UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 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: **000-26727**

BioMarin Pharmaceutical Inc.

(Exact name of registrant as specified in its charter)

Delaware	**68-0397820**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
770 Lindaro Street San Rafael California	**94901**
(Address of principal executive offices)	(Zip Code)

(415) 506-6700

(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, par value $.001	**BMRN**	**The Nasdaq Global Select Market**

Securities registered under 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, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☒	Accelerated filer	☐
Non-accelerated filer	☐	Smaller reporting company	☐
		Emerging Growth company	☐

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

Indicate by check mark whether the registrant 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 the voting and non-voting common stock held by non-affiliates of the registrant as of June 30, 2023 was $10.1 billion, based on the closing price reported for such date on the Nasdaq Global Select Market.

As of February 16, 2024, the registrant had 188,675,622 shares of common stock, par value $0.001, outstanding.

Documents Incorporated by Reference: Specified portions of the registrant's definitive proxy statement for the registrant's 2024 annual meeting of stockholders, which will be filed with the Commission no later than 120 days after the end of the registrant's fiscal year ended December 31, 2023, are incorporated by reference under Part III of this Annual Report on Form 10-K.

BIOMARIN PHARMACEUTICAL INC.

2023 FORM 10-K ANNUAL REPORT
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Unless the context suggests otherwise, references in this Annual Report on Form 10-K to "BioMarin," the "Company," "we," "us," and "our" refer to BioMarin Pharmaceutical Inc. and, where appropriate, its wholly owned subsidiaries.

BioMarin®, BRINEURA®, KUVAN®, NAGLAZYME®, PALYNZIQ®, VIMIZIM® and VOXZOGO® are our registered trademarks. ROCTAVIAN® is our registered trademark in the European Union. ROCTAVIAN™ is our trademark in the United States (U.S.). ALDURAZYME® is a registered trademark of BioMarin/Genzyme LLC. All other brand names and service marks, trademarks and other trade names appearing in this report are the property of their respective owners.

Forward-Looking Statements

This Annual Report on Form 10-K contains "forward-looking statements" as defined under securities laws. Many of these statements can be identified by the use of terminology such as "believes," "expects," "intends," "anticipates," "plans," "may," "will," "could," "would," "projects," "continues," "estimates," "potential," "opportunity" or the negative versions of these terms and other similar expressions. You should not place undue reliance on these types of forward-looking statements, which speak only as of the date that they were made. These forward-looking statements are based on the beliefs and assumptions of our management based on information currently available to management and should be considered in connection with any written or oral forward-looking statements that we may issue in the future as well as other cautionary statements we have made and may make. Our actual results or experience could differ significantly from the forward-looking statements. Factors that could cause or contribute to these differences include those discussed in the section titled "Risk Factors" in Part I, Item 1A of this Annual Report on Form 10-K as well as information provided elsewhere in this Annual Report on Form 10-K. You should carefully consider that information before you make an investment decision. Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this Annual Report on Form 10-K may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements.

Except as required by law, we do not undertake any obligation to release publicly any revisions to these forward-looking statements after completion of the filing of this Annual Report on Form 10-K to reflect later events or circumstances or the occurrence of unanticipated events.

Risk Factors Summary

The following is a summary of the principal risks that could adversely affect our business, financial condition, operating results, cash flows or stock price. Discussion of the risks listed below, and other risks that we face, are discussed in the section titled "Risk Factors" in Part I, Item 1A of this Annual Report on Form 10-K.

Business and Operational Risks

- If we fail to obtain and maintain an adequate level of coverage and reimbursement for our products by third-party payers, the sales of our products would be adversely affected or there may be no commercially viable markets for our products.
- As compared to our other, more traditional products, gene therapy products may present additional challenges with respect to the pricing, coverage, reimbursement, and acceptance of the product.
- Because the target patient populations for our products are relatively small, we must achieve significant market share and maintain high per-patient prices for our products to achieve and maintain profitability.
- If we fail to compete successfully with respect to product sales, we may be unable to generate sufficient sales to recover our expenses related to the development of a product program or to justify continued marketing of a product and our revenues could be adversely affected.
- Changes in methods of treatment of disease could reduce demand for our products and adversely affect revenues.
- If we fail to develop new products and product candidates or compete successfully with respect to acquisitions, joint ventures, licenses or other collaboration opportunities, our ability to continue to expand our product pipeline and our growth and development would be impaired.
- The sale of generic versions of KUVAN by generic manufacturers has adversely affected and will continue to adversely affect our revenues and may cause a decline in KUVAN revenues faster than expected.
- If we do not achieve our projected development goals in the timeframes we announce or fail to achieve such goals, the commercialization of our product candidates may be delayed or never occur and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

Regulatory Risks

- If we fail to obtain regulatory approval to commercially market and sell our product candidates, or if approval of our product candidates is delayed, we will be unable to generate revenues from the sale of these product candidates, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will increase.
- Any product for which we have obtained regulatory approval, or for which we obtain approval in the future, is subject to, or will be subject to, extensive ongoing regulatory requirements by the U.S. Food and Drug Administration (FDA), the European Commission (EC), the European Medicines Agency (EMA) and other comparable international regulatory authorities, and if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, we may be subject to penalties, we will be unable to generate revenues from the sale of such products, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will be increased.
- To obtain regulatory approval to market our products, preclinical studies and costly and lengthy clinical trials are required and the results of the studies and trials are highly uncertain. Likewise, preliminary, initial or interim data from clinical trials should be considered carefully and with caution because the final data may be materially different from the preliminary, initial or interim data, particularly as more patient data become available.
- Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our current and future products, which would adversely affect our revenues and results of operations.
- Government healthcare reform could increase our costs and adversely affect our revenues and results of operations.

Financial and Financing Risks

- If we incur operating losses or are unable to sustain positive cash flows for a period longer than anticipated, we may be unable to continue our operations at planned levels and may be forced to reduce our operations.

Manufacturing Risks

- If we fail to comply with manufacturing regulations, our financial results and financial condition will be adversely affected.
- If we are unable to successfully develop and maintain manufacturing processes for our product candidates to produce sufficient quantities at acceptable costs, we may be unable to support a clinical trial or be forced to terminate a program, or if we are unable to produce sufficient quantities of our products at acceptable costs, we may be unable to meet commercial demand, lose potential revenue, have reduced margins or be forced to terminate a program.
- Supply interruptions may disrupt our inventory levels and the availability of our products and product candidates and cause delays in obtaining regulatory approval for our product candidates, or harm our business by reducing our revenues.

Risks Related to International Operations

- We conduct a significant amount of our sales and operations outside of the U.S., which subjects us to additional business risks that could adversely affect our revenues and results of operations.
- A significant portion of our international sales are made based on special access programs, and changes to these programs could adversely affect our product sales and revenues in these countries.
- Our international operations pose currency risks, which may adversely affect our operating results and net income.

Intellectual Property Risks

- If we are unable to protect our intellectual property, we may not be able to compete effectively or preserve our market shares.
- Competitors and other third parties may have developed intellectual property that could limit our ability to market and commercialize our products and product candidates, if approved.

Part I

Item 1. Business

Overview

Founded in 1997, BioMarin Pharmaceutical Inc. (BioMarin, we, us or our) is a global biotechnology company dedicated to transforming lives through genetic discovery. We develop and commercialize targeted therapies that address the root cause of genetic conditions. Our robust research and development capabilities have resulted in multiple innovative commercial therapies for patients with rare genetic disorders. Our distinctive approach to drug discovery has produced a diverse pipeline of commercial, clinical, and pre-clinical candidates that address a significant unmet medical need, have well-understood biology, and provide an opportunity to be first-to-market or offer a substantial benefit over existing treatment options.

Recent Developments

In 2023, we achieved $2.4 billion in total revenues, including a significant contribution from our ongoing expansion of VOXZOGO, and we continued making important advancements in our product development pipeline. Our key business developments in 2023 include U.S. Food and Drug Administration (FDA) approval of VOXZOGO for children with achondroplasia of all ages with open growth plates in the United States, European Commission (EC) approval to expand the indication for VOXZOGO to treat children with achondroplasia aged four months and older with open growth plates in the European Union (EU), and FDA approval of ROCTAVIAN in the U.S. Please see the disclosures below in this Annual Report on Form 10-K for further discussion of these recent developments.

Commercial Products

Commercial Products	Indication	2023 Net Product Revenues (in millions of U.S. Dollars)
Enzyme Products:		
VIMIZIM (elosulfase alpha)	Mucopolysaccharidosis (MPS) IVA	$ 701.0
NAGLAZYME (galsulfase)	MPS VI	$ 420.3
PALYNZIQ (pegvaliase-pqpz)	Phenylketonuria (PKU)	$ 303.9
BRINEURA (cerliponase alfa)	Neuronal ceroid lipofuscinosis type 2 (CLN2)	$ 161.9
ALDURAZYME (laronidase)	MPS I	$ 131.2
Other Products:		
VOXZOGO (vosoritide)	Achondroplasia	$ 469.9
KUVAN (sapropterin dihydrochloride)	PKU	$ 180.8
ROCTAVIAN (valoctocogene roxaparvovec)	Severe Hemophilia A	$ 3.5

VIMIZIM

VIMIZIM is an enzyme replacement therapy for the treatment of MPS IVA, a lysosomal storage disorder. MPS IVA is a disease characterized by deficient activity of N-acetylgalactosamine-6-sulfatase (GALNS) causing excessive lysosomal storage of certain complex carbohydrates known as glycosaminoglycans (GAGs), such as keratan sulfate and chondroitin sulfate. This excessive storage causes a systemic skeletal dysplasia, short stature, and joint abnormalities, which limit mobility and endurance. Malformation of the chest impairs respiratory function, and looseness of joints in the neck cause spinal instability and potentially spinal cord compression. Other symptoms may include hearing loss, corneal clouding, and heart disease. Initial symptoms often become evident in the first five years of life. The disease substantially limits both the quality and length of life of those affected.

VIMIZIM is approved for marketing in the U.S., the EU and other international markets.

NAGLAZYME

NAGLAZYME is a recombinant form of N-acetylgalactosamine 4-sulfatase (arylsulfatase B) indicated for patients with MPS VI. MPS VI is a debilitating life-threatening genetic disease for which no other drug treatment currently exists and is caused by the deficiency of arylsulfatase B, an enzyme normally required for the breakdown of GAGs. Patients with MPS VI typically become progressively worse and experience multiple severe and debilitating symptoms resulting from the build-up of carbohydrate residues in tissues in the body. These symptoms include: inhibited growth, spinal cord compression, enlarged liver and spleen, joint deformities and reduced range of motion, skeletal deformities, impaired cardiovascular function, upper airway obstruction, reduced pulmonary function, frequent ear and lung infections, impaired hearing and vision, sleep apnea, malaise and reduced endurance.

NAGLAZYME is approved for marketing in the U.S., the EU and other international markets.

PALYNZIQ

PALYNZIQ is a PEGylated recombinant phenylalanine (Phe) ammonia lyase enzyme, which is delivered through subcutaneous injection to reduce blood Phe concentrations. PALYNZIQ is our second approved treatment for PKU. PKU is caused by a deficiency of activity of an enzyme, phenylalanine hydroxylase (PAH), which is required for the metabolism of Phe. Phe is an essential amino acid found in all protein-containing foods. Without sufficient quantity or activity of PAH, Phe accumulates to abnormally high levels in the blood, resulting in a variety of serious neurological complications, including severe mental retardation and brain damage, mental illness, seizures and other cognitive problems. As a result of newborn screening efforts implemented in the 1960s and early 1970s, virtually all PKU patients under the age of 40 in developed countries have been diagnosed at birth. PKU can be managed by a Phe-restricted diet, which is supplemented by nutritional replacement products, like formulas and specially manufactured foods; however, it is difficult for most patients to adhere to the strict diet to the extent needed for achieving adequate control of blood Phe levels.

PALYNZIQ is approved for marketing in the U.S. for adult patients with PKU who have uncontrolled blood Phe concentrations greater than 600 micromol/L on existing management. PALYNZIQ is also approved for marketing in the EU, Australia, and Brazil for patients ages 16 and older who have inadequate blood Phe control (blood Phe concentrations greater than 600 micromol/L) despite prior management with available treatment options.

In the U.S., PALYNZIQ is only available through the PALYNZIQ Risk Evaluation and Mitigation Strategy (REMS) program, which is required by the FDA to mitigate the risk of anaphylaxis while using the product. Notable requirements of our REMS program include the following:

- prescribers must be certified by enrolling in the REMS program and completing training;
- prescribers must prescribe auto-injectable epinephrine with PALYNZIQ;
- pharmacies must be certified with the REMS program and must dispense PALYNZIQ only to patients who are authorized to receive it;
- patients must enroll in the REMS program and be educated about the risk of anaphylaxis by a certified prescriber to ensure they understand the risks and benefits of treatment with PALYNZIQ; and
- patients must have auto-injectable epinephrine available at all times while taking PALYNZIQ.

Please see “Risk Factors” included in Part I, Item 1A of this Annual Report on Form 10-K for a discussion of the risks posed by the REMS program.

BRINEURA

BRINEURA is a recombinant human tripeptidyl peptidase 1 (TPP1) for the treatment of patients with CLN2, a form of Batten disease. CLN2 is an incurable, rapidly progressive disease that typically ends in patient death by 10-12 years of age. Patients are initially healthy but begin to decline at approximately the age of three. BRINEURA is the first treatment approved to slow the progression of loss of ambulation in children with CLN2 disease and was one of the first therapies to go through an accelerated review procedure in the EU.

BRINEURA is administered via intracerebroventricular (ICV) infusion and intended to be used in combination with a delivery device, such as an injector or other delivery system. Please see “Government Regulation – Regulation of Approved Products – Combination Products and Companion Diagnostics” in this Annual Report on Form 10-K for additional information on combination products.

BRINEURA is approved for marketing in the U.S. (for ages three and older) and in the EU (for all ages from birth) and in other international markets.

ALDURAZYME

ALDURAZYME is a highly purified protein that is designed to be identical to a naturally occurring form of the human enzyme alpha-L-iduronidase, a lysosomal enzyme normally required for the breakdown of GAGs. MPS I is a progressive and debilitating life-threatening genetic disease that is caused by the deficiency of alpha-L-iduronidase. Patients with MPS I typically become progressively worse and experience multiple severe and debilitating symptoms resulting from the build-up of carbohydrate residues in all tissues in the body. These symptoms include: inhibited growth, delayed and regressed mental development (in the severe form of the disease), enlarged liver and spleen, joint deformities and reduced range of motion, impaired cardiovascular function, upper airway obstruction, reduced pulmonary function, frequent ear and lung infections, impaired hearing and vision, sleep apnea, malaise and reduced endurance.

We developed ALDURAZYME through collaboration with Sanofi. Under our collaboration agreement with Sanofi, we are responsible for manufacturing ALDURAZYME and supplying it to Sanofi. We receive payments ranging from 39.5% to 50% on worldwide net ALDURAZYME sales by Sanofi depending on sales volume. Sanofi and BioMarin are members of BioMarin/Genzyme LLC, a 50/50 limited liability company (the BioMarin/Genzyme LLC) that: (1) holds the intellectual property relating to ALDURAZYME and other collaboration products and licenses all such intellectual property on a royalty-free basis to Sanofi and BioMarin to allow us to exercise our rights and perform our obligations under the agreements related to the BioMarin/Genzyme LLC, and (2) engages in research and development activities that are mutually selected and funded by Sanofi and us.

ALDURAZYME is approved for marketing in the U.S., the EU and other international markets.

VOXZOGO

VOXZOGO is a once daily injection analog of C-type Natriuretic Peptide (CNP) for the treatment of achondroplasia, the most common form of disproportionate short stature in humans. In patients with achondroplasia, endochondral bone growth, an essential process by which bone tissue is created, is negatively regulated due to a gain of function mutation in fibroblast growth factor receptor 3 gene (FGFR3). VOXZOGO acts as a positive regulator of the signaling pathway downstream of FGFR3 to promote endochondral bone growth.

VOXZOGO is approved for marketing for the treatment of achondroplasia in children with open growth plates of all ages in the U.S. and Japan, children with open growth plates aged four months and older in the EU, and patients in various age ranges for other markets, including Australia and Brazil.

We continue to research VOXZOGO's safety and effectiveness in children with achondroplasia. At the 2023 American College of Medical Genetics and Genomics Annual Clinical Genetics Meeting, we presented updated data demonstrating the long-term benefit of treatment with VOXZOGO and new observational data on disease burden in children with achondroplasia.

In the fourth quarter of 2023, we began the pivotal program with VOXZOGO for the treatment of children with hypochondroplasia. The six-month run-in study will be followed by the 52-week randomized, double-blind, placebo-controlled phase of the 80-participant clinical trial, with the treatment study expected to begin mid-2024. We are engaging global health authorities in the first half of 2024 regarding development programs in idiopathic short stature and multiple genetic short stature pathway conditions, with plans to begin pivotal studies later in 2024.

Please see "Risk Factors" included in Part I, Item 1A of this Annual Report on Form 10-K for a discussion of the risks related to VOXZOGO in the U.S. and international markets.

KUVAN

KUVAN is a proprietary synthetic oral form of 6R-BH4, a naturally occurring enzyme co-factor for PAH, indicated for patients with PKU. KUVAN is the first drug for the treatment of PKU, which is an inherited metabolic disease. We believe that approximately 30% to 50% of those with PKU could benefit from treatment with KUVAN.

KUVAN is approved for marketing in the U.S., the EU and other international markets (excluding Japan). In certain international markets, KUVAN is also approved for, or is only approved for, the treatment of primary BH4 deficiency, a different disorder than PKU.

Generic versions of KUVAN are available in several countries around the world, including multiple generic versions in the U.S. We are also aware that manufacturers are challenging our patent portfolio related to KUVAN in several jurisdictions, and several generic versions of KUVAN have been approved either centrally by the EC or on a country-by-country basis throughout the EU. Please see "Risk Factors" included in Part I, Item 1A of this Annual Report on Form 10-K for a discussion of the risks posed by generic versions of KUVAN in the U.S. and international markets.

ROCTAVIAN

ROCTAVIAN is an adeno associated virus (AAV5) vector gene therapy designed to restore factor VIII plasma concentrations in patients with severe hemophilia A. Hemophilia A, also called factor VIII deficiency or classic hemophilia, is a genetic disorder caused by missing or defective factor VIII, a clotting protein. According to the World Federation of Hemophilia rankings of severity of hemophilia A, the normal range of factor VIII activity levels is between 50% and 150%, expressed as a percentage of normal factor activity in blood, the mild hemophilia A range of factor VIII activity levels is between 5% and 40%, the moderate hemophilia A range of factor VIII activity levels is between 1% and 5%, and the severe hemophilia range of factor VIII activity levels is less than 1%. People living with hemophilia A are not able to form blood clots efficiently and are at risk for

excessive bleeding from modest injuries, potentially endangering their lives. People with severe hemophilia often bleed spontaneously into their muscles or joints.

ROCTAVIAN was conditionally approved by the EC in August 2022 and approved by the FDA in the U.S. in June 2023. Our European launch of ROCTAVIAN is underway following ROCTAVIAN'S conditional approval for marketing in the EU for the treatment of severe hemophilia A in adult patients without a history of factor VIII inhibitors and without detectable antibodies to AAV5. We plan to provide the European Medicines Agency (EMA) further clinical data in an effort to convert our conditional approval to a standard marketing authorization. Please see “Government Regulation – Adaptive Pathways” in this Annual Report on Form 10-K for additional information on conditional marketing authorizations.

We have and continue to collaborate with payers around the world to secure reimbursement for ROCTAVIAN on terms which are intended to assist payers with realizing the value and sharing the risk of a one-time treatment. For example, we have agreed with the German National Association of Statutory Health Insurance Funds an outcome-based prospective cohort model for ROCTAVIAN which will allow future reimbursement to be increased or decreased based on real-world data collected from the German Haemophilia Registry of patients treated with ROCTAVIAN. We are also continuing our development efforts on ROCTAVIAN expansion opportunities.

Please see “Risk Factors” included in Part I, Item 1A of this Annual Report on Form 10-K for a discussion of the risks related to ROCTAVIAN in the U.S. and international markets.

Research and Development Programs

We have multiple clinical and preclinical product candidates in various stages of development that are intended to address the root causes of genetic conditions with a significant unmet medical need. Generally, our development programs have well-understood biology and provide an opportunity to be first-to-market or offer a substantial benefit over existing treatment options.

In 2023, we conducted clinical trials on several product candidates for the treatment of various diseases and progressed pre-clinical activities, including studies intended to support Investigational New Drug (IND) application or Clinical Trial Application (CTA) submissions.

During the first quarter of 2024, our management began a strategic portfolio review of all research and development (R&D) programs to determine which R&D assets have the highest potential patient impact and highest potential value creation for stockholders.

Manufacturing

We manufacture the active pharmaceutical ingredients (API) for ALDURAZYME, NAGLAZYME, PALYNZIQ, VOXZOGO, and ROCTAVIAN in our production facilities located in Novato, California. Our commercial-scale gene therapy manufacturing facility, located in Novato, California, also supports our clinical development activities. This facility has the potential to produce multiple gene therapy products to meet global commercial demand, depending on dose and production mix. We manufacture the API for BRINEURA and VIMIZIM in our manufacturing facility in Shanbally, Cork, Ireland. Our Novato and Shanbally facilities have been inspected and have demonstrated compliance with current Good Manufacturing Practice (cGMP) to the satisfaction of the FDA, the EC and health agencies in other countries. We also have installed aseptic filling and drug product packaging capabilities at the Shanbally site. Regulatory inspections of this new drug product filling facility are planned and/or anticipated over the coming months.

