,520)
Balances at September 30, 2022	82,880	$ 829	$ 4,181,393	$ (4,630)	$ (4,018,735)	$ 158,857
Adjustments to Leases						
Balances at June 30, 2022	—	—	—	—	(67,215)	(67,215)
Vesting of restricted stock	—	—	—	—	—	—
Purchase of shares under ESPP	—	—	—	—	—	—
Issuance of common stock	—	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	—	—
Other comprehensive income (loss)	—	—	—	—	—	—
Net income (loss)	—	—	—	—	4,254	4,254
Balances at September 30, 2022	—	$ —	$ —	$ —	$ (62,961)	$ (62,961)
Other Adjustments						
Balances at June 30, 2022	—	—	(1,854)	—	(551)	(2,405)
Vesting of restricted stock	—	—	—	—	—	—
Purchase of shares under ESPP	—	—	—	—	—	—
Issuance of common stock	—	—	—	—	—	—
Stock-based compensation expense	—	—	(189)	—	—	(189)
Other comprehensive income (loss)	—	—	—	—	—	—
Net income (loss)	—	—	—	—	5,210	5,210
Balances at September 30, 2022	—	$ —	$ (2,043)	$ —	$ 4,659	$ 2,616
As Restated						
Balances at June 30, 2022	73,551	735	4,124,158	(4,416)	(4,009,981)	110,496
Vesting of restricted stock	572	6	(6)	—	—	—
Purchase of shares under ESPP	67	1	238	—	—	239
Issuance of common stock	8,690	87	45,937	—	—	46,024
Stock-based compensation expense	—	—	9,023	—	—	9,023
Other comprehensive income (loss)	—	—	—	(214)	—	(214)
Net income (loss)	—	—	—	—	(67,056)	(67,056)
Balances at September 30, 2022	82,880	$ 829	$ 4,179,350	$ (4,630)	$ (4,077,037)	$ 98,512

Condensed Consolidated Statements of Cash Flows

The following unaudited condensed consolidated statements of cash flow tables present the impacts of the restatement adjustments for the three months ended March 31, 2023 and 2022, the six months ended June 30, 2023 and 2022, and the nine months ended September 30, 2023 and 2022. The year ended December 31, 2023, was not subject to restatement.

	Three Months ended March 31, 2023			
(in thousands)	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Cash flows from operating activities:				
Net income (loss)	$ 21,240	$ (2,351)	$ 41	$ 18,930
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation and amortization	1,082	5,131	(41)	6,172
Stock-based compensation expense	5,391	—	—	5,391
Noncash research and development expense (finance lease)	—	1,441	—	1,441
Noncash operating lease expense	—	7,423	—	7,423
Gain from sale of priority review voucher	(92,930)	—	—	(92,930)
Excess inventory reserve	228	—	2,355	2,583
Other non-cash items	237	—	—	237
Gain on foreign currency exchange rates	—	(108)	—	(108)
Changes in operating assets and liabilities:				
Prepaid expenses and other assets	(9,335)	2,846	2,800	(3,689)
Inventory	(3,586)	610	(2,355)	(5,331)
Operating lease right-of-use assets	11,829	(11,829)	—	—
Accounts payable	(6,443)	7,703	—	1,260
Accrued expenses and other liabilities	(9,065)	1,859	—	(7,206)
Accrued interest payable under finance lease	—	915	—	915
Operating lease liabilities	(8,001)	1,640	—	(6,361)
Deferred revenue	2,715	—	(2,800)	(85)
Net cash (used in) provided by operating activities	(86,638)	15,280	—	(71,358)
Cash flows from investing activities:				
Purchase of property, plant and equipment	(232)	—	—	(232)
Purchases of marketable securities	(19,610)	—	—	(19,610)
Proceeds from maturities of marketable securities	4,021	—	—	4,021
Proceeds from sales of marketable securities	5,853	—	—	5,853
Purchase of intangible assets	(868)	—	—	(868)
Proceeds from sale of priority review voucher	92,972	—	—	92,972
Net cash provided by investing activities	82,136	—	—	82,136
Cash flows from financing activities:				
Proceeds from exercise of stock options and ESPP contributions	7	—	—	7
Proceeds from vesting of restricted stock	(196)	—	—	(196)
Principal payments on finance lease	—	(15,280)	—	(15,280)
Proceeds from the secondary public offering, net of issuance costs	130,645	—	—	130,645
Net cash (used in) provided by financing activities	130,456	(15,280)	—	115,176
Increase (decrease) in cash, cash equivalents and restricted cash	125,954	—	—	125,954
Cash, cash equivalents and restricted cash at beginning of year	158,445	$ —	$ —	158,445
Cash, cash equivalents and restricted cash at end of year	$ 284,399	$ —	$ —	$ 284,399
Reconciliation of cash, cash equivalents and restricted cash:				
Cash and cash equivalents	$ 239,045	$ —	$ —	$ 239,045
Restricted cash included in receivables and other current assets	1,417	—	—	1,417
Restricted cash included in restricted cash and other non-current assets	43,937	—	—	43,937
Total cash, cash equivalents and restricted cash	$ 284,399	$ —	$ —	$ 284,399
Supplemental cash flow disclosures:				
Purchases of property, plant and equipment included in accounts payable and accrued expenses	189	—	—	189
Offering expenses included in accounts payable and accrued expenses	523	—	—	523
Priority review voucher expenses accrued or in AP	43	—	—	43

Right-of-use assets obtained in exchange for finance lease liabilities	—	(214)	—	(214)
Cash paid during the period for income taxes	2	—	—	2

(in thousands)	Six Months ended June 30, 2023			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Cash flows from operating activities:				
Net income (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 lease 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 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 provided by (used in) financing activities	130,011	(28,504)	—	101,507
Increase (decrease) 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

(in thousands)	Nine Months ended September 30, 2023			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Cash flows from operating activities:				
Net income (loss)	$ (123,399)	$ (10,008)	$ 2,316	$ (131,091)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation and amortization	3,124	16,648	(112)	19,660
Stock-based compensation expense	16,013	—	—	16,013
Noncash research and development expense (finance lease)	—	22,223	—	22,223
Noncash operating lease expense	—	23,965	—	23,965
Gain from sale of priority review voucher	(92,930)	—	—	(92,930)
Excess inventory reserve	5,333	1,554	2,315	9,202
Other non-cash items (finance lease)	—	—	—	—
Other non-cash items	19	—	81	100
Gain on foreign currency exchange rates	—	(1,062)	—	(1,062)
Changes in operating assets and liabilities:				
Accounts receivable	(23,000)	—	8,400	(14,600)
Prepaid expenses and other assets	(523)	2,640	—	2,117
Inventory	(24,931)	1,651	(2,917)	(26,197)
Operating lease right-of-use assets	40,101	(40,101)	—	—
Accounts payable	(5,787)	7,183	316	1,712
Accrued expenses and other liabilities	7,125	583	(1,999)	5,709
Accrued interest payable under finance lease	—	3,203	—	3,203
Operating lease liabilities	(30,506)	11,820	—	(18,686)
Deferred revenue	8,152	—	(8,400)	(248)
Net cash used in operating activities	(221,209)	40,299	—	(180,910)
Cash flows from investing activities:				
Purchase of property, plant and equipment	(2,975)	—	—	(2,975)
Purchases of marketable securities	(43,297)	—	—	(43,297)
Proceeds from maturities of marketable securities	99,521	—	—	99,521
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	151,164	—	—	151,164
Cash flows from financing activities:				
Proceeds from exercise of stock options and ESPP contributions	93	—	—	93
Proceeds from vesting of restricted stock	(196)	—	—	(196)
Principal payments on finance lease	—	(40,299)	—	(40,299)
Proceeds from the secondary public offering, net of issuance costs	130,072	—	—	130,072
Net cash provided by (used in) financing activities	129,969	(40,299)	—	89,670
Increase (decrease) in cash, cash equivalents and restricted cash	59,924	—	—	59,924
Cash, cash equivalents and restricted cash at beginning of year	158,445	—	—	158,445
Cash, cash equivalents and restricted cash at end of year	$ 218,369	$ —	$ —	$ 218,369
Reconciliation of cash, cash equivalents and restricted cash:				
Cash and cash equivalents	$ 165,347	$ —	$ —	$ 165,347
Restricted cash included in receivables and other current assets	8,885	—	—	8,885
Restricted cash included in restricted cash and other non-current assets	44,137	—	—	44,137
Total cash, cash equivalents and restricted cash	$ 218,369	$ —	$ —	$ 218,369
Supplemental cash flow disclosures:				
Right-of-use assets obtained in exchange for operating lease liabilities	44,819	(45,527)	—	(708)
Increase (Reduction) of right of use asset and associated operating lease liability due to lease reassessment	8,003	(8,003)	—	—
Purchases of property, plant and equipment included in accounts payable and accrued expenses	941	—	—	941
Offering expenses included in accounts payable and accrued expenses	248	—	—	248
Right-of-use assets obtained in exchange for finance lease liabilities	—	21,508	—	21,508
Cash paid during the period for income taxes	5	—	—	5