We contract with third parties to manufacture KUVAN API. Additionally, most of our drug product manufacturing (which includes vials, syringes, tablets, and powder) is performed externally by contract manufacturers. The volume mix will change as drug product filing operations initiate and expand in the Shanbally site. Packaging operations are effectively split between installed capacity at the Shanbally site and several contract manufacturers. We expect to continue to contract with outside service providers for certain manufacturing services, including drug substance, drug product, and packaging operations for our products. All of our facilities and those of any third-party manufacturers will be subject to periodic inspections confirming compliance with applicable law and must pass inspection before we can manufacture our drugs for commercial sale. Third-party manufacturers’ facilities are subject to periodic inspections to confirm compliance with applicable law and must be cGMP certified. We believe that our current agreements with third-party manufacturers and suppliers provide for ample operating capacity to support the anticipated clinical and commercial demand for these products. In certain instances, there is only one approved contract manufacturer for certain aspects of the manufacturing process. In such cases, we attempt to prevent disruption of supplies through supply agreements, maintaining safety stock and other appropriate strategies.

Raw Materials

Raw materials and supplies required to produce our products and product candidates are available in some instances from one supplier and in other instances from multiple suppliers. In those cases where raw materials are only available through one supplier, such supplier may be either a sole source (the only recognized supply source available to us) or a single source (the only

approved supply source for us among other sources). We have adopted policies to attempt, to the extent feasible, to minimize our raw material supply risks, including maintenance of greater levels of raw materials inventory and implementation of multiple raw materials sourcing strategies, especially for critical raw materials. Although to date we have not experienced any significant delays in obtaining any raw materials from our suppliers, we cannot provide assurance that we will not face shortages from one or more of them in the future. Please see the risk factor, "Supply interruptions may disrupt our inventory levels and the availability of our products and product candidates and cause delays in obtaining regulatory approval for our product candidates, or harm our business by reducing our revenues." described in "Risk Factors" in Part I, Item 1A of this Annual Report on Form 10-K.

Sales and Marketing

We have established a commercial organization, including a sales force, to support our product lines directly in the U.S., Europe, South America and certain other significant markets. For other selected markets, we have signed agreements with other companies to act as distributors of all our products, other than ALDURAZYME. Most of these agreements generally grant the distributor the right to market the product in the territory and the obligation to secure all necessary regulatory approvals for commercial or named patient sales. Additional markets are being assessed at this time and additional agreements may be signed in the future.

Sanofi has the exclusive right to distribute, market and sell ALDURAZYME globally and is required to purchase its requirements exclusively from us.

In the U.S., our products (other than ALDURAZYME) are marketed through our commercial teams, including sales representatives and supporting staff members, who promote our products directly to physicians in specialties appropriate for each product. Outside of the U.S., our sales representatives and supporting staff members market our products (other than ALDURAZYME). We believe that with moderate changes in 2024, the size of our sales force will be appropriate to effectively reach our target customers in markets where our products are directly marketed. The launch of any future products, if approved, will likely require expansion of our commercial organization, including our sales force, in the U.S. and international markets.

We utilize third-party logistics companies to store and distribute our products. Moreover, we use third-party vendors, such as advertising agencies, market research firms and suppliers of marketing and other sales support-related services, to assist with our commercial activities.

Customers

Customers for our products (other than ALDURAZYME) include a limited number of specialty pharmacies and end-users, such as hospitals and non-U.S. government agencies. We also sell our products (other than ALDURAZYME) to our authorized distributors and to certain larger pharmaceutical wholesalers globally, which act as intermediaries between us and end-users and generally do not stock significant quantities of our products. However, in certain countries, governments place large periodic orders for NAGLAZYME and VIMIZIM. The timing of these orders can be inconsistent and can create significant quarter to quarter variation in our revenue. PALYNZIQ is currently distributed in the U.S. pursuant to the REMS program through a limited number of certified specialty pharmacies. During 2023, 36% of our net product revenue was generated by three customers. Sanofi is our sole customer for ALDURAZYME and is responsible for distributing, marketing, and selling ALDURAZYME to third parties.

Competition

Commercial Products

The biopharmaceutical industry is rapidly evolving and highly competitive. Within the industry, there are many public and private companies, including pharmaceutical companies and biotechnology companies that have or may soon initiate programs for the same indications that our products and product candidates are intended to treat. Furthermore, universities and non-profit research organizations may have research programs, both early-stage and clinical, in the same disease areas. Our larger competitors may have advantages over us due to greater financial or scientific resources, lower labor and other costs, or higher headcount and more robust organizational structures, while smaller competitors may have advantages over us due to lower overhead costs, being more nimble, or being able to focus on a narrower set of indications or development programs. Our competitors have considerable experience in drug manufacturing, preclinical and clinical research and development, regulatory affairs, marketing, sales, and distribution. They pursue broad patent portfolios and other intellectual property to protect the products they are developing. Their products may outcompete ours due to one or more factors, including faster progress through preclinical and clinical development, lower manufacturing costs, superior safety and efficacy, lower pricing, stronger patent protection, and better marketing, sales, and distribution capabilities. In this event, our products and product candidates, if approved, could fail to gain significant market share, and as a result, our business, financial condition and results of operations could be adversely affected.

Other than ROCTAVIAN and KUVAN, as described below, our products have no direct approved competition currently on the market in the U.S. or the EU; however, other companies are in the development phase with new and generic products. Our products and product candidates have potential competition from products under development either using similar technology to

our programs or different treatment strategies. The following is a summary of some of the primary possible future competitors for our products and product candidates, but the information below may not include all potential competition.

ALDURAZYME, NAGLAZYME, and VIMIZIM

In the mucopolysaccharidosis field, several companies are researching treatments using small molecules, gene therapy, and other novel technologies. ALDURAZYME, for the treatment of MPS I, has potential competition from clinical stage product candidates from ArmaGen, Inc., JCR Pharmaceuticals Co., Ltd (acquired by ArmaGen, Inc.)., Orchard Therapeutics Plc and RegenxBio Inc. and earlier stage product candidates, including product candidates from Denali Therapeutics Inc. and Immusoft Corporation. NAGLAZYME, for the treatment of MPS VI, has potential competition from clinical stage product candidates from Inventiva S.A. and Paradigm Biopharmaceuticals Limited and other potential candidates in earlier stages. VIMIZIM, for the treatment of MPS IVA, has potential competition from preclinical product candidates from Esteve Pharmaceuticals, S.A., and RegenxBio Inc. and other potential candidates in earlier stages.

BRINEURA

BRINEURA, for the treatment of CLN2, has potential competition from preclinical product candidates from Lexeo Therapeutics, Inc., RegenxBio Inc. and the Roche Group.

PALYNZIQ and KUVAN

There are currently no other approved, non-generic drugs on the market in the U.S. or the EU for the treatment of PKU. However, generic versions of KUVAN are available in several countries around the world, including multiple generic versions in the U.S. We are also aware that manufacturers are challenging our patent portfolio related to KUVAN in several jurisdictions, and several generic versions of KUVAN have been approved either centrally by the EMA or on a country-by-country basis throughout the EU. Please see “Risk Factors” included in Part I, Item 1A of this Annual Report on Form 10-K for a discussion of the risks posed by generic versions of KUVAN in the U.S. and international markets. PALYNZIQ and KUVAN also have potential competition from clinical stage product candidates from Jnana Therapeutics Inc., Nestle Health Science, S.A., Sanofi, S.A., PTC Therapeutics, Inc. Moderna Therapeutics Inc., Agios Pharmaceuticals Inc., SOM Innovation Biotech, S.A., and Synlogic, Inc. and earlier stage product candidates, including product candidates from Generation Bio Co., Moderna Therapeutics, Inc., Poseida Therapeutics, Inc., Tessera Therapeutics, Inc., and Evox Therapeutics Limited. We and other companies are also developing gene therapy product candidates for PKU.

VOXZOGO

VOXZOGO, for the treatment of achondroplasia, could have competition from clinical stage products under development by Ascendis Pharma A/S, Pfizer, Inc., QED Therapeutics, Inc. (a subsidiary of BridgeBio Pharma, Inc.), Ribomic Inc. and Sanofi and preclinical product candidates from other companies, including Astellas Pharma Inc., Tyra Biosciences, Inc., Abbisko Therapeutics Co Ltd, SiSaf Ltd, Peptron Inc., and Immunoforge, Co. Ltd.

ROCTAVIAN

ROCTAVIAN, a gene therapy for the treatment of adults with severe hemophilia A, has potential competition from marketed recombinant factor VIII replacement therapies, including products marketed by Sanofi S.A., Takeda Pharmaceutical Company Limited, Bayer AG, Novo Nordisk A/S, CSL Behring, and Pfizer, Inc., a novel bispecific antibody marketed by the Roche Group, and clinical stage programs, including gene therapy product candidates under development by ASC Therapeutics, Inc., Pfizer, Inc., and the Roche Group. In addition, Novo Nordisk A/S, Pfizer, Inc., the Roche Group and Sanofi are developing novel non-factor replacement product candidates in the clinic for the treatment of hemophilia A.

Research and Development Programs

Substantially all of our clinical and preclinical product candidates have potential competition from several companies, which in some cases, have development programs in later stages than our own.

Patents, Proprietary Rights and Regulatory Exclusivity

Our success depends on an intellectual property portfolio that supports our future revenue streams and also creates barriers to our competitors. We are maintaining and building our patent portfolio through: filing new patent applications; prosecuting existing applications; and licensing and acquiring new patents and patent applications. Furthermore we seek to protect our ownership of know-how, trade secrets and trademarks through an active program of legal mechanisms including registrations, assignments, confidentiality agreements, material transfer agreements, research collaborations and licenses.

U.S. patents, as well as most foreign patents, are generally effective for 20 years from the date the earliest application was filed. U.S. patents that were issued on applications filed before June 8, 1995, may be effective until 17 years from the issue date, if that is later than the 20-year date. In some cases, the patent term may be extended to recapture a portion of the term lost during regulatory review of the claimed therapeutic or, in the case of the U.S., because of U.S. Patent and Trademark Office (USPTO) delays in prosecuting the application. In the U.S., under the Drug Price Competition and Patent Term Restoration Act of 1984 (commonly known as the Hatch-Waxman Act), a patent that covers a drug approved by the FDA may be eligible for patent term extension (for up to five years, but not beyond a total of 14 years from the date of product approval) as compensation for patent term lost during the FDA regulatory review process. The duration and extension of the term of foreign patents varies in accordance with local law. In the EU, Supplementary Protection Certificates (SPCs) are available to extend a patent term up to five years to compensate for patent protection lost during regulatory review. Although all EU Member States must provide SPCs, SPCs must be applied for and granted on a country-by-country basis. Limited exceptions apply to the protection conferred by the SPC.

The table below lists our active patents and patent applications of primary importance for our products other than ALDURAZYME and NAGLAZYME by territory, general subject matter (including composition, methods of treatment and approved use, methods of production and purification, pharmaceutical compositions and clinical formulations) and latest expiry date. With respect to ALDURAZYME and NAGLAZYME, the last of our patents expired in November 2020 and November 2023, respectively. One or more patents with the same or earlier expiry dates may fall under the same general subject matter and are not listed separately in the table below. We continue to pursue additional patents and patent term extensions in the U.S. and other territories covering various aspects of our products that may, if issued, extend patent exclusivity beyond the expiration dates listed in the table below.

Product	Territory	Patent No(s).	General Subject Matter	Patent Expiration
BRINEURA	U.S.	8,029,781	Method of treatment	March 7, 2023[1]
		9,044,473	Method of treatment by administration into the cerebrospinal fluid	February 18, 2032
		10,279,015	Formulation; kit	May 5, 2036
	EU	1673104	Pharmaceutical composition	August 30, 2024
		EP3294345	Formulation	May 5, 2036
PALYNZIQ	U.S.	7,534,595	Composition; method of treating	May 24, 2032[2]
		10,221,408	Purification	February 3, 2031
		9,557,340	Antibody detection assay	July 30, 2029
		11,505,790	Regimen	February 3, 2031
	EU	2152868	Composition; pharmaceutical composition	May 23, 2028 / May 23, 2033[3]
		2531209; 3025728	Formulation; purification	February 3, 2031
ROCTAVIAN	US	9,504,762; 10,463,718; 11,406,690	Compositions, Methods of Treatment, Production	September 10, 2034[4]
		10,512,675; 11,690,898	Formulation, Clinical Methods of Treatment	April 10, 2037 December 19, 2038
	EU	3044231	Compositions, Methods of Treatment	September 10, 2034[5]
VIMIZIM	U.S.	8,128,925	Compositions; methods of treatment	April 10, 2030
		8,765,437	Purification; formulation; methods of treatment	January 10, 2032
	EU	2245145	Composition; use for treating	April 30, 2029[6]
		2595650	Purification; composition; use for treating; formulation	July 22, 2031
VOXZOGO	U.S.	8,198,242	Compositions, Methods of Treatment	June 11, 2030[7]
		9,907,834	Formulation	August 1, 2036
		10,646,550	Clinical methods of treatment	August 1, 2036
	EU	2432489	Compositions, Methods of Treatment	May 20, 2030[8]

(1) Date of expiry includes patent term extension (PTE).

(2) Date of expiry includes the granted PTE.

(3) We applied for SPCs for this patent, and we have to date received SPC to extend the patent expiration to May 23, 2033 in certain European countries, including Austria, Bulgaria, Croatia, Cyprus, Czech Republic, Denmark, Estonia, Finland France, Greece, Hungary, Ireland, Iceland, Italy, Latvia, Lithuania, Luxembourg, Malta, Netherlands, Norway, Poland, Portugal, Spain, Slovakia, Slovenia, Sweden, and United Kingdom.

(4) We filed for a PTE for these patents, and if granted, we expect the patents' expirations will extend to June 29, 2037 for the 9,504,762 patent and November 21, 2036 for the 11,406,690 patent.

(5) We applied for SPCs for this patent and we have to date received SPC to extend the patent expiration to August 25, 2037 in certain European countries, including Austria, Cyprus, Denmark, Estonia, Finland, Hungary, Italy, Lithuania, Luxembourg, Latvia, Malta, Netherlands, and Portugal.

(6) We applied for SPCs for this patent, and we have to date received SPC to extend the patent expiration to April 30, 2029 in certain European countries, including Austria, Belgium, Bulgaria, Cypress, Croatia, Czech Republic, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Iceland, Ireland, Italy, Latvia, Lithuania, Luxembourg, Malta, Netherlands, Norway, Poland, Portugal, Romania, Slovak Republic, Slovenia, Spain, Sweden, Switzerland and the United Kingdom.

(7) We filed for a PTE for this patent, and if granted, we expect the patent expiration will extend to May 20, 2035.

(8) We applied for SPCs for this patent and have been granted SPCs so far in Greece, Estonia, Hungary, Sweden, France, Italy, Austria, Japan, and Australia, extending the patent expiration to May 20, 2035.

In addition to patent protection, certain of our products are entitled to regulatory exclusivity in the U.S. and the EU through the dates set forth below:

Commercial Products	United States Orphan Drug Exclusivity Expiration [1]	United States Biologic Exclusivity Expiration [2]	European Union Orphan Drug Exclusivity Expiration [1]
BRINEURA	2024	2029	2027
PALYNZIQ	2025	2030	2029
ROCTAVIAN	2030	2035	2032
VIMIZIM	Expired	2026	2024
VOXZOGO	2028	Not Applicable	2031

(1) See "Government Regulation—Other Regulation—Orphan Drug Designation" in this Annual Report on Form 10-K for further discussion.

(2) See "Government Regulation—Other Regulation—Exclusivity for Biologics in the U.S." in this Annual Report on Form 10-K for further discussion.

With respect to our clinical product candidates, we believe we have the necessary intellectual property rights to allow us to undertake the development of these candidates. Certain of our product candidates are in therapeutic areas that have been the subject of many years of extensive research and development by academic organizations and third parties who may control patents or other intellectual property that they might assert against us, should one or more of our product candidates in these therapeutic areas succeed in obtaining regulatory approval and thereafter be commercialized. We continually evaluate the intellectual property rights of others in these areas in order to determine whether a claim of infringement may be made by others against us. Should we determine that a third party has intellectual property rights that could impact our ability to freely market a compound we consider a number of factors in determining how best to prepare for the commercialization of any such product candidate. In making this determination we consider, among other things, the stage of development of our product candidate and whether we and our outside counsel believe the intellectual property rights of others are valid, whether we infringe the intellectual property rights of others, whether a license is available upon commercially reasonable terms, whether we will seek to challenge the intellectual property rights of others, and the likelihood of and liability resulting from an adverse outcome should we be found to infringe the intellectual property rights of others.

Government Regulation

Regulation by governmental authorities in the U.S., European countries and other countries is a significant factor in the development, manufacture, commercialization, pricing and reimbursement of our products. Our industry is subject to significant federal, state, local and non-U.S. regulation. Our products require approval from the FDA, the EC (on the basis of the scientific opinions issued by the EMA) and corresponding agencies in other countries before they can be marketed. Failure to comply with applicable U.S. and foreign requirements may subject a company to a variety of administrative or judicial sanctions, such as FDA refusal to approve pending New Drug Applications (NDAs) or Biologics License Applications (BLAs), warning or untitled letters,

investigations, product recalls, product seizures, total or partial suspension or withdrawal of marketing, production or distribution authorizations, injunctions, fines, civil penalties, and criminal prosecution.

Approval Process in the U.S. and EU

Satisfaction of FDA and EU pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity and novelty of the product or disease. Pharmaceutical product development in the U.S. and the EU typically involves preclinical laboratory and animal tests, the submission to the applicable regulatory agency of an application (e.g., an IND in the U.S. or a CTA in the EU), which must become effective before clinical testing may commence, and adequate and well-controlled human clinical trials to establish the safety and effectiveness of the drug for each indication for which marketing approval is sought. On January 31, 2022, Regulation EU No 536/2014 (CTR) became fully applicable in the EU. The CTR established a centralized application procedure where one of the National Competent Authorities (NCA) of the Member States where the trial will take place takes the lead in reviewing certain aspects of the application, while the other NCAs have a lesser involvement than they had under the previous regime established by Directive 2001/20/EC (CTD). The CTD indeed introduced the first set of harmonized rules on clinical trials in the EU but resulted in a patchwork of different national regimes. The CTR was adopted with a view to introducing a more uniform set of the rules across the EU for the authorization of clinical trials. Such authorization still involves the national regulatory authorities and Ethics Committees of each of the EU Member States where the trial is to be conducted. However, the relevant procedures have now been streamlined with a view to facilitating a swifter and more seamless authorization and deployment of multi-center trials occurring in more than one EU Member State. More specifically, the CTR allows sponsors to rely on one single submission for CTAs regardless of the number of Member States where the trial takes place and based on a single harmonized application. Furthermore, under the CTR, deadlines for regulatory approvals are shortened with a view to accelerating the authorization process. The CTR also established an EU Portal which will act as a single-entry point for submission of data and information relating to clinical trials. Until January 30, 2025, the CTD will continue to apply in parallel to the CTR for a transitional period. From January 31, 2025 all trials will have to comply with the CTR.

Preclinical tests include laboratory evaluation, as well as animal studies, to assess the characteristics and potential pharmacology, pharmacokinetics and toxicity of the product. The conduct of the preclinical tests must comply with FDA and/or EU and national regulations and requirements, including good laboratory practices (GLP). The results of preclinical testing, along with other information, including information about product chemistry, manufacturing and controls and a proposed clinical trial protocol are reviewed by the applicable regulatory agency as part of an IND or CTA. Long-term preclinical tests, such as animal tests of reproductive toxicity and carcinogenicity, may continue after the IND or CTA is submitted. Until the CTA or IND is approved or becomes effective following a waiting period, and appropriate reviews have been satisfactorily completed by the applicable Institutional Review Boards (IRBs) or Ethics Committees, we may not start the clinical trial in the relevant jurisdiction.

Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted in compliance with applicable regulations, good clinical practices (GCP), as well as under protocols detailing the objectives of the trial and the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol involving testing on patients and subsequent protocol amendments must be submitted to the FDA as part of the IND and to the relevant regulatory agency in the EU as part of a new CTA.

The regulatory agencies may order the temporary halt or permanent discontinuation of a clinical trial at any time or impose other sanctions if they believe that the clinical trial is not being conducted in accordance with applicable requirements or presents an unacceptable risk to the clinical trial patients. An IRB/Ethics Committee may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB/Ethics Committee's requirements, or may impose other conditions. Clinical trials that are deployed to support NDAs, BLAs or Marketing Authorization Applications (MAAs) for marketing approval are typically conducted in three sequential phases, but the phases may overlap or be combined. Typically, we undertake a three-phase human clinical testing program as follows:

- Phase 1 - the initial introduction of the drug into healthy human subjects or patients, the drug is tested to assess metabolism, pharmacokinetics, pharmacological actions, side effects associated with increasing doses and, if possible, early evidence on effectiveness.
- Phase 2 - usually involves trials in a limited patient population, to determine the effectiveness of the drug for a particular indication or indications, dosage tolerance and optimum dosage, and to identify common adverse effects and safety risks. If a compound demonstrates evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations.
- Phase 3 - undertaken to obtain the additional information about clinical efficacy and safety in a larger number of patients, typically at geographically dispersed clinical trial sites.