(in thousands)	Three Months ended March 31, 2022			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Cash flows from operating activities:				
Net income (loss)	$ (122,152)	$ 15,160	$ 1,440	$ (105,552)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation and amortization	1,014	89	(56)	1,047
Stock-based compensation expense	12,390	—	(1,384)	11,006
Noncash research and development expense (finance lease)	—	4,050	—	4,050
Noncash operating lease expense	—	3,035	—	3,035
Unrealized loss (gain) on equity securities	2,508	—	—	2,508
Excess inventory reserve	7,519	—	—	7,519
Other non-cash items (finance lease)	—	—	—	—
Other non-cash items	189	—	—	189
Gain on foreign currency exchange rates	—	(530)	—	(530)
Changes in operating assets and liabilities:				
Prepaid expenses and other assets	(4,303)	(2,591)	—	(6,894)
Inventory	(8,756)	—	—	(8,756)
Operating right of use assets	6,517	(6,517)	—	—
Accounts payable	2,467	(1,501)	—	966
Accrued expenses and other liabilities	(16,561)	(12,618)	—	(29,179)
Accrued interest payable under finance lease	—	1,014	—	1,014
Operating lease liabilities	(6,128)	5,981	—	(147)
Net cash used in operating activities	(125,296)	5,572	—	(119,724)
Cash flows from investing activities:				
Purchase of property, plant and equipment	(857)	—	—	(857)
Proceeds from maturities of marketable securities	70,783	—	—	70,783
Net cash provided by investing activities	69,926	—	—	69,926
Cash flows from financing activities:				
Proceeds from exercise of stock options and ESPP contributions	9	—	—	9
Principal payments on finance lease	—	(5,572)	—	(5,572)
Net cash provided by (used in) financing activities	9	(5,572)	—	(5,563)
Increase (decrease) in cash, cash equivalents and restricted cash	(55,361)	—	—	(55,361)
Cash, cash equivalents and restricted cash at beginning of year	206,693	—	—	206,693
Cash, cash equivalents and restricted cash at end of year	$ 151,332	$ —	$ —	$ 151,332
Reconciliation of cash, cash equivalents and restricted cash:				
Cash and cash equivalents	$ 106,260	$ —	$ —	$ 106,260
Restricted cash included in receivables and other current assets	1,822	—	—	1,822
Restricted cash included in restricted cash and other non-current assets	43,250	—	—	43,250
Total cash, cash equivalents and restricted cash	$ 151,332	$ —	$ —	$ 151,332
Supplemental cash flow disclosures:				
Right-of-use assets obtained in exchange for operating lease liabilities	26,882	(1,829)	—	25,053
Purchases of property, plant and equipment included in accounts payable and accrued expenses	2,134	—	—	2,134