After completion of the required clinical testing, an application is prepared and submitted to the applicable regulatory agency. Approval of the application by the applicable regulatory agency is required before marketing of the product may begin. In the European Economic Area (i.e., the EU as well as Iceland, Liechtenstein and Norway) (the EEA), there are two types of marketing authorizations (MA), namely: (i) the "Union" MA, which is issued by the EC through the so-called "centralized procedure", based on the positive opinion of the EMA's Committee for Medicinal Products for Human Use (CHMP), and results in a single marketing authorization that is valid across the EEA; and (ii) "National MAs," which are issued by the competent NCAs and only

cover their respective territory. The centralized procedure is mandatory for certain types of products such as: (i) medicinal products derived from certain biotechnology processes, (ii) designated orphan medicinal products, (iii) medicinal products containing a new active substance indicated for the treatment of certain diseases such as HIV/AIDS, cancer, neurodegenerative disorders, diabetes, autoimmune diseases and other auto-immune dysfunctions, viral diseases; and (iv) Advanced Therapy Medicinal Products (ATMPs) (such as gene therapy, somatic cell therapy or tissue-engineered medicines). The NDA, BLA or MAA must include the results of all preclinical, clinical and other testing, a compilation of data relating to the product's pharmacology, chemistry, manufacture and controls and proposed labeling, among other things. In the U.S., each NDA or BLA is subject to a significant user fee at the time of submission, unless a waiver is granted by the FDA. Similarly, in the EU, the submission of an MAA is subject to the payment of fees, a waiver of which may be obtained only under limited circumstances. The FDA and the EMA initially review the applications for a threshold determination that it is sufficiently complete to permit substantive review. The regulatory agency may request additional information rather than accepting an application for filing or validation. Once the submission is accepted, the applicable agency begins an in-depth review. For the FDA, the review period for standard review applications for new molecular entities is typically ten months from the date the FDA files the application and, for priority review of drugs, that is, drugs that the FDA determines address a significant unmet need and represent a significant improvement over existing therapy, the review period is typically six months from the date the FDA files the application. The review process may be extended by the FDA for three additional months to consider new information submitted during the review or clarification regarding information already provided in the submission. The FDA may also refer applications for novel products or products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations. After the FDA evaluates the information provided in the NDA/BLA, it issues an approval letter, or a complete response letter. A complete response letter outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed and the NDA/BLA has been resubmitted, the FDA will re-initiate review. If it is satisfied that the deficiencies have been addressed, the FDA will issue an approval letter.

Under the centralized procedure in the EU, the maximum timeframe for the evaluation of an MAA by the EMA is 210 days. This excludes so-called clock stops, during which additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP. At the end of the review period, the CHMP provides an opinion to the EC. If the opinion is favorable, the EC may then adopt a decision to grant marketing authorization. In the event of a negative opinion, the company may request a re-examination of the application within 15 days of receipt of the negative opinion. The company then has 60 days to provide the CHMP with detailed grounds for requesting the re-examination. Within 60 days of providing this information, the CHMP must re-examine its opinion. The EC follows the recommendation of the CHMP in almost all cases. In exceptional cases, the CHMP might perform an accelerated review of an MAA in no more than 150 days. This is usually when the product is of major interest from the point of view of public health and, in particular, from the viewpoint of therapeutic innovation.

During the review period, the FDA and/or the European authorities may typically inspect one or more clinical sites and/or the sponsor to assure compliance with GCP regulations and may equally inspect the facility or the facilities at which the drug is manufactured to ensure compliance with cGMPs regulations. Neither the FDA nor the EC will approve the product unless compliance is satisfactory and the application contains data that provide substantial evidence that the drug is safe and effective in the indication studied.

Fast Track Designation and Accelerated Approval

The FDA is required to facilitate the development and expedite the review of drugs that are intended for the treatment of a serious or life-threatening condition for which there is no effective treatment and that demonstrate the potential to address unmet medical needs for the condition. Under the FDA's fast track program, the sponsor of a new drug candidate may request that the FDA designate the drug candidate for a specific indication as a fast track drug concurrent with or after the filing of the IND for the drug candidate. The FDA must determine if the drug candidate qualifies for fast track designation within 60 days of receipt of the sponsor's request.

In addition to other benefits, such as the ability to use surrogate endpoints and have greater interactions with the FDA, the FDA may initiate review of sections of a fast track drug's NDA or BLA before the application is complete. This rolling review is available if the applicant provides and the FDA approves a schedule for the submission of the remaining information and the applicant pays applicable user fees. However, the FDA's time period goal for reviewing an application does not begin until the last section of the NDA or BLA is submitted. Additionally, the fast track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Under the fast track program and the FDA's accelerated approval regulations, the FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, 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. In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions, or survives. Surrogate endpoints can often be measured more easily or more

rapidly than clinical endpoints. A drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of a Phase 4 or post-approval clinical trial to confirm the effect on the clinical endpoint. Failure to conduct a required post-approval study or confirm a clinical benefit through a post-marketing study will allow the FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by the FDA. The Food and Drug Omnibus Reform Act (FDORA) added provisions related to the accelerated approval pathway. Pursuant to FDORA, the FDA is authorized to require a post-approval study to be underway prior to approval or within a specified time period following approval. FDORA also requires the FDA to specify conditions of any required post-approval study, which may include milestones such as a target date of study completion and requires sponsors to submit progress reports for required post-approval studies and any conditions required by the FDA not later than 180 days following approval and not less frequently than every 180 days thereafter until completion or termination of the study. FDORA enables the FDA to initiate enforcement action for the failure to conduct with due diligence a required post-approval study, including a failure to meet any required conditions specified by the FDA or to submit timely reports.

Breakthrough Therapy Designation

The FDA is also required to expedite the development and review of the application for approval of drugs that are intended to treat a serious or life-threatening disease or condition where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. Under the breakthrough therapy program, the sponsor of a new drug candidate may request that the FDA designate the drug candidate for a specific indication as a breakthrough therapy concurrent with, or after, the filing of the IND for the drug candidate. The FDA must determine if the drug candidate qualifies for breakthrough therapy designation within 60 days of receipt of the sponsor’s request.

Adaptive Pathways

The EMA has an adaptive pathways approach which allows for early and progressive patient access to a medicine in cases of high medical need. To achieve this goal, several approaches are envisaged including for example identifying small populations with severe disease where a medicine’s benefit-risk balance could be favorable or making more use of real-world data where appropriate to support clinical trial data. The adaptive pathways concept applies primarily to treatments in areas of high medical need where it is difficult to collect data via traditional routes and where large clinical trials would unnecessarily expose patients who are unlikely to benefit from the medicine. The approach builds on regulatory processes already in place within the existing EU legal framework. These include: scientific advice; compassionate use; the conditional MA; patient registries and other pharmacovigilance tools that allow collection of real-life data and development of a risk-management plan for each medicine.

A conditional MA may be granted prior to the submission of comprehensive clinical data if the benefit of the immediate availability on the market of the product is deemed to outweigh the risk inherent in the fact that additional data are still required. In emergency situations, a MA for such medicinal products may be granted also where comprehensive pre-clinical or pharmaceutical data have not been provided. Under this procedure a MA can be granted as soon as sufficient data becomes available to demonstrate that the drug's benefits outweigh its risks, with safeguards and controls in place post-authorization. This procedure can also be combined with a rolling review of data during the development of a promising medicine, to further expedite its evaluation. Conditional MAs are typically subject to obligations that are reviewed annually. These include the obligation to complete ongoing studies, or to conduct new studies, with a view to confirming that the risk-benefit balance is favorable. Conditional MAs are valid for one year and are renewable.

PRIME Program

The EMA launched its PRIME regulatory program to enhance support for the development of therapies that target an unmet medical need. The initiative focuses on drugs that may offer a major therapeutic advantage over existing treatments, or benefit patients with no treatment options. These therapies are considered priority medicines within the EU. Through PRIME, the EMA offers early, proactive and enhanced support to drug developers to optimize the generation of robust data on a therapy’s benefits and risks and enable accelerated assessment of drug applications.

Regulation of Approved Products

Product Marketing and Promotion

A marketing approval authorizes commercial marketing of the drug with specific prescribing information for specific indications. The FDA and European authorities closely regulate the post-approval marketing and promotion of commercial products, including standards and regulations for direct-to-consumer advertising (which is prohibited in the EU for prescription products such as our products), off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the Internet. For further detail, please see "Post-Approval Regulatory Requirements" below.

Regulation of Manufacturing Standards

The FDA as well as other regulatory agencies around the world, regulate and inspect the equipment, facilities, laboratories and processes used in the manufacturing and testing of products prior to granting approval to market products. If after receiving approval from the FDA and other agencies such as the EC we make a material change in manufacturing equipment, location or process, additional regulatory review and approval may be required. We also must adhere to cGMP regulations and product-specific regulations enforced by the FDA and other regulatory agencies through their facilities inspection program. The FDA and other regulatory agencies around the world conduct regular, periodic visits to reinspect our equipment, facilities, laboratories and processes following an initial approval.

Combination Products and Companion Diagnostics

Combination products are defined by the FDA as products composed of two or more regulated components (e.g., a biologic and/or drug and a device). Biologics/drugs and devices each have their own regulatory requirements, and combination products may have additional requirements. For example, in the EU, if a device intended to administer a medicinal product is sold together with such medicinal product in such a way that they form a single integral product which is intended exclusively for use in the given combination and which is not reusable, that single integral product is regulated as a medicinal product. In addition, the relevant general safety and performance requirements established for medical devices by EU medical devices legislation apply to the device component of such combination products. A number of our products qualify as combination products and are regulated under the applicable framework, and we expect that a number of our pipeline product candidates will be evaluated for regulatory approval under such framework as well.

If use of an *in vitro* diagnostic is essential to safe and effective use of a drug or biologic product, then the FDA generally will require approval or clearance of the diagnostic, known as a companion diagnostic, at the same time that the FDA approves the therapeutic product. The review of these *in vitro* companion diagnostics in conjunction with the review of a drug or biologic involves coordination of review by the FDA's Center for Drug Evaluation and Research or Center for Biologics Evaluation and Research and by the FDA's Center for Devices and Radiological Health. Approval and clearance of a companion diagnostic also requires a high level of coordination between the drug or biologic manufacturer and device manufacturer, if different companies. Most companion diagnostics require approval of a premarket approval application (PMA). The PMA process, including the gathering of clinical and preclinical data and the submission to and review by the FDA, can take several years or longer. PMA approval is not guaranteed, and the FDA may ultimately respond to a PMA submission with a not approvable determination based on deficiencies in the application and require additional clinical trial or other data that may be expensive and time-consuming to generate and that can substantially delay approval. After a device is placed on the market, it remains subject to significant regulatory requirements.

Post-Approval Regulatory Requirements

Following approval, the FDA and the regulatory authorities around the world will impose certain post-approval requirements related to a product. As a condition of NDA or BLA approval, the FDA may require a REMS, to help ensure that the benefits of the drug outweigh the potential risks. A REMS can include medication guides, communication plans for healthcare professionals, and elements to assure safe use, such as special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring and the use of patient registries. Moreover, product approval may require substantial post-approval testing and surveillance to monitor the drug's safety or efficacy. Similar rules apply outside of the U.S. For example, products approved in the EU may be subject to post-authorization requirements such as the obligation to perform post-authorization efficacy studies (PAES) or post-authorization safety studies (PASS) imposed as conditions to the MA, or other Risk Minimization Measures (RMMs), such as educational programs or controlled access programs, which may sometimes vary from one EU Member State to another. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing. Moreover, if a company obtains original approval for a product via an accelerated approval pathway, the company will be typically required to conduct a post-marketing confirmatory trial to verify and describe the clinical benefit in support of full approval. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of the marketing approval for a product.

Commercial products may be marketed only for the approved indications and in accordance with the provisions of the approved labeling. Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, may require a submission to and approval by the FDA or the EC, as applicable, before the change can be implemented. An NDA/BLA or MAA supplement for a new indication typically requires clinical data similar to that in the original application, and similar procedures and actions in reviewing NDA/BLA or MAA supplements as in reviewing NDAs/BLAs and MAAs.

Adverse event reporting and submission of periodic reports is required following marketing approval. Either the FDA or the EC/EMA may also require post-marketing testing, known as Phase 4 testing, a risk evaluation and mitigation strategy, and surveillance to monitor the effects of an approved product or place conditions on an approval that could restrict the distribution or use of the product. In addition, quality control as well as the manufacture, packaging, and labeling procedures must continue to conform to cGMPs after approval. Drug and biological product manufacturers and certain of their subcontractors are subject to periodic unannounced inspections by the FDA, the EMA/NCAs, during which the inspectors audit manufacturing facilities to assess

compliance with cGMPs. Regulatory authorities may withdraw product approvals or request product recalls if a company fails to comply with regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered. In addition, prescription drug manufacturers in the U.S. must comply with applicable provisions of the Drug Supply Chain Security Act and provide and receive product tracing information, maintain appropriate licenses, ensure they only work with other properly licensed entities and have procedures in place to identify and properly handle suspect and illegitimate products. Similarly, in the EU, stringent rules have been introduced to fight medicine falsifications and to ensure that the trade in medicines is subject to rigorous controls. Measures required to ensure that include: a unique identifier and an anti-tampering device on the outer packaging of drugs, stringent rules on import of active pharmaceutical ingredients and record-keeping requirements for wholesale distributors.

Approval Regulation Outside of the U.S. and the EU

For marketing outside the U.S. and the EU, we are subject to non-U.S. regulatory requirements governing human clinical testing and marketing approval for our products. These requirements vary by jurisdiction, can differ from those in the U.S. and the EU and may require us to perform additional preclinical or clinical testing. The amount of time required to obtain necessary approvals may be longer or shorter than that required for FDA or EC approval. In many countries outside of the U.S., approvals for pricing, coverage and reimbursement offered by third-party payers, including government payers and private insurance plans, are also required.

Other Regulation

Exclusivity for Biologics in the U.S.

The Biologics Price Competition and Innovation Act of 2009 (BPCIA), which was enacted as part of the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 (as amended, the PPACA), created an abbreviated approval pathway for biological products that are demonstrated to be “biosimilar” to or “interchangeable” with an FDA-licensed reference biological product. Biosimilars are licensed based on FDA’s findings of safety, purity, and potency for a prior FDA-licensed product called a reference product. There must be no differences in conditions of use, route of administration, dosage form, and strength to rely on a given reference product, and there can be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency. Biosimilarity must be shown through analytical studies, animal studies, and at least one comparative clinical study, absent a waiver from the FDA. A biosimilar also may meet the higher hurdle of interchangeability such that it can be substituted for a reference product without the intervention of the prescribing health care provider. For licensure as an interchangeable biosimilar, a sponsor must demonstrate that the biosimilar product can be expected to produce the same clinical result as the reference product in any given patient, and for a product that is administered more than once to an individual, that the risk of switching in terms of safety or diminished efficacy of alternating or switching between the reference product and biosimilar product is not greater than the risk of maintaining the patient on the reference product. The first biosimilar product was approved under the BPCIA in 2015, and the first interchangeable product was approved in 2021. Complexities associated with the larger, and often more complex, structures of biological products, as well as the process by which such products are manufactured, pose significant hurdles to implementation that are still being evaluated by the FDA. A reference biologic is granted 12 years of data exclusivity from the time of first licensure of the reference product during which no biosimilar referencing such biologic can be licensed by FDA, and no such biosimilar application relying on the reference product can be submitted for four years from the date of first licensure of the reference product. The first biologic product submitted under the abbreviated approval pathway that is determined to be interchangeable with the reference product is eligible for exclusivity precluding marketing of interchangeable biosimilars referencing the same reference product for the lesser of (i) one year after first commercial marketing of the first interchangeable biosimilar to be approved, (ii) eighteen months after the first interchangeable biosimilar is approved if there is not patent challenge, (iii) eighteen months after resolution of a lawsuit over the patents of the reference biologic in favor of the first interchangeable biosimilar applicant, or (iv) 42 months after the first interchangeable biosimilar’s application has been approved if the interchangeable applicant has been sued under the BPCIA and any related patent litigation is ongoing within the 42-month period.

Data Exclusivity and Market Exclusivity in the EU

The EU provides opportunities for market and data exclusivity for all products containing a New Active Substance, or NAS (such as a chemical, biological or radiopharmaceutical substance not previously authorized as a medicinal product in the EU), which have been granted an MA. These products receive eight years of data exclusivity and an additional two years of market exclusivity. The data exclusivity period prevents generic or biosimilar applicants from relying on the pre-clinical and clinical trial data contained in the dossier of the reference product when applying for a generic or biosimilar MA in the EU during a period of eight years from the date on which the reference product was first authorized in the EU. The market exclusivity period prevents a successful generic or biosimilar applicant from commercializing its product in the EU until ten years have elapsed from the initial MA of the reference product in the EU. The overall ten-year market exclusivity period can be extended to a maximum of eleven years if, during the first eight years of those ten years, the MA holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies.

Orphan Drug Designation

Orphan drug designation is granted by the FDA and the EC to drugs intended to treat a rare disease or condition, which in the U.S. is defined as having a prevalence of less than 200,000 individuals in the U.S. or as a condition that affects more than 200,000 individuals in the U.S. and for which there is no reasonable expectation that the costs of development of said drug will be recovered from sales in the U.S. In the EU, orphan drug designation is available if a sponsor can establish: that the medicine is intended for the diagnosis, prevention or treatment of (1) a life-threatening or chronically debilitating condition affecting no more than five in 10,000 people in the EU, which is equivalent to around 250,000 people or fewer, or (2) a life-threatening, seriously debilitating or serious and chronic condition in the EU and that without incentives derived from the orphan status, it is unlikely that the marketing of the medicinal product in the EU would generate sufficient return to justify the necessary investment. For either of these criteria, the applicant must demonstrate that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the EU or, if such method exists, the medicinal product will be of significant benefit to those affected by that condition. Orphan drug designation must be requested before submitting a marketing application and, in the EU, it must be maintained until the time of the granting of the MA. Orphan designation is indeed lost in the EU if it is established that the product no longer meets the orphan criteria at the time a MA is granted for such product.

Orphan drug designation does not shorten the regulatory review and approval process. However, if an orphan drug later receives approval for the indication for which it has designation, the relevant regulatory authority may not approve any other applications to market the same drug for the same condition, except in limited circumstances, for seven years in the U.S. and ten years in the EU (extendable to twelve years for medicines that have complied with an agreed Pediatric Investigation Plan (PIP) pursuant to Regulation 1901/2006) and, in addition, a range of other benefits during the development and regulatory review process are available in the EU, including scientific assistance for study protocols, authorization through the centralized marketing authorization procedure covering all member countries and a reduction or elimination of registration and marketing authorization fees. Among the benefits of orphan drug designation in the U.S. are tax credits for certain research and a waiver of the NDA/BLA application user fee. Orphan drug exclusive marketing rights may be lost under certain conditions, such as if the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug. A competitor may also demonstrate that its proposed product is “clinically superior” to a product with orphan drug exclusivity, allowing for approval and market entry of the same drug for the same condition during the first product’s orphan drug exclusivity period. In the EU, a MA may be granted to a similar medicinal product with the same orphan indication during the regulatory exclusivity period with the consent of the MA holder for the original orphan medicinal product or if the MA holder of the original orphan medicinal product is unable to supply sufficient quantities. A MA may also be granted to a similar medicinal product with the same orphan indication if the second applicant can establish that its medicinal product is safer, more effective or otherwise clinically superior to the original orphan medicinal product. The period of market exclusivity may, in addition, be reduced to six years if, at the end of the fifth year, it can be demonstrated on the basis of available evidence that the criteria for its designation as an orphan medicine are no longer satisfied, for example if the original orphan medicinal product has become sufficiently profitable not to justify maintenance of market exclusivity.

Healthcare Reform

The U.S. federal and state governments continue to propose and pass legislation designed to regulate the healthcare industry, including legislation that seeks to directly or indirectly regulate pharmaceutical drug pricing. For more information, see Item 1A. Risk Factors “Government healthcare reform could increase our costs and adversely affect our revenue and results of operations.”

In addition, in the EU, EMA, the EC and other comparable regulatory authorities continue to propose and pass legislation and issue additional guidelines that may affect the applicable legislative framework. In particular, the EU pharmaceutical legislation is currently the subject of a review process, in the context of the Pharmaceutical Strategy for Europe initiative, launched by the EC in November 2020. The EC’s proposal for a revision of several legislative instruments related to medicinal products (potentially reducing the duration of regulatory exclusivity, revising the eligibility for expedited pathways, etc.) was published on April 26, 2023. The proposed revisions remain to be agreed and adopted by the European Parliament and European Council. The proposals may be substantially revised before adoption. The revisions may, however, have a significant impact on our activities in the long term.

Other Regulatory Requirements

In addition to FDA restrictions on marketing of pharmaceutical products, several other types of state and federal laws have been applied to restrict certain business and marketing practices in the pharmaceutical industry in recent years. These laws include anti-kickback, false claims, patient data privacy and security, and transparency statutes and regulations.

The federal Anti-Kickback Statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. The PPACA amended the intent requirement of the federal Anti-Kickback and certain other criminal healthcare fraud statutes such that a person or entity no longer needs to have actual knowledge of these statutes or specific intent to violate them in order to commit a violation. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and

prescribers, purchasers and formulary managers on the other. Although there are a number of statutory exceptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exceptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exception or safe harbor.

Federal false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. The PPACA amended the statute so that 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 false claims laws. Recently, several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly inflating drug prices they report to pricing services, which in turn are used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws.

The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for, among other things, executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and their implementing regulations, also imposes obligations, including mandatory contractual terms, on certain types of individuals and entities, with respect to safeguarding the privacy, security and transmission of individually identifiable health information.

The federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services (CMS) information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), physician assistants, certain types of advanced practice nurses and teaching hospitals, and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by the physicians and their immediate family members.

The majority of states also have statutes or regulations similar to the federal Anti-Kickback Statute 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 now require pharmaceutical companies to report expenses relating to the marketing and promotion of pharmaceutical products and to report gifts and payments to individual physicians in these states while other states prohibit various other marketing-related activities. Other states require submission or disclosure of certain pricing information. Still other states require the posting of information relating to clinical studies and their outcomes. In addition, states including California, Connecticut, Nevada and Massachusetts require pharmaceutical companies to implement compliance programs or marketing codes and additional states are considering similar proposals. Compliance with these laws is difficult and time consuming, and companies that do not comply with these state laws face civil penalties. Sanctions under these federal and state laws may include significant penalties, including administrative and criminal sanctions, civil monetary penalties, damages, monetary fines, disgorgement, exclusion of a company from federal healthcare programs, integrity oversight and reporting obligations, criminal fines, contractual damages, reputational harm, diminished profits and future earnings, curtailment of operations and imprisonment.

The U.S. Foreign Corrupt Practices Act (FCPA), to which we are subject, prohibits corporations and individuals from engaging in certain activities to obtain or retain business or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any non-U.S. government official, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity. Similar laws exist in other countries, such as the U.K., that restrict improper payments to public and private parties. Many countries have laws prohibiting these types of payments within the respective country. In the EU, for example, harmonized rules prohibit gifts, pecuniary advantages or benefits in kind to Health Care Professionals (HCPs) unless they are inexpensive and relevant to the practice of medicine or pharmacy. Similarly, strict rules apply to hospitality at sales promotion events. Based on these rules, a body of industry guidelines and sometimes national laws in force in individual EU Member States has been introduced to fight improper payments or other transfers of value to HCPs, and in general inducements that may have a broadly promotional character. Historically, pharmaceutical companies have been the target of FCPA and other anti-corruption and similar investigations, as well as of wide media attention, sometimes resulting in significant penalties, image and other costs for such companies.

Pricing and Reimbursement

Because the course of treatment for patients using our products is expensive, sales of our products depend, in significant part, on the availability and extent of coverage and reimbursement offered by third-party payers, including government payers and private insurance plans. Governments may regulate access to, prices of or reimbursement levels for our products to control costs or to affect levels of use of our products, and private insurers may be influenced by government reimbursement methodologies.

Third-party payers carefully review and increasingly challenge the prices charged for drugs, examine their medical necessity, and review their cost effectiveness. Reimbursement rates from private companies vary depending on the third-party

payer, the insurance plan and other factors. One payer's determination to provide coverage for a product does not assure that other payers will also provide coverage for the product. Moreover, the process for determining whether a third-party payer will provide coverage for a product may be separate from the process for setting the price of a product or for establishing the reimbursement rate that such a payer will pay for the product. Obtaining coverage and adequate reimbursement for our products may be particularly difficult because of the higher prices often associated with drugs administered under the supervision of a physician. A payer's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable us to maintain high enough price levels to realize sufficient revenues from our investment in product development. In addition, emphasis on managed care in the U.S. has increased and we expect will continue to increase the pressure on pharmaceutical pricing. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we or our collaborators receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Outside of the U.S. our products are paid for by a variety of payers, with governments being the primary source of payment. Reimbursement in the EU and many other territories must be negotiated on a country-by-country basis and in many countries the product cannot be commercially launched until pricing and/or reimbursement is approved. In many countries the government closely regulates drug pricing and reimbursement and often has a significant discretion in determining whether a product will be reimbursed at all and, if it is, how much will be paid. Negotiating prices with governmental authorities can delay commercialization of our products. Payers in many countries use a variety of cost-containment measures that can include referencing prices in other countries and using those reference prices to set their own price, mandatory price cuts and rebates. This international patchwork of price regulation has led to different prices across countries and some cross-border trade in our products from markets with lower prices. Even after a price is negotiated, countries frequently request or require adjustments to the price and other concessions over time.

Government Pricing and Reimbursement Programs for Marketed Drugs in the U.S.

Medicaid, the 340B Drug Pricing Program, and Medicare

Federal law requires that a pharmaceutical manufacturer, as a condition of having its products receive federal reimbursement under Medicaid and Medicare Part B, must pay rebates to state Medicaid programs for all units of its covered outpatient drugs dispensed to Medicaid beneficiaries and paid for by a state Medicaid program under either a fee-for-service arrangement or through a managed care organization. This federal requirement is effectuated through a Medicaid drug rebate agreement between the manufacturer and the Secretary of Health and Human Services. CMS administers the Medicaid drug rebate agreements, which provide, among other things, that the drug manufacturer will pay rebates to each state Medicaid agency on a quarterly basis and report certain price information on a monthly and quarterly basis. The rebates are based on prices reported to CMS by manufacturers for their covered outpatient drugs. For non-innovator products, generally generic drugs marketed under abbreviated new drug applications (referred to as ANDAs), the rebate amount is 13% of the average manufacturer price (AMP) for the quarter. The AMP is the weighted average of prices paid to the manufacturer (1) directly by retail community pharmacies and (2) by wholesalers for drugs distributed to retail community pharmacies. For innovator products (i.e., drugs that are marketed under NDAs or BLAs), the rebate amount is the greater of 23.1% of the AMP for the quarter or the difference between such AMP and the best price for that same quarter. The best price is essentially the lowest price available to non-governmental entities. Innovator products may also be subject to an additional rebate that is based on the amount, if any, by which the product's AMP for a given quarter exceeds the inflation-adjusted baseline AMP, which for most drugs is the AMP for the first full quarter after launch. Since 2017, non-innovator products are also subject to an additional rebate. To date, the rebate amount for a drug has been capped at 100% of the AMP; however, effective January 1, 2024, this cap was eliminated, which means that a manufacturer could pay a rebate amount on a unit of the drug that is greater than the average price the manufacturer receives for the drug.

The terms of participation in the Medicaid drug rebate program impose an obligation to correct the prices reported in previous quarters, as may be necessary. Any such corrections could result in additional or lesser rebate liability, depending on the direction of the correction. In addition to retroactive rebates, if a manufacturer were found to have knowingly submitted false information to the government, federal law provides for civil monetary penalties for failing to provide required information, late submission of required information, and false information.

A manufacturer must also participate in a federal program known as the 340B drug pricing program in order for federal funds to be available to pay for the manufacturer's drugs and biological products under Medicaid and Medicare Part B. Under this program, the participating manufacturer agrees to charge certain safety net healthcare providers no more than an established discounted price for its covered outpatient drugs. The formula for determining the discounted price is defined by statute and is based on the AMP and the unit rebate amount as calculated under the Medicaid drug rebate program, discussed above. Manufacturers are required to report pricing information to the Health Resources and Services Administration (HRSA) on a quarterly basis. HRSA has also issued regulations relating to the calculation of the ceiling price as well as imposition of civil monetary penalties for each instance of knowingly and intentionally overcharging a 340B covered entity. There is ongoing litigation that may restrict the number of third-party contract pharmacies that can dispense drugs that manufacturers sell to 340B covered entities and who qualifies as patients of these 340B covered entities. The outcome of this litigation may change the scope of the 340B program in coming years.

Federal law also requires that manufacturers report data on a quarterly basis to CMS regarding the pricing of drugs that are separately reimbursable under Medicare Part B. These are generally drugs, such as injectable products, that are administered “incident to” a physician service and are not generally self-administered. The pricing information submitted by manufacturers is the basis for reimbursement to physicians and suppliers for drugs covered under Medicare Part B. Under the Inflation Reduction Act (IRA), manufacturers are also required to provide quarterly rebates for certain single-source drugs and biologics (including biosimilars) covered under Medicare Part B with prices that increase faster than the rate of inflation. This requirement started on January 1, 2023, for drugs approved on or before December 1, 2020, and begins six quarters after a drug is first marketed for all other drugs. As with the Medicaid drug rebate program, federal law provides for civil monetary penalties for failing to provide required information, late submission of required information, and false information.

Medicare Part D provides prescription drug benefits for seniors and people with disabilities. Medicare Part D enrollees once had a gap in their coverage (between the initial coverage limit and the point at which catastrophic coverage begins) where Medicare did not cover their prescription drug costs, known as the coverage gap. However, beginning in 2019, Medicare Part D enrollees paid 25% of brand drug costs after they reached the initial coverage limit - the same percentage they were responsible for before they reached that limit - thereby closing the coverage gap from the enrollee’s point of view. Most of the cost of closing the coverage gap is being borne by innovator companies and the government through subsidies. Each manufacturer of drugs approved under NDAs or BLAs is required to enter into a Medicare Part D coverage gap discount agreement and provide a 70% discount on those drugs dispensed to Medicare Part D enrollees in the coverage gap, in order for its drugs to be reimbursed by Medicare Part D. Beginning in 2025, the IRA eliminates the coverage gap under Medicare Part D by significantly lowering the enrollee maximum out-of-pocket cost and requiring manufacturers to subsidize, through a newly established manufacturer discount program, 10% of Part D enrollees’ prescription costs for brand drugs above a deductible and below the out-of-pocket maximum, and 20% once the out-of-pocket maximum has been reached. Although these discounts represent a lower percentage of enrollees’ costs than the current discounts required below the out-of-pocket maximum (that is, in the coverage gap phase of Part D coverage), the new manufacturer contribution required above the out-of-pocket maximum could be considerable for very high-cost patients and the total contributions by manufacturers to a Part D enrollee’s drug expenses may exceed those currently provided. The IRA also requires manufacturers to provide annual Medicare Part D rebates for single-source drugs and biological products with prices that increase faster than the rate of inflation.

The IRA also allows the U.S. Department of Health and Human Services (HHS) to directly negotiate the selling price of a statutorily specified number of drugs and biologics each year that CMS reimburses under Medicare Part B and Part D. Only high-expenditure single-source drugs that have been approved for at least 7 years (11 years for biologics) can qualify for negotiation, with the negotiated price taking effect two years after the selection year. Negotiations for Medicare Part D products begin in 2024 with the negotiated price taking effect in 2026, and negotiations for Medicare Part B products begin in 2026 with the negotiated price taking effect in 2028. In August 2023, HHS announced the ten Medicare Part D drugs and biologics that it selected for negotiations, and by October 1, 2023, each manufacturer of the selected drugs signed a manufacturer agreement to participate in the negotiations. HHS will announce the negotiated maximum fair price by September 1, 2024, and this price cap, which cannot exceed a statutory ceiling price, will come into effect on January 1, 2026. A drug or biological product that has an orphan drug designation for only one rare disease or condition will be excluded from the IRA's price negotiations requirements, but loses that exclusion if it has designations for more than one rare disease or condition, or if is approved for an indication that is not within that single designated rare disease or condition, unless such additional designation or such disqualifying approvals are withdrawn by the time CMS evaluates the drug for selection for negotiation.

U.S. Federal Contracting and Pricing Requirements

Manufacturers are also required to make their covered drugs, which are generally drugs approved under NDAs or BLAs, available to authorized users of the Federal Supply Schedule (FSS) of the General Services Administration. The law also requires manufacturers to offer deeply discounted FSS contract pricing for purchases of their covered drugs by the Department of Veterans Affairs, the Department of Defense, the Coast Guard, and the Public Health Service (including the Indian Health Service) in order for federal funding to be available for reimbursement or purchase of the manufacturer’s drugs under certain federal programs. FSS pricing to those four federal agencies for covered drugs must be no more than the Federal Ceiling Price (FCP), which is at least 24% below the Non-Federal Average Manufacturer Price (Non-FAMP) for the prior year. The Non-FAMP is the average price for covered drugs sold to wholesalers or other middlemen, net of any price reductions.

The accuracy of a manufacturer’s reported Non-FAMPs, FCPs, or FSS contract prices may be audited by the government. Among the remedies available to the government for inaccuracies is recoupment of any overcharges to the four specified federal agencies based on those inaccuracies. If a manufacturer were found to have knowingly reported false prices, in addition to other penalties available to the government, the law provides for significant civil monetary penalties per incorrect item. Finally, manufacturers are required to disclose in FSS contract proposals all commercial pricing that is equal to or less than the proposed FSS pricing, and subsequent to award of an FSS contract, manufacturers are required to monitor certain commercial price reductions and extend commensurate price reductions to the government, under the terms of the FSS contract Price Reductions Clause. Among the remedies available to the government for any failure to properly disclose commercial pricing and/or to extend FSS contract price reductions is recoupment of any FSS overcharges that may result from such omissions.

Disclosure of Clinical Trial Information

Sponsors of clinical trials of FDA-regulated products, including drugs and biologics, are required to register and disclose certain clinical trial information. Information related to the product, patient population, phase of investigation, study sites and investigators, and other aspects of the clinical trial are then made public as part of the registration. Sponsors are also obligated to discuss the results of their clinical trials after completion. In certain circumstances, disclosure of the results of these trials can be delayed for up to two years after the date of completion of the trial. Competitors may use this publicly available information to gain knowledge regarding the progress of development programs. In the EU there is an increasing trend requiring public disclosure of development data, in particular clinical trial data. These data were traditionally regarded as Confidential Commercial Information (CCI); however, under policies adopted in the EU, clinical study data submitted to the EMA in MAAs, including preclinical data, and patient level data, may be subject to public disclosure. This is confirmed in the CTR, the new EU legislation on clinical trials, according to which clinical trial applications and all the related documentation are uploaded and stored in the Clinical Trials Information System (CTIS) which is managed by the EMA. Confirming the transparency principle, the CTR provides that the information stored in such system is publicly accessible unless confidentiality is justified on the basis of a limited set of exceptions. These exceptions, which are to be interpreted narrowly in the EU, include the protection of CCI, in particular through taking into account the status of the MA for the applicable product; however, CCI is overridden in those cases where the authorities conclude that there is an overriding public interest in disclosure. Case law of the Court of Justice of the EU has also confirmed the absence of a general presumption of confidentiality over documents containing clinical and preclinical data provided to the EMA in support of a MAA.

Pediatric Indications

In the U.S., under the Pediatric Research Equity Act of 2007 (PREA), most NDAs or BLAs or supplements to NDAs or BLAs must contain data to assess the safety and effectiveness of the drug for the claimed indication(s) in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the drug is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by statute or regulation, PREA does not apply to any drug for an indication for which orphan drug designation has been granted; the orphan drug exemption, however, does not apply where the product is a molecularly-targeted oncology drug. The Best Pharmaceuticals for Children Act (BPCA) provides sponsors of NDAs with an additional six-month period of market exclusivity for all unexpired patent or non-patent exclusivity on all forms of the drug containing the active moiety if the sponsor submits results of pediatric studies specifically requested by the FDA under BPCA within required timeframes. The BPCIA provides sponsors of BLAs an additional six-month extension for all unexpired non-patent market exclusivity on all forms of the biological containing the active moiety pursuant to the BPCA if the conditions under the BPCA are met.

In the EU, companies developing a new medicinal product must agree to a PIP with the EMA and must conduct pediatric clinical trials in accordance with that PIP, unless a deferral or waiver is granted by the EMA on request by the applicant (e.g., because the relevant disease or condition occurs only in adults). The PIP requirement also applies when a MA holder intends to add a new indication, pharmaceutical form or route of administration for a medicinal product that has already been authorized. The MAA for the product must include the results of pediatric clinical trials conducted in accordance with the PIP, unless a waiver applies, or a deferral has been granted, in which case the pediatric clinical trials must be completed at a later date. Once all the studies and measures agreed have been conducted in accordance with the PIP, products are eligible for a six -month extension of the protection under a supplementary protection certificate (if any is in effect at the time of approval) or, in the case of orphan medicinal products, a two -year extension of the orphan market exclusivity. This pediatric reward is granted subject to specific conditions. These conditions include that the applicant demonstrates having complied with all the measures contained in the PIP, that the summary of product characteristics, and if appropriate the package leaflet, reflects the results of studies conducted in compliance with such PIP, and that the product is authorized in all Member States. The rewards for conducting studies in the pediatric population can be granted irrespective of the fact that the information generated in compliance with the agreed PIP fails to lead to the authorization of a pediatric indication.

Privacy and Security Legislation

In the ordinary course of our business, we may process personal or sensitive data. Accordingly, we are, or may become, subject to numerous data privacy and security obligations, including federal, state, local, and foreign laws, regulations, guidance, and industry standards related to data privacy, security, and protection. Such obligations may include, without limitation, the Federal Trade Commission Act, the California Consumer Privacy Act of 2018 (CCPA), the Canadian Personal Information Protection and Electronic Documents Act, the EU's General Data Protection Regulation 2016/679 (EU GDPR), the EU GDPR as it forms part of United Kingdom (UK) law by virtue of section 3 of the European Union (Withdrawal) Act 2018 (UK GDPR).

The legislative and regulatory environments regarding privacy and data protection are continually evolving and developing, in response to increasing global attention. In the U.S., for example, we are subject to the CCPA along with the California Privacy Rights Act of 2020 (CPRA). The CCPA imposes obligations on covered businesses to provide specific disclosures related to a business's collecting, using, and disclosing personal data and to respond to certain requests from California residents related to their personal data (for example, requests to know of the business's personal data processing activities, to delete the individual's personal data, and to opt out of certain personal data disclosures). Also, the CCPA provides for

civil penalties and a private right of action for data breaches which may include an award of statutory damages. In addition, the CPRA, effective January 1, 2023, expanded the CCPA. The CPRA, among other things, gives California residents the ability to limit use of certain sensitive personal data, establish restrictions on personal data retention, expands the types of data breaches that are subject to the CCPA's private right of action, and establishes a new California Privacy Protection Agency to implement and enforce the new law.

Other jurisdictions where we operate have enacted or proposed similar legislation and/or regulations. Several states within the United States have enacted or proposed data privacy laws. Additionally, we are, or may become, subject to various U.S. federal and state consumer protection laws which require us to publish statements that accurately and fairly describe how we handle personal data and choices individuals may have about the way we handle their personal data.

We are also subject to the EU's General Data Protection Regulation GDPR, which requires that personal data is only collected for specified, explicit and legal purposes as set out in the GDPR or local laws, and the data may then only be processed in a manner consistent with those purposes. The personal data collected and processed must be adequate, relevant and not excessive in relation to the purposes for which it is collected and processed, it must be held securely, not transferred outside of the EEA (unless certain steps are taken to ensure an adequate level of protection), and must not be retained for longer than necessary for the purposes for which it was collected. The GDPR also requires companies processing personal data to implement adequate technical measures in order to ensure the most appropriate level of security which may vary depending on different factors such as the categories of processed personal data, the state of the art, the costs of implementation and the nature, scope, context and purposes of processing as well as the risk of varying likelihood and severity for the rights and freedoms of natural persons. In addition, the GDPR requires companies processing personal data to take certain organizational steps to ensure that they have adequate records, policies, security, training and governance frameworks in place to ensure the protection of data subject rights, including as required to respond to complaints and requests from data subjects. For example, the GDPR requires us to make more detailed disclosures to data subjects, requires disclosure of the legal basis on which we can process personal data, provides for conditions under which a valid consent for processing can be obtained, requires the appointment of a data protection officer where sensitive personal data (i.e., health data) is processed on a large scale, imposes mandatory data breach notification throughout the EEA and imposes additional obligations when contracting with service providers or partners. In addition, to the extent a company processes, controls or otherwise uses "special category" of personal data (including patients' health or medical information, genetic information and biometric information), more stringent rules apply, further limiting the circumstances and the manner in which a company is legally permitted to process that data.

Human Capital

As of December 31, 2023, we had 3,401 employees worldwide, of whom 1,509 were in operations, 807 were in research and development, 535 were in sales and marketing and 550 were in administration. Of the 3,401 employees as of December 31, 2023, 2,282 employees were in the U.S. and Canada, and 1,119 employees were in outside of North America, including 902 in Europe and the Middle East, 141 in Latin America and 76 in Asia Pacific. We also leverage temporary workers to fill short-term positions for our business and manufacturing needs. A significant portion of our employee base in the U.S. and Ireland works onsite supporting manufacturing and laboratory operations.