(in thousands)	Six Months ended June 30, 2022			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Cash flows from operating activities:				
Net income (loss)	$ (222,290)	$ 28,419	$ 1,963	$ (191,908)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation and amortization	2,358	179	(108)	2,429
Stock-based compensation expense	21,298	—	(1,855)	19,443
Noncash research and development expense (finance lease)	—	4,050	—	4,050
Noncash operating lease expense	—	9,738	—	9,738
Unrealized loss (gain) on equity securities	3,135	—	—	3,135
Excess inventory reserve	7,519	—	—	7,519
Other non-cash items	661	234	—	895
Gain on foreign currency exchange rates	—	(1,874)	—	(1,874)
Changes in operating assets and liabilities:				
Prepaid expenses and other assets	(9,629)	(6,389)	—	(16,018)
Operating right of use assets	17,636	(17,636)	—	—
Accounts payable	(1,175)	590	—	(585)
Accrued expenses and other liabilities	(28,565)	(9,308)	—	(37,873)
Accrued interest payable under finance lease	—	1,924	—	1,924
Operating lease liabilities	(10,602)	8,656	—	(1,946)
Net cash used in operating activities	(219,654)	18,583	—	(201,071)
Cash flows from investing activities:				
Purchase of property, plant and equipment	(6,836)	—	—	(6,836)
Proceeds from maturities of marketable securities	108,225	—	—	108,225
Proceeds from sales of marketable securities	30,213	—	—	30,213
Net cash provided by investing activities	131,602	—	—	131,602
Cash flows from financing activities:				
Principal payments on finance lease	—	(18,583)	—	(18,583)
Proceeds from the secondary public offering, net of issuance costs	8,043	—	—	8,043
Net cash provided by (used in) financing activities	8,043	(18,583)	—	(10,540)
Increase (decrease) in cash, cash equivalents and restricted cash	(80,009)	—	—	(80,009)
Cash, cash equivalents and restricted cash at beginning of year	206,693	—	—	206,693
Cash, cash equivalents and restricted cash at end of year	$ 126,684	$ —	$ —	$ 126,684
Reconciliation of cash, cash equivalents and restricted cash:				
Cash and cash equivalents	$ 81,499	$ —	$ —	$ 81,499
Restricted cash included in receivables and other current assets	1,635	—	—	1,635
Restricted cash included in restricted cash and other non-current assets	43,550	—	—	43,550
Total cash, cash equivalents and restricted cash	$ 126,684	$ —	$ —	$ 126,684
Supplemental cash flow disclosures:				
Right-of-use assets obtained in exchange for operating lease liabilities	218,836	(3,796)	—	215,040
Purchases of property, plant and equipment included in accounts payable and accrued expenses	842	—	—	842

(in thousands)	Nine Months ended September 30, 2022			
	As Previously Reported	Adjustments to Leases	Other Adjustments	As Restated
Cash flows from operating activities:				
Net income (loss)	$ (298,810)	$ 32,673	$ 7,173	$ (258,964)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation and amortization	3,745	268	(129)	3,884
Stock-based compensation expense	30,509	—	(2,044)	28,465
Noncash research and development expense (finance lease)	—	11,866	—	11,866
Noncash operating lease expense	—	16,517	—	16,517
Unrealized loss (gain) on equity securities	3,135	—	—	3,135
Excess inventory reserve	7,519	—	—	7,519
Other non-cash items	2,890	234	—	3,124
Gain on foreign currency exchange rates	—	(3,301)	—	(3,301)
Changes in operating assets and liabilities:				
Prepaid expenses and other assets	(6,197)	(6,508)	—	(12,705)
Operating right of use assets	29,650	(29,650)	—	—
Accounts payable	(7,552)	537	—	(7,015)
Accrued expenses and other liabilities	(39,046)	(10,388)	—	(49,434)
Accrued interest payable under finance lease	—	3,464	—	3,464
Operating lease liabilities	(22,523)	11,580	—	(10,943)
Net cash used in operating activities	(296,680)	27,292	5,000	(264,388)
Cash flows from investing activities:				
Purchase of property, plant and equipment	(8,100)	—	—	(8,100)
Proceeds from maturities of marketable securities	125,095	—	—	125,095
Proceeds from sales of marketable securities	30,216	—	—	30,216
Purchase of intangible assets	—	—	(5,000)	(5,000)
Net cash provided by investing activities	147,211	—	(5,000)	142,211
Cash flows from financing activities:				
Proceeds from exercise of stock options and ESPP contributions	3	—	—	3
Principal payments on finance lease	—	(27,292)	—	(27,292)
Proceeds from the secondary public offering, net of issuance costs	54,365	—	—	54,365
Net cash provided by (used in) financing activities	54,368	(27,292)	—	27,076
Increase (decrease) in cash, cash equivalents and restricted cash	(95,101)	—	—	(95,101)
Cash, cash equivalents and restricted cash at beginning of year	206,693	—	—	206,693
Cash, cash equivalents and restricted cash at end of year	$ 111,592	$ —	$ —	$ 111,592
Reconciliation of cash, cash equivalents and restricted cash:				
Cash and cash equivalents	$ 66,478	$ —	$ —	$ 66,478
Restricted cash included in receivables and other current assets	1,565	—	—	1,565
Restricted cash included in restricted cash and other non-current assets	43,549	—	—	43,549
Total cash, cash equivalents and restricted cash	$ 111,592	$ —	$ —	$ 111,592
Supplemental cash flow disclosures:				
Right-of-use assets obtained in exchange for operating lease liabilities	229,636	(14,596)	—	215,040
Increase (Reduction) of right of use asset and associated operating lease liability due to lease reassessment	(2,833)	2,833	—	—
Purchases of property, plant and equipment included in accounts payable and accrued expenses	176	—	—	176
Offering expenses included in accounts payable and accrued expenses	298	—	—	298
Right-of-use assets obtained in exchange for finance lease liabilities	—	10,800	—	10,800