Diversity, Equity and Inclusion

At BioMarin, prejudice, racism and intolerance are unacceptable. We are committed to diversity, equity and inclusion (DEI) across all aspects of our organization, including hiring, promotion and development practices. At the direction of BioMarin's senior leadership team, our human resources department has implemented policies and programs to foster DEI at all levels of the organization. In addition, the Corporate Governance and Nominating and Compensation Committees of our Board of Directors regularly receive reports on our DEI policies and programs and offer valuable insights and recommendations to management in addition to providing appropriate oversight.

As of December 31, 2023, racial and ethnic minorities represented 49% of our employees in the U.S. Globally, 51% of our workforce were women and 52% of our positions at director-level and above were held by women. We are committed to continuing our ongoing efforts to ensure diversity in all positions, including leadership.

Equality, inclusiveness and belonging are central to BioMarin's culture, and we work to make our company a place where every employee feels heard, respected and valued. We believe encouraging and incorporating ideas from employees with different backgrounds helps us better serve our patients, achieve our business goals and objectives and provide employees with a fulfilling work experience. Since 2020, BioMarin's DEI Employee Advisory Committees have helped to define our DEI roadmap and ensure that perspectives from employees of different age, gender, sexual orientation, race, ethnicity, tenure, level and location are considered in how we build the most inclusive environment. We also continue to support our employee resource groups that build community for employees from underrepresented populations. Our foundational DEI training is a pillar of our DEI strategy and is required for all employees, and we offer opportunities for advanced DEI training for all employees as well. In addition, we provide mentorship and leadership development programs, including programs designed specifically for underrepresented employees.

We are honored to be recognized as a company of choice. In 2023 we were recognized for the third year in a row as a Best Place to Work for lesbian, gay, bisexual, transgender and queer (LGBTQ+) equality by the Human Rights Campaign, scoring 100% on their Corporate Equality Index, one of the foremost benchmarking surveys and reports in the U.S. measuring corporate policies and practices related to LGBTQ+ workplace equality.

Compensation, Benefits and Well-being

We offer competitive compensation and benefits in order to attract and retain excellent employees and support their overall well-being. Our total rewards compensation package includes market-competitive salary, the potential to earn bonuses or sales commissions, equity, healthcare benefits, retirement savings plans, paid time off and family leave, wellness programs such as subsidized access to fitness centers and onsite fitness facilities, free flu vaccinations and an Employee Assistance Program and other mental health services.

We believe employees should be paid for the value of their work, regardless of race, ethnicity, gender or other protected characteristics. To this end, we benchmark and tie compensation to market data as well as to an employee's experience, function, and performance. We regularly review our workforce compensation practices and strive for equity. Specifically, we partner with independent, third-party experts to conduct a regular and detailed pay equity assessment to determine whether gender and race/ethnicity have a significant impact on pay levels across the organization. This pay equity analysis is conducted on an employee's total compensation, including base pay, bonus and equity. If we identify any pay gap across the organization, we typically make adjustments to mitigate such gaps. Our managers also receive training in how to recognize and prevent discrimination in hiring, performance management and compensation decisions.

Professional Growth and Development

We help our employees develop the skills and capabilities to support BioMarin's growth and innovation. We continually invest in our employees' career growth and provide them with a wide range of development opportunities, including face-to-face, virtual and self-directed learning, mentoring, mobile coaching and external development. We offer our employees career-specific training and resources and support development opportunities through company sponsored programs in addition to our tuition reimbursement program. We also provide our high-potential employees with a variety of leadership coaching and management programs.

Patient and Community Connections

We are striving to support our local communities around the world by developing programs that inspire and enrich both our patient populations and the areas where we live and work. We actively engage with underrepresented populations through a variety of outreach and programs. We collaborate with Biotech Partners, a non-profit organization in the San Francisco Bay Area focused on helping students who are underrepresented in the biotechnology field to gain experience through classroom instruction and paid internships. We also partner with Health Career Connection, a national non-profit that prepares the next generation of diverse, transformational health, equity and racial justice leaders by providing promising undergraduate college students from underrepresented backgrounds and under-resourced communities with paid internship programs, health equity scholars programs and alumni professional development initiatives. In addition, we award annual scholarships to students living with rare disease through our Rare Scholars program.

Other Information

We were incorporated in Delaware in October 1996. Our principal executive offices are located at 770 Lindaro Street, San Rafael, California 94901 and our telephone number is (415) 506-6700. Our annual reports on Form 10-K, quarterly reports on Form 10-Q, proxy statements, current reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended (the Exchange Act) are available free of charge at www.bmrn.com as soon as reasonably practicable after electronically filing such reports with the Security and Exchange Commission (the SEC). Such reports and other information may be accessed through the SEC's website at www.sec.gov. Information contained in our website is not part of this or any other report that we file with or furnish to the SEC.

Item 1A. Risk Factors

An investment in our securities involves a high degree of risk. We operate in a dynamic and rapidly changing industry that involves numerous risks and uncertainties. The risks and uncertainties described below are not the only ones we face. Other risks and uncertainties, including those that we do not currently consider material, may impair our business. If any of the risks discussed below actually occur, our business, financial condition, operating results or cash flows could be materially adversely affected. This could cause the value of our securities to decline, and you may lose all or part of your investment.

Business and Operational Risks

If we fail to obtain and maintain an adequate level of coverage and reimbursement for our products by third-party payers, the sales of our products would be adversely affected or there may be no commercially viable markets for our products.

The course of treatment for patients using our products is expensive. For all our products except ROCTAVIAN, we expect patients to need treatment for extended periods, and for some products throughout the lifetimes of the patients. We expect that most families of patients will not be capable of paying for this treatment themselves. There will be no commercially viable market for our products without coverage and reimbursement from third-party payers. Additionally, even if there is a commercially viable market, if the level of reimbursement is below our expectations, our revenues and gross margin will be adversely affected.

Third-party payers, such as government or private healthcare insurers, carefully review and increasingly challenge the prices charged for drugs. Reimbursement rates from private companies vary depending on the third-party payer, the insurance plan and other factors. Obtaining coverage and adequate reimbursement for our products may be particularly difficult because of the higher prices often associated with drugs administered under the supervision of a physician. Reimbursement systems in international markets vary significantly by country and by region, and reimbursement approvals must be obtained on a country-by-country basis.

Government authorities and other third-party payers are developing increasingly sophisticated methods of controlling healthcare costs, such as by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payers are requiring that drug companies provide them with predetermined discounts from list prices as a condition of coverage, are using restrictive formularies and preferred drug lists to leverage greater discounts in competitive classes, and are challenging the prices charged for medical products. Further, no uniform policy requirement for coverage and reimbursement for drug products exists among third-party payers in the U.S. Therefore, coverage and reimbursement for drug products can differ significantly from payer to payer. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payer separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

We cannot be sure that coverage and reimbursement will be available for any product that we commercialize or will continue to be available for any product that we have commercialized and, if reimbursement is available, what the level of reimbursement will be. 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 based on new legislation, the availability of alternative therapies and their pricing, coverage and reimbursement decisions by third-party payers, or other factors. Coverage and reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. If coverage and reimbursement are not available or reimbursement is available only to limited levels, we may not successfully commercialize any product candidate for which we obtain marketing approval or continue to market any product that has already been commercialized.

Reimbursement in the European Union (EU) and many other territories must be negotiated on a country-by-country basis and in many countries the product cannot be commercially launched until pricing and/or reimbursement is approved. The timing to complete the negotiation process in each country is highly uncertain, and in some countries, we expect that it will exceed 12 months. Even after a price is negotiated, countries frequently request or require reductions to the price and other concessions over time.

For our future products, we will not know what the reimbursement rates will be until we are ready to market the product and we actually negotiate the rates. If we are unable to obtain sufficiently high reimbursement rates for our products, they may not be commercially viable or our future revenues and gross margin may be adversely affected.

As compared to our other, more traditional products, gene therapy products may present additional challenges with respect to the pricing, coverage, reimbursement, and acceptance of the product.

In addition to the risks set forth in this Risk Factors section associated with commercializing more traditional pharmaceutical drugs, there are additional, unique commercial risks associated with gene therapy products like ROCTAVIAN. Due to the relative novelty of gene therapy and the potential to provide extended duration therapeutic treatment with a one-time

administration, we face uncertainty with respect to the pricing, coverage and reimbursement of these products. In order to recover our research and development costs and commercialize one-time treatments on a profitable basis, the cost of a single administration of ROCTAVIAN is substantial, and it is likely other gene therapy products would also require relatively high prices. Therefore, coverage and reimbursement by governments and other third-party payers is essential for the vast majority of patients to be able to afford ROCTAVIAN or other gene therapy products that we may commercialize in the future. Accordingly, sales of our gene therapy products will depend substantially on the extent to which its cost will be paid by third-party payers. Even if coverage is provided, the reimbursement amounts approved by third-party payers may not be high enough to allow us to realize sufficient revenues from our investment in the development of our gene therapy products.

With respect to ROCTAVIAN specifically, we have entered into, and plan to enter into additional, outcomes-based agreements for the product with third-party payers to assist with realizing the value and sharing the risk of a one-time treatment, which make us subject to potential repayments if a patient does not respond to therapy or the therapeutic effect of the drug falls below specified thresholds. Although we will record reserves for potential refunds under the outcomes-based agreements for ROCTAVIAN in the same period as sales, our revenues and financial results could be adversely affected if our assumptions underlying our refund reserves differ from actual experience or otherwise underestimate refund obligations. Additionally, the novelty and increased complexity of reimbursement with outcomes-based arrangements heightens the risk that our price reporting may be inaccurate or delayed, which may result in fines and liability.

We also face uncertainty as to whether gene therapy will gain the acceptance of the public or the medical community. The commercial success of ROCTAVIAN or any other gene therapy product candidate that may be approved in the future will depend, in part, on the acceptance of physicians, patients and third-party payers of gene therapy products in general, and our product in particular, as medically necessary, cost-effective and safe. In particular, our success will depend upon physicians prescribing our product in lieu of existing treatments they are already familiar with and for which greater clinical data may be available. Moreover, physicians and patients may delay acceptance of one of our gene therapy treatments until the product has been on the market for a certain amount of time. Although administration of a gene therapy product like ROCTAVIAN is intended to correct an inborn genetic defect for at least several years, there is a risk that the therapeutic effect will not be durable and production of the desired protein or ribonucleic acid will decrease more quickly or cease entirely earlier than expected. If the therapeutic effect decreases significantly or ceases entirely, it is uncertain whether redosing is possible or would be effective. Furthermore, because gene therapy treatment is irreversible, there may be challenges in managing side effects, particularly those caused by potential overproduction of the desired protein. Adverse effects would not be able to be reversed or relieved by stopping dosing, and we may have to develop additional clinical safety procedures. Additionally, because the new gene copies are designed to reside permanently in a patient, there is a risk that they will disrupt other normal biological molecules and processes, including other healthy genes, and we may not learn the nature and magnitude of these side effects until long after clinical trials have been completed. Negative public opinion or more restrictive government regulations could have a negative effect on our business and financial condition and may delay or impair the successful commercialization of, and demand for, ROCTAVIAN or future gene therapy products.

Because the target patient populations for our products are relatively small, we must achieve significant market share and maintain high per-patient prices for our products to achieve and maintain profitability.

All of our products target diseases with relatively small patient populations. Our two newest products, VOXZOGO and ROCTAVIAN, address potentially larger patient populations than most of our other products; however, their market sizes are considerably smaller than many drugs marketed by other pharmaceutical and biotechnology companies. As a result, our per-patient prices must be relatively high in order to recover our development and manufacturing costs and achieve and maintain profitability. For BRINEURA, NAGLAZYME and VIMIZIM in particular, we must market worldwide to achieve significant market penetration of the product. In addition, because the number of potential patients in each disease population is small, it is not only important to find patients who begin therapy to achieve significant market penetration of the product, but we also need to be able to maintain these patients on therapy for an extended period of time. Due to the expected costs of treatment for our products, we may be unable to maintain or obtain sufficient market share at a price high enough to justify our product development efforts and manufacturing expenses.

If we fail to compete successfully with respect to product sales, we may be unable to generate sufficient sales to recover our expenses related to the development of a product program or to justify continued marketing of a product and our revenues could be adversely affected.

Our competitors may develop, manufacture and market products that are more effective or less expensive than ours. They may also obtain regulatory approvals for their products faster than we can obtain them (including those products with orphan drug designation, which may prevent us from marketing our product entirely for seven years, along with other regulatory exclusivities that could block approval) or commercialize their products before we do. With respect to ROCTAVIAN, we face a highly developed and competitive market for hemophilia A treatments. As we commercialize ROCTAVIAN, we may face intense competition from large pharmaceutical companies with extensive resources and established relationships in the hemophilia A community. If we do not compete successfully, our revenues would be adversely affected, and we may be unable to generate sufficient sales to recover our expenses related to the development of a product program or to justify continued marketing of a product.

Changes in methods of treatment of disease could reduce demand for our products and adversely affect revenues.

Even if our product candidates are approved, if doctors elect a course of treatment which does not include our products, this decision would reduce demand for our products and adversely affect revenues. For example, if gene therapy becomes widely used as a treatment of genetic diseases, the use of enzyme replacement therapy, such as ALDURAZYME, NAGLAZYME, and VIMIZIM in MPS diseases, could be greatly reduced. Changes in treatment method can be caused by the introduction of other companies' products or the development of new technologies or surgical procedures which may not directly compete with ours, but which have the effect of changing how doctors decide to treat a disease.

If we fail to develop new products and product candidates or compete successfully with respect to acquisitions, joint ventures, licenses or other collaboration opportunities, our ability to continue to expand our product pipeline and our growth and development would be impaired.

Our future growth and development depend in part on our ability to successfully develop new products from our development activities. The development of biopharmaceutical products is very expensive and time intensive and involves a great degree of risk. The outcomes of research and development programs, especially for innovative biopharmaceuticals like gene therapy products, are inherently uncertain and may not result in the commercialization of any products.

Our competitors compete with us to attract organizations for acquisitions, joint ventures, licensing arrangements or other collaborations. To date, several of our former and current product programs have been acquired through acquisitions and several of our former and current product programs have been developed through licensing or collaborative arrangements, such as ALDURAZYME, KUVAN and NAGLAZYME. These collaborations include licensing proprietary technology from, and other relationships with, academic research institutions. Our future success will depend, in part, on our ability to identify additional opportunities and to successfully enter into partnering or acquisition agreements for those opportunities. If our competitors successfully enter into partnering arrangements or license agreements with academic research institutions, we will then be precluded from pursuing those specific opportunities. Because each of these opportunities is unique, we may not be able to find a substitute. Several pharmaceutical and biotechnology companies have already established themselves in the field of genetic diseases. These companies have already begun many drug development programs, some of which target diseases that we are also targeting or may target in the future, and have already entered into partnering and licensing arrangements with academic research institutions, reducing the pool of available opportunities.

Universities and public and private research institutions also compete with us. While these organizations primarily have educational or basic research objectives, they may develop proprietary technology and acquire patents that we may need for the development of our product candidates. We will attempt to license this proprietary technology, if available. These licenses may not be available to us on acceptable terms, if at all. If we are unable to compete successfully with respect to acquisitions, joint venture and other collaboration opportunities, we may be limited in our ability to develop new products and to continue to expand our product pipeline.

The sale of generic versions of KUVAN by generic manufacturers has adversely affected and will continue to adversely affect our revenues and may cause a decline in KUVAN revenues faster than expected.

Generic versions of KUVAN are available in several countries around the world, including multiple generic versions in the U.S. and the EU. This generic competition has adversely affected and will continue to adversely affect our revenues from KUVAN, and we cannot accurately predict the rate of decline of KUVAN revenues in these countries. We are also aware that manufacturers are challenging our patent portfolio related to KUVAN in several jurisdictions, and several generic versions of KUVAN have been approved either centrally by the European Commission (EC) or on a country-by-country basis throughout the EU. If these patent challenges are successful, or if a manufacturer chooses to offer a generic version of KUVAN, notwithstanding our existing patents, our revenues from KUVAN may decline faster than expected.

If we do not achieve our projected development goals in the timeframes we announce or fail to achieve such goals, the commercialization of our product candidates may be delayed or never occur and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

For planning purposes, we estimate the timing of the accomplishment of various scientific, clinical, regulatory and other product development goals, which we sometimes refer to as milestones. These milestones may include the commencement or completion of scientific studies and clinical trials and the submission of regulatory filings. From time to time, we publicly announce the expected timing of some of these milestones. All of these milestones are based on a variety of assumptions. The actual timing of these milestones can vary dramatically compared to our estimates or the milestones may never be achieved, in many cases for reasons beyond our control. For example, in 2021 and early 2022, we announced that we planned to resubmit our Biologics License Application (BLA) for ROCTAVIAN to the Food and Drug Administration (FDA) in the first half of 2022; however, we did not file the BLA until the third quarter of 2022 due to the additional time we needed to include supplemental information and analyses of data requested by the FDA. If we do not meet development milestones as publicly announced, the commercialization of our

products may be delayed or never occur and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

We have in the past and may in the future enter into licensing arrangements, and we may not realize the benefits of such licensing arrangements.

We have in the past and may in the future enter into licensing arrangements with third parties. It is possible that we may not achieve financial or strategic benefits that justify a specific license, or we may otherwise not realize the benefits of such licensing arrangement. Further, licensing arrangements impose various diligence, milestone and royalty payment and other obligations on us. If we fail to comply with our obligations under any current or future licenses, our licensors may have the right to terminate these license agreements, which could harm our business prospects, financial condition and results of operations. Additionally, counterparties to our license agreements have in the past alleged and may in the future allege that we have breached a license agreement, which can result in litigation or other disputes that can divert management's attention away from our business and require us to expend resources, as well as potentially having to negotiate new or reinstated licenses with less favorable terms. Any such situation could adversely affect our business, financial condition, and results of operations.

Activist investor actions threatened or commenced against us have and could in the future cause us to incur substantial costs, divert management's attention and resources, cause uncertainty about the strategic direction of our business and adversely affect our business, financial position and results of operations.

We have been, and may in the future be, subject to activities initiated by activist investors. In December 2023, we entered into a Cooperation Agreement with Elliott Investment Management L.P., Elliott Associates, L.P. and Elliott International, L.P. (collectively, "Elliott"). We may not be successful in engaging constructively with one or more investors in the future despite our efforts to maintain constructive and ongoing communications with all investors, including Elliott. Resulting actions taken by activist investors from time to time have and could in the future conflict with our strategic direction, divert the attention of our Board of Directors, management, and employees, be costly and time-consuming, and disrupt the momentum in our business and operations, as well as our ability to execute our strategic plan. These types of actions may also create perceived uncertainties as to the future direction of our business or strategy, which may be exploited by our competitors and may make it more difficult to attract and retain qualified personnel, and may impact our relationships with investors, vendors, customers and other third parties. These types of actions could also impact the market price and the volatility of our common stock. In addition, we may choose to initiate, or may become subject to, litigation as a result of activist investor actions, which would serve as a further distraction to our Board of Directors, senior management and employees and could require us to incur significant additional costs.

Regulatory Risks

If we fail to obtain regulatory approval to commercially market and sell our product candidates, or if approval of our product candidates is delayed, we will be unable to generate revenues from the sale of these product candidates, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will increase.

We must obtain regulatory approval to market and sell our product candidates. For example, in the U.S., we must obtain FDA approval for each product candidate that we intend to commercialize, and in the EU, we must obtain approval from the EC, based on the opinion of the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA). The FDA and EC approval processes are typically lengthy and expensive, and approval is never certain. To obtain regulatory approval, we must first show that our product candidates are safe and effective for target indications through preclinical studies and clinical trials. Preclinical studies and clinical development are long, expensive and uncertain processes. Completion of clinical trials may take several years, and failure may occur at any stage of development. The length of time required varies substantially according to the type, complexity, novelty and intended use of a product candidate. Interim results of a preclinical test or clinical trial do not necessarily predict final results, and acceptable results in early clinical trials may not be repeated in later clinical trials. Accordingly, there are no assurances that we will obtain regulatory approval for any of our product candidates. Furthermore, there can be no assurance that approval of one of our product candidates by one regulatory authority will mean that other authorities will also approve the same product candidate. Similarly, in the EU, a positive CHMP opinion for approval of a product candidate does not guarantee that the EC will approve the product candidate. Moreover, regulatory authorities may approve a product candidate for fewer or more limited indications than requested. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates.

We have had fewer interactions with regulatory authorities outside the U.S. and the EU as compared to our interactions with the FDA, the EC and the EMA. The approval procedures vary among countries and can involve additional clinical testing, and the time required to obtain approval may differ from that required to obtain FDA or EC approval. Moreover, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Approval by the FDA or EC does not ensure approval by regulatory authorities in other countries, and approval by one or more non-U.S. regulatory authorities does not ensure approval by regulatory authorities in other non-U.S. countries or by the FDA or EC. However, a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in others. The non-U.S. regulatory approval process may include all of the risks associated with obtaining FDA or EC approval. We may not obtain non-U.S.

regulatory approvals on a timely basis, if at all. We may not be able to file for regulatory approvals and even if we file, we may not receive necessary approvals to commercialize our product candidates in any market.

We also rely on independent third-party Contract Research Organizations (CROs) to file some of our non-U.S. marketing applications, and while we keep a close oversight on the activities we delegate to CROs, important aspects of the services performed for us by the CROs are out of our direct control. If we fail to adequately manage our CROs, if the CRO elects to prioritize work on our projects below other projects or if there is any dispute or disruption in our relationship with our CROs, the filing of our applications may be delayed.

Although the FDA, the EC and the EMA have programs to facilitate expedited development and accelerated approval processes, the timelines agreed under legislative goals or mandated by regulations are subject to the possibility of substantial delays. Accordingly, even if any of our applications receives a designation to facilitate expedited development and accelerated approval processes, these designations may not result in faster review or approval for our product candidates compared to product candidates considered for approval under conventional procedures and, in any event, do not assure ultimate approval of our product candidates by regulatory authorities. In addition, the FDA, the EC, the EMA and other comparable international regulatory authorities have substantial discretion over the approval process for pharmaceutical products. These regulatory authorities may not agree that we have demonstrated the requisite level of product safety and efficacy to warrant approval and may require, and in the past have required, additional data. If we fail to obtain regulatory approval for our product candidates, we will be unable to market and sell those product candidates, which would have a negative effect on our business and financial condition.

Regulatory authorities and the new requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional or larger studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval studies, limitations or restrictions. For example, on August 18, 2020, the FDA issued a Complete Response Letter (CRL) to our BLA for ROCTAVIAN for the treatment of adults with severe hemophilia A. In the CRL, the FDA introduced a new request for two-year follow-up safety and efficacy data on all study participants from our ongoing Phase 3 study of ROCTAVIAN. In January 2022, we announced results from the requested two-year data analysis from our Phase 3 study. In the third quarter of 2022, we resubmitted our BLA, and the FDA subsequently accepted our submission with an original Prescription Drug User Fee Act (PDUFA) target action date of March 31, 2023. In early 2023, we supplemented our BLA by submitting our three-year analysis of the global Phase 3 study of ROCTAVIAN, which the FDA deemed to be a Major Amendment to our BLA due to the substantial amount of additional data, and extended the PDUFA target action date by three months. The FDA approved ROCTAVIAN for the treatment of adults with severe hemophilia A on June 29, 2023. Further, on April 26, 2023, the EC adopted a proposal for a new Directive and Regulation to revise the existing pharmaceutical legislation. If adopted in the form proposed, the recent EC proposals to revise the existing EU laws governing authorization of medicinal products may result in a decrease in data and market exclusivity for our product candidates in the EU.

In addition, some of our product candidates are intended to be used in combination with a medical device, such as an injector or other delivery system. Some of these products intended to be used with a medical device may be regulated as “combination products” in the U.S. and the EU, which are generally defined as products consisting of components from two or more regulatory categories (e.g., drug/device, device/biologic, drug/biologic). In the U.S., each component of a combination product is subject to the requirements established by the FDA for that type of component, whether a new drug, biologic or device. In order to facilitate pre-market review of combination products, the FDA designates one of its centers to have primary jurisdiction for the pre-market review and regulation of the overall product based upon a determination by the FDA of the primary mode of action of the combination product. The determination whether a product is a combination product or two separately regulated products is made by the FDA on a case-by-case basis. In the EU, medical devices and medicinal products are regulated separately, through different legislative instruments. The related applicable requirements will vary depending on the type of drug-device combination product. If, for example, a device intended to administer a medicinal product is sold together with such medicinal product in such a way that they form a single integral product which is intended exclusively for use in the given combination and which is not reusable, that single integral product is regulated as a medicinal product. In addition, the relevant general safety and performance requirements (GSPRs) established for medical devices by EU medical devices legislation apply to the device component of such combination products. In addition, some of our products require use with an *in vitro* companion diagnostic. For example, ROCTAVIAN is approved with a companion diagnostic test intended to detect pre-existing anti-AAV5 antibodies, which may render the gene therapy less effective or ineffective. Our other products and product candidates may also require use with an *in vitro* companion diagnostic if the FDA determines that the companion diagnostic is essential for safe and effective use of the product candidate. The FDA generally will require approval or clearance of the diagnostic, known as a companion diagnostic, at the same time that the FDA approves the therapeutic product. Most companion diagnostics require approval of a premarket approval application. In the EU, companion diagnostics are deemed to be *in vitro* diagnostic medical devices and must conform with the applicable GSPRs. To demonstrate compliance with the GSPRs, companion diagnostics must undergo a conformity assessment by a Notified Body. If the related medicinal product has been, or is in the process of being, authorized through the centralized procedure for the authorization of medicinal products, the Notified Body will, before it can issue a CE Certificate of Conformity, be required to seek a scientific opinion from the EMA on the suitability of the companion diagnostic for use in relation to the medicinal product concerned. For medicinal products that have been or are in the process of authorization through any other route provided in EU legislation, the Notified Body must seek the opinion of the national competent authority of an EU Member State. Our product candidates intended for use with separately regulated devices, such as companion diagnostics, or expanded indications that we may seek for our

products used with such devices, may not be approved or may be substantially delayed in receiving approval if the devices do not gain and/or maintain their own regulatory approvals, clearances, or certifications. Where approval of the drug or biologic product and device is sought under a single application, such as a drug with an injector or delivery system, the increased complexity of the review process may delay approval. The FDA and EU review processes and related criteria are complex, which could also lead to delays in the approval process. In addition, because these devices are provided by unaffiliated third-party companies, we are dependent on the sustained cooperation and effort of those third-party companies both to obtain regulatory approval and to maintain their own regulatory compliance. Failure of third-party companies to assist in the approval process or to maintain their own regulatory compliance could delay or prevent approval of our product candidates, or limit our ability to sell a product once it is approved.

Furthermore, despite our recent success obtaining regulatory approval for ROCTAVIAN in the U.S. and conditional approval in the EU, we may experience regulatory challenges for other gene therapy product candidates that cause significant delays or unanticipated costs, or that cannot be solved. Although numerous companies are currently advancing gene therapy product candidates through clinical trials, the FDA and EC have only approved a relatively small number of vector-based gene therapy products thus far. As a result, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our future gene therapy product candidates in any jurisdiction. Regulatory requirements governing gene and cell therapy products are still evolving and may continue to change in the future. Further, the FDA continues to develop and publish new guidance and policies, generally, by releasing one or more gene therapy-specific guidance documents each year. These guidance documents and other recent policy statements demonstrate that regulatory requirements for gene therapies are likely to continue to evolve based upon factors such as the intended disease or class of diseases, product type or mechanism of action, broader considerations such as the kinds of evidence that will be required for gene therapy products to take advantage of expedited development programs, and the experiences obtained by FDA when applying their legal and regulatory authorities to an evolving field, like gene therapy products. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring our gene therapy product candidates to market could have a negative effect on our business and financial condition.

From time to time during the development and regulatory approval process for our products and product candidates, we engage in discussions with the FDA, the EC, the EMA and other comparable international regulatory authorities regarding our development programs, including discussions about the regulatory requirements for approval. As part of these discussions, we sometimes seek advice in the design of our clinical programs from various regulatory authorities globally, but we do not always follow such guidance. This increases the chance of adverse regulatory actions, but we try to always provide appropriate scientific evidence to support approval. Moreover, sometimes different regulatory authorities provide different or conflicting advice. While we attempt to harmonize the advice we receive from multiple regulatory authorities, it is not always practical to do so. Also, we may choose not to harmonize conflicting advice when harmonization would significantly delay clinical trial data or is otherwise inappropriate. If we are unable to effectively and efficiently resolve and comply with the inquiries and requests of the FDA, the EC, the EMA and other comparable international regulatory authorities, the approval of our product candidates may be delayed and their value may be reduced.

Any product for which we have obtained regulatory approval, or for which we obtain approval in the future, is subject to, or will be subject to, extensive ongoing regulatory requirements by the FDA, the EC, the EMA and other comparable international regulatory authorities, and if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, we may be subject to penalties, we will be unable to generate revenues from the sale of such products, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will be increased.

Our products have received regulatory approval to be commercially marketed and sold in the U.S., the EU, and certain other countries except ROCTAVIAN, which has received regulatory approval to be commercially marketed in the U.S. and conditional approval to be commercially marketed in the EU. Any product for which we have obtained regulatory approval, or for which we obtain regulatory approval in the future, along with the manufacturing processes and practices, post-approval clinical research, product labeling, advertising and promotional activities for such product, are subject to continual requirements of, and review by, the FDA, the EC, the EMA and/or other comparable international and national regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, current Good Manufacturing Practices (cGMP) requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, import and export requirements and record keeping.

An example of the ongoing regulatory requirements our products are subject to is the PALYNZIQ Risk Evaluation and Mitigation Strategy (REMS) program. In the U.S., PALYNZIQ is only available through the REMS program, which is required by the FDA to mitigate the risk of anaphylaxis while using the product. Notable requirements of our REMS program include the following:

- prescribers must be certified by enrolling in the REMS program and completing training;
- prescribers must prescribe auto-injectable epinephrine with PALYNZIQ;
- pharmacies must be certified with the REMS program and must dispense PALYNZIQ only to patients who are authorized to receive it;

- patients must enroll in the REMS program and be educated about the risk of anaphylaxis by a certified prescriber to ensure they understand the risks and benefits of treatment with PALYNZIQ; and
- patients must have auto-injectable epinephrine available at all times while taking PALYNZIQ.

Failure of prescribers, pharmacies or patients to enroll in our REMS program or to successfully complete and comply with its requirements may result in regulatory action from the FDA or decreased sales of PALYNZIQ. The restrictions and requirements under our REMS program, as well as potential changes to these restrictions and requirements in the future, subject us to increased risks and uncertainties, any of which could harm our business. The requirement for a REMS program can materially affect the potential market for and profitability of a drug. We cannot predict whether the FDA will request, seek to require or ultimately require modifications to, or impose additional requirements on, the PALYNZIQ REMS program, or whether the FDA will permit modifications to the PALYNZIQ REMS program that we consider warranted. Any modifications required or rejected by the FDA could make it more difficult or expensive for us to distribute PALYNZIQ in the U.S., impair the safety profile of PALYNZIQ, disrupt continuity of care for PALYNZIQ patients and/or negatively affect sales of PALYNZIQ.

In addition, in the EU, the marketing authorization for BRINEURA was granted under “exceptional circumstances”. As a result, the risk-benefit balance of BRINEURA is reviewed annually and the marketing authorization may be withdrawn if the risk-benefit ratio is no longer favorable. The conditional marketing authorization for ROCTAVIAN is, moreover, valid for one year and must be reviewed annually until all related conditions have been fulfilled to permit transfer to a full authorization. Failure to continue to show favorable risk-benefit balance for BRINEURA or satisfy the conditions related to ROCTAVIAN’s conditional marketing authorization could result in the withdrawal of the marketing approvals for these products.

Moreover, promotional communications with respect to prescription drugs, including biologics, are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product’s approved labeling and Summary of Product Characteristics. In particular, a product may not be promoted for uses that are not approved by the FDA or the EC as reflected in the product’s approved labeling. Although the FDA and other comparable international and national regulatory authorities do not regulate a physician’s choice of drug treatment made in the physician’s independent medical judgment, they do restrict promotional communications from companies or their sales force with respect to off-label uses of products for which marketing clearance has not been issued. The FDA and other national competent authorities or international regulatory authorities actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant civil, criminal and administrative penalties. Thus, we are not able to promote any products we develop for indications or uses for which they are not approved. Additionally, in the EU, it is prohibited to promote prescription drugs to the general public and we are therefore limited to promote our products exclusively to healthcare professionals. Public prosecutors, industry associations, healthcare professionals and other members of the public closely scrutinize advertising and promotion of any product in the EU.

Moreover, if original FDA approval for one of our product candidates is granted via the accelerated approval pathway, we will be required to conduct a post-marketing confirmatory trial to verify and describe the clinical benefit in support of full approval. An unsuccessful post-marketing study or failure to complete such a study with due diligence could result in the withdrawal of the FDA’s marketing approval for a product candidate. For example, VOXZOGO is approved in the U.S. under accelerated approval based on an improvement in annualized growth velocity. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory studies. To fulfill this post-marketing requirement, we intend to use our ongoing open-label extension studies compared to available natural history. In addition, the FDA and the EC often require post-marketing testing and surveillance to monitor the effects of products. The FDA, the EC and other comparable international regulatory authorities may condition approval of our product candidates on the completion of such post-marketing clinical studies. These post-marketing studies may suggest that a product causes undesirable side effects or may present a risk to the patient.

Discovery after approval of previously unknown problems with any of our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in actions such as:

- the issuance of safety alerts, press releases or other communications containing warnings about related products;
- modifications to promotional materials or corrective information to healthcare professionals;
- restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials;
- suspensions or restrictions on our operations, including product manufacturing processes;
- restrictions on the marketing of a product;
- restrictions on product distribution;
- requirements to conduct post-marketing clinical trials;
- untitled or warning letters or other adverse publicity;
- withdrawal of the products from the market
- suspended or withdrawn regulatory approvals;

- refusal or delays to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- refusal to permit the import or export of our products;
- product seizure;
- fines, restitution or disgorgement of profits or revenue;
- injunctions; or
- imposition of civil or criminal penalties.

If such regulatory actions are taken, our value and our operating results will be adversely affected. Additionally, if the FDA, the EC or any other comparable international regulatory authorities withdraws its approval of a product, we will be unable to generate revenues from the sale of that product in the relevant jurisdiction, our potential for generating positive cash flow will be diminished and the capital necessary to fund our operations will be increased. Accordingly, we continue to expend significant time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance, post-marketing studies and quality control.

To obtain regulatory approval to market our products, preclinical studies and costly and lengthy clinical trials are required and the results of the studies and trials are highly uncertain. Likewise, preliminary, initial or interim data from clinical trials should be considered carefully and with caution because the final data may be materially different from the preliminary, initial or interim data, particularly as more patient data become available.

As part of the drug development process, we must conduct, at our own expense, preclinical studies in the laboratory, including studies in animals, and clinical trials on humans for each product candidate. The number of preclinical studies and clinical trials that regulatory authorities require varies depending on the product candidate, the disease or condition the drug is being developed to address and regulations applicable to the particular drug. Generally, new drugs for diseases or conditions that affect larger patient populations, are less severe, or are treatable by alternative strategies must be validated through additional preclinical and clinical trials and/or clinical trials with higher enrollments. With respect to our early-stage product candidates, we may need to perform multiple preclinical studies using various doses and formulations before we can begin clinical trials, which could result in delays to our development timeline. Furthermore, even if we obtain favorable results in preclinical studies, the results in humans may be significantly different. After we have conducted preclinical studies, we must demonstrate that our product candidates are safe and efficacious for the intended indication and for use in the targeted human patients in order to receive regulatory approval for commercial sale. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials, and favorable data from interim analyses do not ensure the final results of a trial will be favorable. From time to time, we have and may in the future publish or report preliminary, initial or interim data from our clinical trials. Preliminary, initial or interim data from our clinical trials may not be indicative of the final results of the trial and are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and/or more patient data become available. In this regard, such data may show initial evidence of clinical benefit, but as patients continue to be followed and more patient data become available, there is a risk that any therapeutic effects will not be durable in patients and/or will decrease over time or cease entirely. Preliminary, initial or interim data also remain subject to audit and verification procedures that may result in the final data being materially different from such preliminary, initial or interim data. As a result, preliminary, initial or interim data should be considered carefully and with caution until the final data are available.

Product candidates may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials, or despite having favorable data in connection with an interim analysis. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Also, as noted above, we do not always follow the advice of regulatory authorities or comply with all of their requests regarding the design of our clinical programs. In those cases, we may choose a development program that is inconsistent with the advice of regulatory authorities, which may limit the jurisdictions where we conduct clinical trials and/or adversely affect our ability to obtain approval in those jurisdictions where we do not follow the regulatory advice.

Adverse or inconclusive clinical results could stop us from obtaining regulatory approval of our product candidates. Additional factors that can cause delay or termination of our clinical trials include:

- slow or insufficient patient enrollment;
- slow recruitment of, and completion of necessary institutional approvals at, clinical sites;
- budgetary constraints or prohibitively high clinical trial costs;
- longer treatment time required to demonstrate efficacy;
- lack of sufficient supplies of the product candidate;

- adverse medical events or side effects in treated patients, including immune reactions;
- lack of effectiveness of the product candidate being tested;
- availability of competitive therapies to treat the same indication as our product candidates;
- regulatory requests for additional clinical trials or preclinical studies;
- deviations in standards for Good Clinical Practice (GCP); and
- disputes with or disruptions in our relationships with clinical trial partners, including CROs, clinical laboratories, clinical sites, and principal investigators.

Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our current and future products, which would adversely affect our revenues and results of operations.

We expect that coverage and reimbursement may be increasingly restricted in all the markets in which we sell our products. The escalating cost of healthcare has led to increased pressure on the healthcare industry to reduce costs. In particular, drug pricing by pharmaceutical companies has been under scrutiny for many years and continues to be subject to intense political and public debate in the U.S. and abroad. Governmental and private third-party payers have proposed healthcare reforms and cost reductions. A number of federal and state proposals to control the cost of healthcare, including the cost of drug treatments, have been made in the U.S. Specifically, there have been several recent U.S. congressional inquiries and proposed bills and enacted legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. Further, Congress and the executive branch have each indicated that they will continue to seek new legislative and/or administrative measures to control drug costs. In some international markets, the government controls the pricing, which can affect the profitability of drugs. Current government regulations and possible future legislation regarding healthcare may affect coverage and reimbursement for medical treatment by third-party payers, which may render our products not commercially viable or may adversely affect our future revenues and gross margins.

International operations are also generally subject to extensive price and market regulations, and there are many proposals for additional cost-containment measures, including proposals that would directly or indirectly impose additional price controls or mandatory price cuts or reduce the value of our intellectual property portfolio. As part of these cost containment measures, some countries have imposed and continue to propose revenue caps limiting the annual volume of sales of our products. Some of these caps are significantly below the actual demand in certain countries, and if the trend regarding revenue caps continues, our future revenues and gross margins may be adversely affected. For example, in the EU, governments influence the price of medicinal products through their pricing and reimbursement rules and control of national healthcare systems that fund a large part of the cost of those products to consumers. EU Member States are free to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed to by the government. An EU Member State may approve a specific price for the medicinal product, or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market, including volume-based arrangements, caps and reference pricing mechanisms. Other EU Member States allow companies to fix their own prices for medicines but monitor and control company profits. The downward pressure on healthcare costs in general, particularly prescription medicines, has become very intense. Pharmaceutical products may face competition from lower-priced products in foreign countries that have placed price controls on pharmaceutical products and may also compete with imported foreign products. Furthermore, there is no assurance that a product will be considered medically reasonable and necessary for a specific indication or cost-effective by third-party payers. There is also no assurance that an adequate level of reimbursement will be established even if coverage is available or that the third-party payers' reimbursement policies will not adversely affect our business.

We cannot predict the extent to which our business may be affected by these or other potential future legislative or regulatory developments. However, future price controls or other changes in pricing regulation or negative publicity related to our product pricing or the pricing of pharmaceutical drugs generally could restrict the amount that we are able to charge for our current and future products or our sales volume, which would adversely affect our revenues and results of operations.

Government healthcare reform could increase our costs and adversely affect our revenues and results of operations.

Our industry is highly regulated and changes in law may adversely impact our business, operations or financial results. In the U.S., there have been and continue to be a number of legislative initiatives to contain healthcare costs. In the U.S., there have been several recent congressional inquiries, proposed and enacted federal and state legislation and executive action designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the cost of drugs under Medicare, and reform government program reimbursement methodologies for drug products. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payers. Recently, several healthcare reform initiatives culminated in the enactment of the Inflation Reduction

Act (IRA) in August 2022, which allows, among other things, U.S. Department of Health and Human Services (HHS) to negotiate the selling price of a statutorily specified number of drugs and biologics each year that the CMS reimburses under Medicare Part B and Part D. Only high-expenditure single-source drugs that have been approved for at least 7 years (11 years for biologics) can be selected by CMS for negotiation. The IRA permits the Secretary of HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. HHS has and will continue to issue and update guidance as these programs are implemented. Manufacturers that fail to comply with the IRA may be subject to various penalties, including civil monetary penalties. The IRA's provisions are taking effect progressively starting in 2023, although they may be subject to legal challenges. Thus, while it is unclear how the IRA will be implemented, it will likely have a significant impact on the pharmaceutical industry.

Prior to the IRA, the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the PPACA) is a sweeping measure intended to, among other things, expand healthcare coverage within the U.S., primarily through the imposition of health insurance mandates on employers and individuals and expansion of the Medicaid program. Several provisions of the law have affected us and increased certain of our costs. Since its enactment, there have been executive, judicial and congressional challenges to certain aspects of the PPACA. Although the PPACA has generally been upheld thus far, it is unclear how continued challenges to the law may impact the PPACA and our business. In addition, other legislative changes have been adopted since the PPACA was enacted. Some of these changes have resulted in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and, accordingly, our financial operations.

In addition, individual states in the U.S. have also increasingly enacted laws and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, price disclosure and reporting requirements, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Moreover, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs.