22. Subsequent events

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 2024 Term Loan is collateralized by substantially all the Company's assets. 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 minimum cash coverage requirement, and a minimum net product revenue requirement. 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. The Company was in compliance with the minimum cash coverage covenant as of March 31, 2024, however, the Company's current business plan anticipates noncompliance with the minimum cash requirements within the twelve months of the issuance of these 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.

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	Description of the Registrant's Securities	—	—	—	Filed herewith
4.3**	Form of Warrant Agreement	8-K	001-35966	4.1	August 14, 2024
4.4	Form of Warrant Agreement Amendment	8-K	001-35966	4.2	August 14, 2024
10.1#	2013 Stock Option and Incentive Plan and forms of award agreement thereunder	S-1/A	333-188605	10.3	June 4, 2013
10.2	Form of Indemnification Agreement between the Registrant and each of its Executive Officers and Directors	S-1	333-188605	10.4	May 14, 2013
10.3†	Patent and Know-How License Agreement No. 07554F30, dated May 14, 2009, by and between the Registrant (formerly known as Genetix Pharmaceuticals Inc.) and INSERM-TRANSFERT, as amended	S-1	333-188605	10.7	May 14, 2013
10.4††	Amendment No. 3 to Patent and Know-How License Agreement No. 07554F33, dated October 29, 2014, by and between the Registrant (formerly known as Genetix, Inc.) and INSERM-TRANSFERT	—	—	—	Filed herewith
10.5	Amendment No. 4 to Patent and Know-How License Agreement No. 07554F33, dated December 16, 2015, by and between the Registrant (formerly known as Genetix, Inc.) and INSERM-TRANSFERT	—	—	—	Filed herewith
10.6†	License Agreement, dated September 13, 2011, by and between the Registrant and Institut Pasteur, as amended	S-1	333-188605	10.8	May 14, 2013
10.7†	Amendment No. 3 to License Agreement, dated September 10, 2013, by and between the Registrant and Institut Pasteur	10-Q	001-35966	10.2	November 14, 2013
10.8†	Amendment No. 4 to License Agreement, dated April 1, 2015, by and between the Registrant and Institut Pasteur	10-Q	001-35966	10.10	May 6, 2015
10.9†	License Agreement, dated December 7, 2011, by and between the Registrant and Research Development Foundation	S-1	333-188605	10.9	May 14, 2013
10.10†	Novation Agreement, dated April 2, 2012, by and between the Registrant and The Board of Trustees of the Leland Stanford Junior University	S-1	333-188605	10.10	May 14, 2013
10.11†	License Agreement, dated December 23, 2015, by and between the Registrant and SIRION Biotech GmbH	10-K	001-35966	10.23	February 21, 2019
10.12††	Clinical and Commercial Supply Agreement – Viral Vector Product, dated November 27, 2017, by and between the Registrant and SAFC Carlsbad, Inc., as amended	10-Q	001-35966	10.25	August 1, 2019
10.13††	Amendment No. 2 to Clinical and Commercial Supply Agreement Viral Vector Product by and between the Registrant and SAFC Carlsbad, Inc.	8-K	001-35966	10.1	January 21, 2020