Likewise, in many EU Member States, legislators and other policymakers continue to propose and implement healthcare cost-containing measures in response to the increased attention being paid to healthcare costs in the EU. Certain of these changes could impose limitations on the prices we will be able to charge for our commercial products and any product candidates or the amounts of reimbursement available for these products from governmental and private third-party payers, may increase the tax obligations on pharmaceutical companies or may facilitate the introduction of generic competition with respect to our products. Further, an increasing number of EU Member States and other non-U.S. countries use prices for medicinal products established in other countries as “reference prices” to help determine the price of the product in their own territory. If the price of one of our products decreases substantially in a reference price country, it could impact the price for that product in other countries. Consequently, a downward trend in prices of our products in some countries could contribute to similar downward trends elsewhere, which would have a material adverse effect on our revenues and results of operations. Moreover, some EU Member States require the completion of additional studies that compare the cost-effectiveness of a particular medicinal product candidate to currently available therapies. This Health Technology Assessment (HTA) process, which is currently governed by the national laws of the individual EU Member States, is the procedure according to which the assessment of the public health impact, therapeutic impact and the economic and societal impact of use of a given medicinal product in the national healthcare systems of the individual country is conducted. The outcome of HTA regarding specific medicinal products will often influence the pricing and reimbursement status granted to these medicinal products by the competent authorities of individual EU Member States. In 2022, the EC adopted the HTA regulation, which is intended to boost cooperation among EU Member States in evaluating new medicinal products. The HTA regulation will apply starting in 2025 and may result in increased downward pricing pressure in the EU.

We anticipate that the IRA, PPACA and other healthcare reform measures that may be adopted in the future in the U.S. or abroad, may result in more rigorous coverage criteria and an additional downward pressure on the reimbursement our customers may receive for our products. Legally mandated price controls on payment amounts by governmental and private third-party payers or other restrictions could harm our business, results of operations, financial condition and prospects. 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.

If we fail to obtain or maintain orphan drug exclusivity for some of our products, our competitors may obtain approval to sell the same drugs to treat the same conditions and our revenues will be reduced.

As part of our business strategy, we have developed and may in the future develop some drugs that may be eligible for FDA and EU orphan drug designation. Under the Orphan Drug Act, the FDA may designate a 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 U.S. or as a condition that affects more than 200,000 individuals in the U.S. and for which there is no reasonable expectation that the costs of development of said drug will be recovered from sales in the U.S. In the EU, pursuant to the Regulation (EC) No. 141/2000 (the Orphan Regulation), as implemented by Regulation (EC) No. 847/2000, orphan drug designation is available if a sponsor can establish that: (1) the medicine is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting no more than five in 10,000 people in the EU at the time the application is made, or, (2) that it is intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition in the EU and that

without incentives derived from the orphan status, it is unlikely that the marketing of the medicine in the EU would generate sufficient return to justify the necessary investment. In both cases, the applicant must demonstrate that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the EU or, if such method exists, the medicine will be of significant benefit to those affected by that condition.

In the U.S., the company that first obtains FDA approval for a designated orphan drug for a given rare disease receives marketing exclusivity for use of that drug for the designated condition for a period of seven years. Orphan drug exclusive marketing rights may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug. In addition, the FDA may approve another drug during a period of orphan drug exclusivity if the second drug is found to be clinically superior to the first drug. In the EU, a ten-year period of market exclusivity for the approved therapeutic indication (extendable to twelve years for orphan drugs that have complied with an agreed Pediatric Investigation Plan (PIP) pursuant to Regulation 1901/2006), during which the EMA cannot accept another marketing authorization application or accept an application to extend existing authorizations for similar medicinal products for the same indication and no related marketing authorization (MA) can be granted. MAs may also be granted to a similar medicinal product with the same orphan indication if: (i) the applicant can establish that the second medicinal product, although similar to the orphan medicinal product already authorized is safer, more effective or otherwise clinically superior to the orphan medicinal product already authorized; (ii) the MA holder for the first orphan medicinal product grants its consent; or (iii) if the MA holder of the orphan medicinal product is unable to supply sufficient quantities. MAs may also be granted for the same therapeutic indication in relation to products that are not similar. The period of market exclusivity may, in addition, be reduced to six years if, at the end of the fifth year, it can be demonstrated on the basis of available evidence that the criteria for its designation as an orphan medicine are no longer satisfied, for example if the original orphan medicinal product has become sufficiently profitable not to justify maintenance of market exclusivity. Because the extent and scope of patent protection for some of our products is limited, orphan drug designation and resulting regulatory exclusivity is especially important for our products that are eligible for orphan drug designation. For eligible products, we plan to rely on the exclusivity period under the Orphan Drug Act and/or the Orphan Regulation, as applicable, to maintain a competitive position. If we do not obtain orphan drug designation and related regulatory exclusivity for our products that do not have broad patent protection or if a competing product is determined to be "clinically superior" to any of our products that has secured orphan drug exclusivity, our competitors may then sell the same drug to treat the same condition and our revenues will be reduced.

Even though we have obtained orphan drug designation for certain of our product candidates and even if we obtain orphan drug designation for our future product candidates, due to the uncertainties associated with developing biopharmaceutical products, we may not be the first to obtain marketing approval for any particular orphan indication, which means that we may not obtain orphan drug regulatory exclusivity and could also potentially be blocked from approval of certain product candidates until the competitor product's orphan drug exclusivity period expires. Moreover, with respect to certain biologics and gene therapies, there may be some uncertainty regarding how similarity between product candidates designed to treat the same rare disease or condition may affect such product candidates' orphan drug regulatory exclusivities. For biologics and gene therapies, the FDA's determination of whether a drug is the same drug or a different drug will be based on the principal molecular structural features of the products. For gene therapy products, the FDA has stated in guidance that it generally intends to consider certain key features such as transgenes and vectors used in gene therapy products to be principal molecular structural features. The FDA has not yet proffered additional information on orphan drug sameness for gene therapy or similar products. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition and the same drug can be approved for different conditions and potentially used off-label in the orphan indication. Even after an orphan drug is approved and granted orphan drug exclusivity, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is safer or 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.

We may face competition from biosimilars approved through an abbreviated regulatory pathway.

Our ALDURAZYME, BRINEURA, NAGLAZYME, PALYNZIQ, ROCTAVIAN and VIMIZIM products are regulated by the FDA as biologics under the Federal Food, Drug, and Cosmetic Act and the Public Health Service Act (the PHS Act). Biologics require the submission of a BLA and licensure by the FDA prior to being marketed in the U.S. The Biologics Price Competition and Innovation Act of 2009 (BPCIA) created a regulatory pathway under the PHS Act for the abbreviated licensure of biological products that are demonstrated to be "biosimilar" to or "interchangeable" with an FDA-licensed biological product. A similar abridged MA process is available to biosimilar products in the EU. In particular, applicants for MAs of biosimilars are required to demonstrate through comprehensive comparability studies with the reference biological medicine that: a) their biological medicine is highly similar to the reference medicine, notwithstanding natural variability inherent to all biological medicines; and b) there are no clinically meaningful differences between the biosimilar and the reference medicine in terms of safety, quality and efficacy.

In the U.S., in order to meet the standard of interchangeability, a sponsor must demonstrate that the biosimilar product can be expected to produce the same clinical result as the reference product in any given patient, and for a product that is administered more than once, that the risk of switching in terms of safety or diminished efficacy of alternating or switching between the reference product and biosimilar product is not greater than the risk of maintaining the patient on the reference product. The BPCIA establishes a period of 12 years of data exclusivity for reference products but such data exclusivity only blocks licensure of

biosimilars relying on the product as a reference product; it will not prevent the licensure of the same product for the same or different indications that does not seek to rely on reference product data. In the EU, a medicinal product containing a new active substance benefits from eight years of data exclusivity, during which biosimilar applications referring to the data of that product may not be accepted by the regulatory authorities, and a further two years of market exclusivity, during which biosimilar applications may be submitted and the reference product's data may be referenced but biosimilar products may not be placed on the market. The two-year period may be extended to three years if during the first eight years a new therapeutic indication with significant clinical benefit over existing therapies is approved. Our products approved under BLAs in the U.S. or as a result of Marketing Authorization Applications (MAAs) in the EU, as well as our product candidates that may be approved in the future, could be reference products for biosimilar marketing applications.

Changes in funding for the FDA, the EMA, other comparable regulatory authorities and other government agencies or government shutdowns could hinder the ability of such authorities and agencies to hire and retain key leadership and other personnel or otherwise prevent those authorities and agencies from performing normal functions on which the operation of our business may rely, which could negatively impact our business.

Changes in funding levels of regulatory authorities and government agencies can affect their ability to hire and retain key personnel and carry out their normal functions that support our business. For example, the ability of the FDA or the EMA to timely review and approve INDs or MAAs for our product candidates may be hindered by a lack of resources and qualified personnel. In addition, funding of other regulatory authorities and government agencies on which our operations rely, including those that fund research and development activities, is subject to the political budget process, which is inherently fluid and unpredictable.

Government shutdowns could also impact the ability of regulatory authorities and government agencies to function normally and support our operations. For example, the U.S. federal government has shut down repeatedly since 1980, including for a period of 35 days beginning on December 22, 2018. During a shutdown, certain regulatory authorities and agencies, such as the FDA, have had to furlough key personnel and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

Financial and Financing Risks

If we incur operating losses or are unable to sustain positive cash flows for a period longer than anticipated, we may be unable to continue our operations at planned levels and may be forced to reduce our operations.

Since we began operations in March 1997, we have been engaged in substantial research and development and capital investments, and we have operated at a net loss for most years since our inception and there is no guarantee that we will achieve or maintain profitability in the future. Our future profitability and cash flows depend on our marketing and selling of our products, the receipt of regulatory approval of our product candidates, our ability to successfully manufacture and market any products, either by ourselves or jointly with others, our spending on our development programs, the impact of any possible future business development transactions and other risks set forth in this Risk Factors section. The extent of our future losses and the timing of profitability and positive cash flows are highly uncertain. If we are unable to sustain profitability and positive cash flows on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

If we fail to obtain the capital necessary to fund our operations, our financial results and financial condition will be adversely affected and we will have to delay or terminate some or all of our product development programs.

As of December 31, 2023, we had cash, cash equivalents and investments totaling $1.7 billion and debt obligations of $1.1 billion (undiscounted), which consisted of our 0.599% senior subordinated convertible notes due in 2024 (the 2024 Notes) and our 1.25% senior subordinated convertible notes due in 2027 (the 2027 Notes). The 2024 Notes and the 2027 Notes (collectively, the Notes), if not converted, will be required to be repaid in cash at maturity in August 2024 and May 2027, respectively. We will need cash not only to pay the ongoing interest due on the Notes during their term, but also to repay the principal amount of the Notes if not converted.

We may require additional financing to fund the repayment of the Notes, future milestone payments and our future operations, including the commercialization of our products and product candidates currently under development, preclinical studies and clinical trials, and potential licenses and acquisitions. We may be unable to raise additional financing due to a variety of factors, including our financial condition, the status of our product programs, and the general condition of the financial markets. If we fail to raise any necessary additional financing we may have to delay or terminate some or all of our product development programs and our financial condition and operating results will be adversely affected.

We expect to continue to spend substantial amounts of capital for our operations for the foreseeable future. The amount of capital we will need depends on many factors, including:

- our ability to successfully market and sell our products;

- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities the progress and success of our preclinical studies and clinical trials (including studies and the manufacture of materials);
- the timing, number, size and scope of our preclinical studies and clinical trials;
- the time and cost necessary to obtain regulatory approvals and the costs of post-marketing studies which may be required by regulatory authorities;
- the progress of research programs carried out by us;
- any changes made to, or new developments in, our existing collaborative, licensing and other commercial relationships or any new collaborative, licensing and other commercial relationships that we may establish;
- Sanofi's ability to continue to successfully commercialize ALDURAZYME; and
- whether our convertible debt is converted to common stock in the future.

Moreover, our fixed expenses such as rent, license payments, interest expense and other contractual commitments are substantial and may increase in the future. These fixed expenses may increase because we may enter into:

- additional licenses and collaborative agreements;
- additional contracts for product manufacturing; and
- additional financing facilities or arrangements.

We will need to raise additional funds from equity or debt securities, loans or collaborative agreements if we are unable to satisfy our liquidity requirements. The sale of additional equity and/or equity-linked securities will result in additional dilution to our stockholders. Furthermore, additional financing may not be available in amounts or on terms satisfactory to us or at all. This could result in the delay, reduction or termination of our research, which could harm our business.

We have incurred substantial indebtedness that may decrease our business flexibility, access to capital, and/or increase our borrowing costs, which may adversely affect our operations and financial results.

As of December 31, 2023, we had $1.1 billion (undiscounted) principal amount of indebtedness, including $495.0 million (undiscounted) principal amount of indebtedness under the 2024 Notes and $600.0 million (undiscounted) principal amount of indebtedness under the 2027 Notes. Our indebtedness may:

- limit our ability to borrow additional funds for working capital, capital expenditures, acquisitions or other general business purposes;
- limit our ability to use our cash flow or obtain additional financing for future working capital, capital expenditures, acquisitions or other general business purposes;
- require us to use a substantial portion of our cash flow from operations to make debt service payments;
- limit our flexibility to plan for, or react to, changes in our business and industry;
- place us at a competitive disadvantage compared to our less leveraged competitors; and
- increase our vulnerability to the impact of adverse economic and industry conditions.

If we default under any series of the Notes, such series of Notes could become immediately due and payable and it could lead to defaults under the other series of Notes.

In addition, our ability to refinance our indebtedness will depend on the capital markets and our financial condition at such time.

Our outstanding indebtedness consists primarily of the 2024 Notes and 2027 Notes, which, if not converted, will be required to be repaid in cash at maturity in August 2024 and May 2027, respectively. While we could seek to obtain additional third-party financing to pay for any amounts due in cash upon maturity of the Notes, we cannot be sure that such third-party financing will be available on commercially reasonable terms, if at all.

Manufacturing Risks

If we fail to comply with manufacturing regulations, our financial results and financial condition will be adversely affected.

Prior to commercialization of our products, regulatory authorities must approve marketing applications that identify authorized manufacturing facilities operated by us or our contract manufacturers that are in compliance with cGMP requirements. In addition, our pharmaceutical manufacturing facilities are continuously subject to scheduled and unannounced regulatory inspections by the FDA, and other comparable EU and other national and international regulatory authorities, before and after product approval, to monitor and ensure compliance with cGMP and other regulations. Our manufacturing facilities in the U.S. are licensed for the manufacture of PALYNZIQ, ROCTAVIAN, ALDURAZYME, BRINEURA, NAGLAZYME, VIMIZIM, and VOXZOGO. Our manufacturing facility in Shanbally, Cork, Ireland is licensed for the manufacture of VIMIZIM and BRINEURA and packaging operations for VOXZOGO and PALYNZIQ. In addition, our third-party manufacturers' facilities involved with the manufacture of our products have also been inspected and approved by various regulatory authorities. Although we are not involved in the day-to-day operations of our contract manufacturers, we are ultimately responsible for ensuring that our products are manufactured in accordance with cGMP regulations.

Due to the complexity of the processes used to manufacture our products and product candidates, we may be unable to continue to pass or initially pass federal, national or international regulatory inspections in a cost-effective manner. For the same reason, any potential third-party manufacturer of our products or our product candidates may be unable to comply with cGMP regulations in a cost-effective manner and may be unable to initially or continue to pass a federal, national or international regulatory inspection.

If we, or third-party manufacturers with whom we contract, are unable to comply with manufacturing regulations, we may be subject to delay of approval of our product candidates, warning or untitled letters, fines, unanticipated compliance expenses, recall or seizure of our products, total or partial suspension of production and/or enforcement actions, including injunctions, and criminal or civil prosecution. These possible sanctions would adversely affect our financial results and financial condition.

If we are unable to successfully develop and maintain manufacturing processes for our product candidates to produce sufficient quantities at acceptable costs, we may be unable to support a clinical trial or be forced to terminate a program, or if we are unable to produce sufficient quantities of our products at acceptable costs, we may be unable to meet commercial demand, lose potential revenue, have reduced margins or be forced to terminate a program.

Due to the complexity of manufacturing our product candidates and products, we may not be able to manufacture sufficient quantities. Our inability to produce enough of our product candidate at acceptable costs may result in the delay or termination of development programs. With respect to our commercial portfolio, we may not be able to manufacture our products successfully with a commercially viable process or at a scale large enough to support their respective commercial markets or at acceptable margins. For example, demand for VOXZOGO in certain markets has outpaced our projections in recent quarters, and we have and could continue to face challenges meeting demand, requiring us to postpone planned entry into additional markets until VOXZOGO inventory levels increase or delay certain VOXZOGO development activities. As a result of such inventory constraints, we have and could continue to lose potential VOXZOGO revenues that may never be recouped and our VOXZOGO development program could be adversely impacted.

The development of commercially viable manufacturing processes typically is very difficult to achieve and is often very expensive and may require extended periods of time. Changes in manufacturing processes (including manufacturing cell lines), equipment or facilities (including moving manufacturing from one of our facilities to another one of our facilities or a third-party facility, or from a third-party facility to one of our facilities) may require us to complete clinical trials to receive regulatory approval of any manufacturing modifications.

Our gene therapy product and product candidates are based on relatively novel technology, which presents additional manufacturing risks in relation to our other, more traditional drug development programs. Gene therapy products are complex and have only in limited cases been manufactured at scales sufficient for pivotal trials and commercialization. Few pharmaceutical contract manufacturers specialize in gene therapy products and those that do are still developing appropriate processes and facilities for large-scale production. We invested a considerable amount of capital building our own commercial gene therapy manufacturing facility, which may be subject to significant impairment if our gene therapy programs are unsuccessful. As we develop, seek to optimize and operate the gene therapy manufacturing process, we will likely face technical and scientific

challenges, considerable capital costs, and potential difficulty in recruiting and hiring experienced, qualified personnel. There may also be unexpected technical or operational issues during clinical or commercial manufacturing campaigns. As a result, we could experience manufacturing delays that prevent us from completing our gene therapy clinical studies in a timely manner, if at all, or commercializing our gene therapy products on a profitable basis, if at all.

Also, we may be required to demonstrate product comparability between a biological product made after a manufacturing change and the product made before implementation of the change through additional types of analytical and functional testing or may have to complete additional clinical studies. If we contract for manufacturing services with an unproven process, our contractor is subject to the same uncertainties, high standards and regulatory controls, and may therefore experience difficulty if further process development is necessary.

Even a developed manufacturing process can encounter difficulties. Problems may arise during manufacturing for a variety of reasons, including human error, mechanical breakdowns, problems with raw materials and cell banks, malfunctions of internal information technology systems, and other events that cannot always be prevented or anticipated. Many of the processes include biological systems, which add significant complexity, as compared to chemical synthesis. We expect that, from time to time, consistent with biotechnology industry expectations, certain production lots will fail to produce product that meets our quality control release acceptance criteria. To date, our historical failure rates for all of our product programs have been within our expectations, which are based on industry norms. If the failure rate increased substantially, we could experience increased costs, lost revenue, damage to customer relations, time and expense investigating the cause and, depending upon the cause, similar losses with respect to other lots or products. If problems are not discovered before the product is released to the market, recall and product liability costs may also be incurred.

In order to produce product within our time and cost parameters, we must continue to produce product within our expected success rate and yield expectations. Because of the complexity of our manufacturing processes, it may be difficult or impossible for us to determine the cause of any particular lot failure and we must effectively take corrective action in response to any failure in a timely manner.

We currently rely on third parties for portions of the manufacture of each of our products. If those manufacturers are unwilling or unable to fulfill their contractual obligations or satisfy demand outside of or in excess of the contractual obligations, we may be unable to meet demand for these products or sell these products at all and we may lose potential revenue. Further, the availability of suitable contract manufacturing capacity at scheduled or optimum times is not certain.

In addition, our manufacturing processes subject us to a variety of federal, state, supranational, national, and local laws and regulations governing the use, generation, manufacture, storage, handling and disposal of hazardous materials and wastes resulting from their use. We incur significant costs in complying with these laws and regulations.

Supply interruptions may disrupt our inventory levels and the availability of our products and product candidates and cause delays in obtaining regulatory approval for our product candidates, or harm our business by reducing our revenues.

We depend on single-source suppliers for critical raw materials and a limited number of manufacturing facilities to manufacture our finished products and product candidates. Numerous factors could cause interruptions in the supply or manufacture of our products and product candidates, including:

- timing, scheduling and prioritization of production by our contract manufacturers or a breach of our agreements by our contract manufacturers;
- labor interruptions;
- changes in our sources for manufacturing;
- the timing and delivery of shipments;
- our failure to locate and obtain replacement suppliers and manufacturers as needed on a timely basis; and
- conditions affecting the cost and availability of raw materials, including inflation.

If one of our suppliers or manufacturers fails or refuses to supply us with necessary raw materials or finished products or product candidates on a timely basis or at all, it would take a significant amount of time and expense to qualify a new supplier or manufacturer. We may not be able to obtain active ingredients or finished products from new suppliers or manufacturers on acceptable terms and at reasonable prices, or at all.

Any interruption in the supply of finished products could hinder our ability to distribute finished products to meet commercial demand and adversely affect our financial results and financial condition.

With respect to our product candidates, production of product is necessary to perform clinical trials and successful registration batches are necessary to file for approval to commercially market and sell product candidates. Delays in obtaining clinical material or registration batches could adversely impact our clinical trials and delay regulatory approval for our product candidates.

If our Manufacturing, Marketing and Sales Agreement with Sanofi were terminated, we could be prevented from continuing to commercialize ALDURAZYME or our ability to successfully commercialize ALDURAZYME would be delayed or diminished.