Exhibit Number	Exhibit Title	Incorporated by Reference			
		Form	File no.	Exhibit	Filing Date
10.14	Amendment No. 3 to Clinical and Commercial Supply Agreement Viral Vector Product by and between the Registrant and SAFC Carlsbad, Inc.	10-K	001-35966	10.28	February 23, 2021
10.15	Amendment No. 4 to Clinical and Commercial Supply Agreement Viral Vector Product by and between the Registrant and SAFC Carlsbad, Inc.	10-Q	001-35966	10.1	August 4, 2022
10.16	Amendment No. 5 to Clinical and Commercial Supply Agreement Viral Vector Product by and between the Registrant and SAFC Carlsbad, Inc.	10-Q	001-35966	10.1	November 7, 2022
10.17	Amendment No. 6 to Clinical and Commercial Supply Agreement Viral Vector Product by and between the Registrant and SAFC Carlsbad, Inc.	—	—	—	Filed herewith
10.18††	Master Manufacturing Services Agreement, dated June 3, 2016, by and between the Registrant and Lonza Houston, Inc.	10-K	001-35966	10.22	March 29, 2023
10.19††	Amendment to Master Manufacturing Services Agreement, dated October 23, 2017, by and between the Registrant and Lonza Houston, Inc.	10-K	001-35966	10.23	March 29, 2023
10.20††	Second Amendment to Master Manufacturing Services Agreement, dated September 21, 2023, by and between the Registrant and Lonza Houston, Inc.	10-Q	001-35966	10.1	November 7, 2023
10.21††	Development and Manufacturing Services Agreement, dated February 28, 2012, by and between the Registrant and Minaris Regenerative Medicine, LLC (formerly known as Hitachi Advanced Therapeutic Solutions, LLC, PCT Cell Therapy Services, PCT, LLC, a Caladrius Company, and Progenitor Cell Therapy, LLC), as amended	—	—	—	Filed herewith
10.22††	Commercial Manufacturing Services Agreement, dated November 3, 2017, by and between the Registrant and Thermo Fisher (successor in interest to Henogen SA), as amended	—	—	—	Filed herewith
10.23#†	Consulting Agreement, dated May 26, 2022, by and between bluebird bio, Inc. and Danforth Advisors, LLC, as amended	10-Q	001-35966	10.5	November 7, 2022
10.24#	Employment Agreement, dated January 7, 2021, by and between the Registrant and Andrew Obenshain	10-K	001-35966	10.23	March 4, 2022
10.25#	Employment Agreement, dated April 20, 2021, by and between the Registrant and Thomas Klima	10-K	001-35966	10.25	March 4, 2022
10.26#	Employment Agreement, dated October 31, 2022, by and between the Registrant and Richard Colvin	10-K	001-35966	10.36	March 29, 2023
10.27#	Employment Agreement, dated January 1, 2023, by and between the Registrant and Joseph Vittiglio	10-K	001-35966	10.37	March 29, 2023
10.28#	Employment Agreement, dated May 28, 2024, by and between the Registrant and O. James Sterling	8-K	001-35966	10.1	May 29, 2024
10.29#	2013 Employee Stock Purchase Plan	S-1/A	333-188605	10.17	June 4, 2013
10.30#	First Amendment of the Bluebird Bio, Inc. 2013 Employee Stock Purchase Plan	10-K	001-35966	10.38	February 21, 2018
10.31#	Second Amendment of the Bluebird Bio, Inc. 2013 Employee Stock Purchase Plan	S-8	333-257135	99.1	June 15, 2021
10.32#	2021 Inducement Plan and forms of award agreements thereunder	S-8	333-257135	99.2	June 15, 2021
10.33#	First Amendment to the bluebird bio, Inc. 2021 Inducement Plan	S-8	333-257135	99.3	March 4, 2022
10.34#	2023 Incentive Award Plan and forms of award agreements thereunder	S-8	333-272714	99.1	June 16, 2023
10.35#	Form of Performance-Based Restricted Stock Unit Agreement under the bluebird bio, Inc. 2023 Incentive Award Plan	—	—	—	Filed herewith

Exhibit Number	Exhibit Title	Incorporated by Reference			
		Form	File no.	Exhibit	Filing Date
10.36#	Executive Cash Incentive Bonus Plan	S-1	333-188605	10.18	May 14, 2013
10.37#	Non-Employee Director Compensation Policy	—	—	—	Filed herewith
10.38††	Sublease, dated April 16, 2019, by and between the Registrant and Aventis Inc.	10-Q	001-35966	10.42	August 1, 2019
10.39	Amendment to Sublease, dated April 19, 2019, by and between the Registrant and Aventis Inc.	10-Q	001-35966	10.43	August 1, 2019
10.40**	Office Lease Agreement, dated November 2, 2021, by and between the Registrant and Assembly Row 5B, LLC	10-Q	001-35966	10.30	November 5, 2021
10.41**	Sub-sublease Agreement, by and between the Registrant and Meta Platforms, Inc.	10-K	001-35966	10.36	March 4, 2022
10.42**	Sublease, dated October 31, 2022, by and between Finch Therapeutics, Inc. and bluebird bio, Inc.	10-Q	001-35966	10.6	November 7, 2022
10.43†††**	Securities Purchase Agreement, dated September 7, 2021, by and among the Registrant and the institutional investors named therein	8-K	001-35966	10.1	September 8, 2021
10.44	Registration Rights Agreement, dated September 7, 2021, by and among the Registrant and the persons listed on the attached Schedule A thereto	8-K	001-35966	10.2	September 8, 2021
10.45	Tax Matters Agreement, dated as of November 3, 2021, by and between the Registrant and 2seventy bio, Inc.	8-K	001-35966	10.1	November 4, 2021
10.46**	Employee Matters Agreement, dated as of November 3, 2021, by and between the Registrant and 2seventy bio, Inc.	8-K	001-35966	10.2	November 4, 2021
10.47**	Intellectual Property License Agreement, dated as of November 3, 2021, by and between the Registrant and 2seventy bio, Inc.	8-K	001-35966	10.3	November 4, 2021
10.48**	Transition Services Agreement, dated as of November 3, 2021, by and between the Registrant and 2seventy bio, Inc.	8-K	001-35966	10.4	November 4, 2021
10.49**	Transition Services Agreement, dated as of November 3, 2021, by and between 2seventy bio, Inc. and the Registrant	8-K	001-35966	10.5	November 4, 2021
10.50**	Amendment to the Transition Services Agreement, by and between 2seventy bio, Inc. and the Registrant	10-Q	001-35966	10.2	August 4, 2022
10.51†††**	Invoice Purchase and Sale Agreement, dated as of December 14, 2023, between the Registrant and Alterna Capital Solutions LLC	8-K	001-35966	10.1	December 18, 2023
10.52†††**	Loan and Security Agreement, dated as of March 15, 2024, between the Registrant, as Borrower, and Hercules Capital, Inc., as Lender	8-K	001-35966	10.1	May 3, 2024
10.53†††**	First Amendment to Loan and Security Agreement, dated April 30, 2024, between the Registrant, as Borrower, and Hercules Capital, Inc., as Lender	8-K	001-35966	10.2	May 3, 2024
10.54	Second Amendment to Loan and Security Agreement, dated July 9, 2024, between the Registrant, as Borrower, and Hercules Capital, Inc., as Lender	8-K	001-35966	10.1	July 11, 2024
10.55	Third Amendment to Loan and Security Agreement, dated August 13, 2024, between the Registrant, as Borrower, and Hercules Capital, Inc., as Lender	8-K	001-35966	10.1	August 14, 2024
10.56	Fourth Amendment to Loan and Security Agreement, dated August 29, 2024, between the Registrant, as Borrower, and Hercules Capital, Inc., as Lender	8-K	001-35966	10.1	August 30, 2024
23.1	Consent of Ernst & Young LLP	—	—	—	Filed herewith
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

Exhibit Number	Exhibit Title	Incorporated by Reference			
		Form	File no.	Exhibit	Filing Date
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
97	Policy for Recovery of Erroneously Awarded Compensation	—	—	—	Filed 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

† Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment and this exhibit has been submitted separately to the SEC.

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

# Indicates a management contract or any compensatory plan, contract or arrangement.

* 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.

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.

By: /s/ Andrew Obenshain

Andrew Obenshain
President, Chief Executive Officer and Director

Date: September 13, 2024

SIGNATURES AND POWER OF ATTORNEY

We, the undersigned directors and officers of bluebird bio, Inc. (the "Company"), hereby severally constitute and appoint Andrew Obenshain and O. James Sterling and each of them singly, our true and lawful attorneys, with full power to them, and to each of them singly, to sign for us and in our names in the capacities indicated below, any and all amendments to this Annual Report on Form 10-K, and to file or cause to be filed the same, with all exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys, and each of them, full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith, as fully to all intents and purposes as each of us might or could do in person, and hereby ratifying and confirming all that said attorneys, and each of them, or their substitute or substitutes, shall do or cause to be done by virtue of this Power of Attorney.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

Name	Title	Date
/s/ Andrew Obenshain Andrew Obenshain	President, Chief Executive Officer and Director *(Principal Executive Officer and Duly Authorized Officer)*	September 13, 2024
/s/ O. James Sterling O. James Sterling	Chief Financial Officer *Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)*	September 13, 2024
/s/ Mark Vachon Mark Vachon	Director	September 13, 2024
/s/ John O. Agwunobi, M.D. John O. Agwunobi, M.D.	Director	September 13, 2024
/s/ Michael Cloonan Michael Cloonan	Director	September 13, 2024
/s/ Charlotte Jones-Burton, M.D. Charlotte Jones-Burton, M.D.	Director	September 13, 2024
/s/ Lis Leiderman, M.D. Lis Leiderman, M.D.	Director	September 13, 2024
/s/ Nick Leschly Nick Leschly	Director	September 13, 2024
/s/ Richard Paulson Richard Paulson	Director	September 13, 2024
/s/ Najoh Tita-Reid Najoh Tita-Reid	Director	September 13, 2024

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CORPORATE AND STOCKHOLDER INFORMATION

Board of Directors

Mark Vachon [1, 2]
Chairman – Independent
Formerly of GE

John O. Agwunobi, MD [1, 3]
Former Chairman & Chief Executive Officer,
Herbalife Nutrition

Michael Cloonan
President and Chief Executive Officer,
Sionna Therapeutics

Charlotte Jones-Burton, MD, MS [3]
Founder, Women of Color in Pharma (WOCIP);
Former SVP, Product Development & Strategy,
Chinook Therapeutics

Elisabeth Leiderman, MD [1, 2]
Chief Financial Officer & Chief Development Officer
Dewpoint Therapeutics

Nick Leschly
Former Chief Kairos Officer, 2seventy bio

Andrew Obenshain
Chief Executive Officer, bluebird bio

Richard Paulson
President and Chief Executive Officer,
Karyopharm Therapeutics Inc.

Najoh Tita-Reid [2, 3]
Chief Brand and Experience Officer, Mars Petcare

[1] *Audit Committee*
[2] *Compensation Committee*
[3] *Nominating and Corporate Governance Committee*

Executive Officers

Richard A. Colvin, PhD, MD
Chief Medical Officer

Thomas J. Klima
Chief Commercial & Operating Officer

Andrew Obenshain
President and Chief Executive Officer

O. James Sterling
Chief Financial Officer

Joseph Vittiglio
Chief Legal and Business Officer

Independent Registered Public Accounting Firm

Ernst & Young LLP
200 Clarendon Street
Boston, MA 02116
www.ey.com

Annual Meeting of Stockholders

The 2024 Annual Meeting of Stockholders will be held on November 6, 2024, at 8:30 a.m. Eastern Time, at 455 Grand Union Boulevard, Somerville, MA 02145.

Common Stock Listing

NASDAQ: BLUE

Corporate Counsel

Latham & Watkins LLP
200 Clarendon Street
Boston, MA 02116
Phone: 617.948.6000
www.lw.com

Transfer Agent / Registrar

Equiniti Trust Company, LLC
48 Wall Street
New York, NY 10005
Phone: 800.937.5449
www.equiniti.com

Investor Relations

Courtney O'Leary
Phone: 978.621.734

Corporate Headquarters
bluebird bio, Inc. ©
455 Grand Union Boulevard
Somerville, MA 02145

www.bluebirdbio.com

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