Either party may terminate the Manufacturing, Marketing and Sales Agreement (the MMS Agreement) between Sanofi and us related to ALDURAZYME for specified reasons, including if the other party is in material breach of the MMS Agreement, has experienced a change of control, as such term is defined in the MMS Agreement, or has declared bankruptcy and also is in breach of the MMS Agreement. Although we are not currently in breach of the MMS Agreement, there is a risk that either party could breach the MMS Agreement in the future. Either party may also terminate the MMS Agreement upon one-year prior written notice for any reason.

If the MMS Agreement is terminated for breach, the breaching party will transfer its interest in the BioMarin/Genzyme LLC to the non-breaching party, and the non-breaching party will pay a specified buyout amount for the breaching party's interest in ALDURAZYME and in the BioMarin/Genzyme LLC. If we are the breaching party, we would lose our rights to ALDURAZYME and the related intellectual property and regulatory approvals. If the MMS Agreement is terminated without cause, the non-terminating party would have the option, exercisable for one year, to buy out the terminating party's interest in ALDURAZYME and in the BioMarin/Genzyme LLC at a specified buyout amount. If such option is not exercised, all rights to ALDURAZYME will be sold and the BioMarin/Genzyme LLC will be dissolved. In the event of termination of the buyout option without exercise by the non-terminating party as described above, all right and title to ALDURAZYME is to be sold to the highest bidder, with the proceeds to be split between Sanofi and us in accordance with our percentage interest in the BioMarin/Genzyme LLC.

If the MMS Agreement is terminated by either party because the other party declared bankruptcy, the terminating party would be obligated to buy out the other party and would obtain all rights to ALDURAZYME exclusively. If the MMS Agreement is terminated by a party because the other party experienced a change of control, the terminating party shall notify the other party, the offeree, of its intent to buy out the offeree's interest in ALDURAZYME and the BioMarin/Genzyme LLC for a stated amount set by the terminating party at its discretion. The offeree must then either accept this offer or agree to buy the terminating party's interest in ALDURAZYME and the BioMarin/Genzyme LLC on those same terms. The party who buys out the other party would then have exclusive worldwide rights to ALDURAZYME. The Amended and Restated Collaboration Agreement between us and Sanofi will automatically terminate upon the effective date of the termination of the MMS Agreement and may not be terminated independently from the MMS Agreement.

If we were obligated or given the option to buy out Sanofi's interest in ALDURAZYME and the BioMarin/Genzyme LLC, and thereby gain exclusive rights to ALDURAZYME, we may not have sufficient funds to do so and we may not be able to obtain the financing to do so. If we fail to buy out Sanofi's interest, we may be held in breach of the agreement and may lose any claim to the rights to ALDURAZYME and the related intellectual property and regulatory approvals. We would then effectively be prohibited from developing and commercializing ALDURAZYME. If this happened, not only would our product revenues decrease, but our share price would also decline.

Risks Related to International Operations

We conduct a significant amount of our sales and operations outside of the U.S., which subjects us to additional business risks that could adversely affect our revenues and results of operations.

A significant portion of the sales of our products are generated from countries other than the U.S., and we expect international markets will continue to be important for the sales of any products approved in the future. We have operations in Canada and in several European, Middle Eastern, Asian, and Latin American countries. We expect that we will continue to expand our international operations in the future. International operations inherently subject us to a number of risks and uncertainties, including:

- the increased complexity and costs inherent in managing international operations;
- diverse regulatory and compliance requirements, and changes in those requirements that could restrict our ability to manufacture, market and sell our products;
- geopolitical and economic instability, such as the instability caused by Russia's invasion of Ukraine;
- diminished protection of intellectual property in some countries outside of the U.S.;
- trade protection measures and import or export licensing requirements;
- difficulty in staffing and managing international operations;

- differing labor regulations and business practices;
- potentially negative consequences from changes in or interpretations of tax laws;
- changes in international medical reimbursement policies and programs;
- financial risks such as longer payment cycles, difficulty collecting accounts receivable, exposure to fluctuations in foreign currency exchange rates and potential currency controls imposed by non-U.S. governments;
- regulatory and compliance risks that relate to maintaining accurate information and control over sales and distributors' and service providers' activities that may fall within the purview of the Foreign Corrupt Practices Act (the FCPA); and
- rapidly evolving global laws and regulations relating to data protection and the privacy and security of commercial and personal information.

Any of these factors may, individually or as a group, have a material adverse effect on our business and results of operations. For example, Russia's invasion of Ukraine and the related impacts to Ukraine's infrastructure and healthcare system has significantly impacted our ability to provide our therapies to patients in Ukraine. Sanctions issued by the U.S. and other countries against Russia and Belarus in response to the attack on Ukraine and related counter-sanctions issued by Russia have made it very difficult for us to operate in Russia and may have a material adverse impact on our ability to sell our products and/or collect receivables from customers in Russia and Belarus.

As we continue to expand our existing international operations, we may encounter new risks. For example, as we focus on building our international sales and distribution networks in new geographic regions, we must continue to develop relationships with qualified local distributors and trading companies. If we are not successful in developing and maintaining these relationships, we may not be able to grow sales in these geographic regions. These or other similar risks could adversely affect our revenues and profitability.

A significant portion of our international sales are made based on special access programs, and changes to these programs could adversely affect our product sales and revenues in these countries.

We make a significant portion of our initial international sales of newly launched products through early access, special access or "named patient sales" programs in markets where we are not required to obtain regulatory approval before establishing these programs. For example, a significant portion of our international sales of VOXZOGO since the product's launch have been made through such programs. The specifics of the programs vary from country to country. Generally, special approval must be obtained to initiate such programs, and in some cases, special approval must be obtained for each patient. The approval normally requires an application to national competent authorities in which the product is intended to be supplied or a lawsuit accompanied by evidence of medical need.

These programs are not well defined in some countries and are subject to changes in requirements, funding levels, unmet medical need and classification of the disease treated by our product. Any change to these programs could adversely affect our ability to sell our products in those countries and delay sales. If the programs are not funded by the respective government, there could be insufficient funds to pay for all patients. Further, governments have and may continue to undertake unofficial measures to limit purchases of our products, including initially denying coverage for purchasers, delaying orders, requiring additional in-country testing and denying or taking excessively long to approve customs clearance. Any such actions could materially delay or reduce our revenues from such countries.

Without the special access programs, we would need to seek full product approval or official reimbursement to commercially market and sell our products in certain jurisdictions. This can be an expensive and time-consuming process and may subject our products to additional price controls. Because the number of patients is so small in some countries, it may not be economically feasible to seek, obtain and maintain a full product approval or official reimbursement, and therefore the sales in such country would be permanently reduced or eliminated. For all of these reasons, if the special access programs that we are currently using are eliminated or restricted, our revenues could be adversely affected.

Our international operations pose currency risks, which may adversely affect our operating results and net income.

A significant and growing portion of our revenues and earnings, as well as our substantial international assets and liabilities, are exposed to changes in foreign exchange rates. As we operate in multiple foreign currencies, including the Euro, the Brazilian Real, the Russian Ruble, the Colombian Peso, the Argentine Peso and several other currencies, changes in those currencies relative to the U.S. Dollar (USD) will impact our revenues and expenses. If the USD were to weaken against another currency, assuming all other variables remained constant, our revenues would increase, having a positive impact on earnings, and our overall expenses would increase, having a negative impact on earnings. Conversely, if the USD were to strengthen against another currency (as was the case for many currencies in 2022), assuming all other variables remained constant, our revenues would decrease, having a negative impact on earnings, and our overall expenses would decrease, having a positive impact on earnings. In addition, because our financial statements are reported in USD, changes in currency exchange rates between the

USD and other currencies have had, and will continue to have, an impact on our results of operations. Therefore, significant changes in foreign exchange rates can impact our results and our financial guidance.

We implement currency hedges intended to reduce our exposure to changes in certain foreign currency exchange rates. However, our hedging strategies may not be successful, and any of our unhedged foreign exchange exposures will continue to be subject to market fluctuations. These risks could cause a material adverse effect on our business, financial position and results of operations and could cause the market value of our common stock to decline.

U.S. export control and economic sanctions may adversely affect our business, financial condition and operating results. Moreover, compliance with such regulatory requirements may increase our costs and negatively impact our ability to sell our products and collect cash from customers.

Our products are subject to U.S. export control laws and regulations, including the U.S. Export Administration Regulations and various economic and trade sanctions regulations administered by the U.S. Treasury Department's Office of Foreign Assets Control (OFAC). Exports of our products and solutions must be made in compliance with these laws and regulations. Changes to these laws and regulations, or to the countries, governments, persons or activities targeted by such laws, could result in decreased use of our products, or hinder our ability to export or sell our products to existing or potential customers, which would likely adversely affect our results of operations, financial condition or strategic objectives. For example, sanctions issued by the U.S. and other jurisdictions against Russia and Belarus in response to the invasion of Ukraine have made it very difficult for us to operate in Russia and may have a material adverse impact on our ability to sell our products and/or collect receivables from customers in Russia and Belarus. Moreover, if we fail to comply with these laws and regulations, we could be subject to substantial civil or criminal penalties, including the possible loss of export or import privileges and fines.

We rely on a general license from OFAC to sell our medicines for eventual use by hospital and clinic end-users in Iran. The use of this OFAC general license requires us to observe strict conditions with respect to products sold, end-user limitations and payment requirements. Although we believe we have maintained compliance with the general license requirements, there can be no assurance that the general license will not be revoked, the general license will be renewed in the future or we will remain in compliance with the general license. A violation of the OFAC general license could result in substantial fines, sanctions, civil or criminal penalties, competitive or reputational harm, litigation or regulatory action and other consequences that might adversely affect our results of operations, financial condition or strategic objectives.

Moreover, U.S. export control and economic sanctions may make operating in certain countries more difficult and expensive. For example, we may be unable to find distributors or financial institutions willing to facilitate the sale of our products and collection of cash from such sales in a cost-effective manner, if at all.

Failure to comply with applicable anti-corruption legislation could result in fines, criminal penalties and materially adversely affect our business, financial condition and results of operations.

We are required to comply with anti-corruption and anti-bribery laws in the jurisdictions in which we operate, including the FCPA in the U.S. and other similar laws in other countries in which we do business. We operate in a number of countries that are recognized to have a reputation for corruption and pose an increased risk of corrupt practices. We also regularly interact with government regulators in many countries, including those that are considered higher risk for corruption, in order to secure regulatory approval to manufacture and distribute our products. The anti-corruption and anti-bribery laws to which we are subject generally prohibit companies and their intermediaries from making improper payments to non-U.S. government officials or other persons for the purposes of influencing official decisions or obtaining or retaining business and/or other benefits. These laws also require us to make and keep books and records that accurately and fairly reflect our transactions and to devise and maintain an adequate system of internal accounting controls. As part of our business, we deal with state-owned business enterprises, the employees and representatives of which may be considered non-U.S. government officials for purposes of applicable anti-corruption laws.

Although we have adopted policies and procedures designed to ensure that we, our employees and third-party agents will comply with such laws, there can be no assurance that such policies or procedures will work effectively at all times or protect us against liability under these or other laws for actions taken by our employees, partners and other third parties with respect to our business. If we are not in compliance with anti-corruption laws and other laws governing the conduct of business with government entities and/or officials (including local laws), we may be subject to criminal and civil penalties and other remedial measures, which could harm our business, financial condition, results of operations, cash flows and prospects. Investigations of any actual or alleged violations of such laws or policies related to us could harm our business, financial condition, results of operations, cash flows and prospects.

Moreover, there has been enhanced scrutiny of company-sponsored patient assistance programs, including insurance premium and co-pay assistance programs and donations to third-party independent charities that provide such assistance. There has also been enhanced scrutiny by governments on reimbursement support offerings, clinical education programs and promotional speaker programs. If we, our third-party agents or donation recipients are deemed to have failed to comply with laws,

regulations or government guidance in any of these areas, we could be subject to criminal or civil sanctions. Any similar violations by our competitors could also negatively impact our industry reputation and increase scrutiny over our business and our products.

We face credit risks from government-owned or sponsored customers outside of the U.S. that may adversely affect our results of operations.

Our product sales to government-owned or supported customers in various countries outside of the U.S. are subject to significant payment delays due to government funding and reimbursement practices. This has resulted and may continue to result in an increase in days sales outstanding due to the average length of time that we have accounts receivable outstanding. If significant changes were to occur in the reimbursement practices of these governments or if government funding becomes unavailable, we may not be able to collect on amounts due to us from these customers and our results of operations would be adversely affected.

Intellectual Property Risks

If we are unable to protect our intellectual property, we may not be able to compete effectively or preserve our market shares.

Where appropriate, we seek patent protection for certain aspects of our technology. Patent protection may not be available for some of the products we are developing. If we must spend significant time and money protecting or enforcing our patents, designing around patents held by others or licensing, potentially for large fees, patents or other proprietary rights held by others, our business and financial prospects may be harmed.

The patent positions of biopharmaceutical products are complex and uncertain. The scope and extent of patent protection for some of our products and product candidates are particularly uncertain because key information on some of our product candidates has existed in the public domain for many years. The composition and genetic sequences of animal and/or human versions of ALDURAZYME, NAGLAZYME and many of our product candidates have been published and are believed to be in the public domain. The chemical structure of 6R-BH4 (the active ingredient in KUVAN) has also been published. Publication of this information may prevent us from obtaining or enforcing patents relating to our products and product candidates, including without limitation composition-of-matter patents, which are generally believed to offer the strongest patent protection.

We own or have licensed patents and patent applications related to our products. However, these patents and patent applications do not ensure the protection of our intellectual property for a number of reasons, including without limitation the following:

- With respect to pending patent applications, unless and until actually issued, the protective value of these applications is impossible to determine. We do not know whether our patent applications will result in issued patents.
- Patents have limited duration and expire.
- Enforcing patents is expensive and may absorb significant time of our management. Management would spend less time and resources on developing products, which could increase our operating expenses and delay product programs.
- Receipt of a patent may not provide much, if any, practical protection. For example, if we receive a patent with a narrow scope, then it will be easier for competitors to design products that do not infringe on our patent.
- The Leahy-Smith America Invents Act of 2011, which reformed certain patent laws in the U.S., may create additional uncertainty. Among the significant changes are switching from a “first-to-invent” system to a “first-to-file” system, and the implementation of new procedures that permit competitors to challenge our patents in the U.S. Patent and Trademark Office after grant.

It is also unclear whether our trade secrets are adequately protected. Our current and former employees, consultants or contractors may unintentionally or willfully disclose trade secrets to competitors. Enforcing a claim that someone else illegally obtained and is using our trade secrets, as with patent litigation, is expensive and time consuming, requires significant resources and has an unpredictable outcome. In addition, courts outside of the U.S. are sometimes less willing to protect trade secrets. Furthermore, our competitors may independently develop equivalent knowledge, methods and know-how, in which case we would not be able to enforce our trade secret rights against such competitors.

In the EU, materials we submit to the EMA in connection with our clinical trials that were traditionally regarded as confidential, proprietary information, such as study protocols, information regarding manufacturing methods and controls, and intermediate data analyses, are now subject to public disclosure. Moreover, clinical trial data submitted to the EMA in our MAAs are also available to the public. We are only permitted to redact from public disclosures commercially confidential information, a standard which is construed narrowly and subject to the interpretation and final decision of the EU regulatory authorities. EU regulations have resulted and will continue to result in the EMA’s public disclosure of certain of our proprietary information related to recently completed and future clinical trials and MAA submissions. The move toward public disclosure of such development

information could adversely affect our business in many ways, including, for example, resulting in the disclosure of our confidential methodologies for development of our products, preventing us from obtaining intellectual property right protection for innovations, requiring us to allocate significant resources to prevent other companies from violating our intellectual property rights, adding even more complexity to processing health data from clinical trials consistent with applicable data privacy regulations, increasing scrutiny of our product candidates and products, and enabling competitors to use our clinical trial information and data to gain approvals for their own products.

Competitors may interfere with our patent process in a variety of ways. Competitors may claim that they invented the claimed invention prior to us or that they filed their application for a patent on a claimed invention before we did. Competitors may also claim that we are infringing on their patents and therefore we cannot practice our technology. Competitors may also contest our patents by showing the patent examiner or a court that the invention was not original, was not novel or was obvious, for example. In litigation, a competitor could claim that our issued patents are not valid or are unenforceable for a number of reasons. If a court agrees, we would not be able to enforce that patent. Moreover, follow-on manufacturers, including generic and biosimilar manufacturers, may use litigation and regulatory means to obtain approval for generic, biosimilar, or other follow-on versions of our products notwithstanding our filed patents or patent applications.

If we are unable to protect our intellectual property, third parties could develop competing products, which could adversely affect our revenues and financial results generally.

Competitors and other third parties may have developed intellectual property that could limit our ability to market and commercialize our products and product candidates, if approved.

Similar to us, competitors continually seek intellectual property protection for their technology. Several of our products, such as ROCTAVIAN, and development programs, focus on therapeutic areas that have been the subject of extensive research and development by third parties for many years. Due to the amount of intellectual property in our field of technology, we cannot be certain that we do not infringe intellectual property rights of competitors or that we will not infringe intellectual property rights of competitors granted or created in the future. For example, if a patent holder believes our product infringes its patent, the patent holder may sue us even if we have received patent protection for our technology. If someone else claims we infringe their intellectual property, we would face a number of issues, including the following:

- Defending a lawsuit takes significant executive resources and can be very expensive.
- If a court decides that our product infringes a competitor’s intellectual property, we may have to pay substantial damages.
- With respect to patents, in addition to requiring us to pay substantial damages, a court may prohibit us from making, selling, offering to sell, importing or using our product unless the patent holder licenses the patent to us. The patent holder is not required to grant us a license. If a license is available, it may not be available on commercially reasonable terms. For example, we may have to pay substantial royalties or grant cross licenses to our patents and patent applications.
- We may need to redesign our product so it does not infringe the intellectual property rights of others.
- Redesigning our product so it does not infringe the intellectual property rights of competitors may not be possible or could require substantial funds and time.

We may also support and collaborate in research conducted by government organizations, hospitals, universities or other educational institutions. These research partners may be unwilling to grant us any exclusive rights to technology or products derived from these collaborations. For example, under the Bayh-Dole Act which only applies to patents for inventions generated from federally funded research, the U.S. Department of Commerce may allow the government to use “march-in” rights for prescription drug patents as a means to control prices.

If we do not obtain required licenses or rights, we could encounter delays in our product development efforts while we attempt to design around other patents or may be prohibited from making, using, importing, offering to sell or selling products requiring these licenses or rights. There is also a risk that disputes may arise as to the rights to technology or products developed in collaboration with other parties. If we are not able to resolve such disputes and obtain the licenses or rights we need, we may not be able to develop or market our products.

Risks Related to Ownership of Our Securities

Our stock price has been and may in the future be volatile, and an investment in our stock could suffer a decline in value.

Our stock price has been and may in the future be volatile. Our valuation and stock price may have no meaningful relationship to current or historical earnings, asset values, book value or many other criteria based on conventional measures of stock value. The market price of our common stock have, and in the future could, fluctuate due to factors including:

- product sales and profitability of our products;
- manufacturing, supply or distribution of our product candidates and products
- progress of our product candidates through the regulatory process and our ability to successfully commercialize any such products that receive regulatory approval;
- results of clinical trials, announcements of technological innovations or new products by us or our competitors;
- generic competition to KUVAN tablets and powder described above in this Risk Factors section or potential generic competition from future competitors;
- government regulatory action affecting our product candidates, our products or our competitors' product candidates and products in both the U.S. and non-U.S. countries;
- developments or disputes concerning patent or proprietary rights;
- general market conditions and fluctuations for the emerging growth and pharmaceutical market sectors;
- economic conditions in the U.S. or abroad;
- negative publicity about us or the pharmaceutical industry;
- changes in the structure of healthcare payment systems;
- cybersecurity incidents experienced by us or others in our industry;
- broad market fluctuations in the U.S., the EU or in other parts of the world;
- actual or anticipated fluctuations in our operating results, including due to timing of large periodic orders for our products by governments in certain countries;
- changes in company assessments or financial estimates by securities analysts;
- certain actions by activist investors that may be threatened or commenced against us;
- acquisitions of products, businesses, or other assets; and
- sales of our shares of stock by us, our significant stockholders, or members of our management or Board of Directors.

Furthermore, the stock markets have recently experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many companies. In some cases, these fluctuations have been unrelated or disproportionate to the operating performance of those companies. In the past, companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. For example, in September 2020, after a substantial drop in our stock price that followed an announcement providing a regulatory update regarding ROCTAVIAN, we and certain of our officers were sued in a putative class action lawsuit alleging violations of the federal securities laws for allegedly making materially false or misleading statements. In addition, in October 2021, after a drop in our stock price that followed an announcement providing a regulatory update regarding BMN 307, we and certain of our current and former officers were sued in a putative class action lawsuit alleging violations of the federal securities laws for allegedly making materially false or misleading statements. We may be the target of additional litigation of this type in the future as well. Securities litigation against us could result in substantial costs and divert our management's time and attention from other business concerns, which could harm our business.

In addition, our stock price can be materially adversely affected by factors beyond our control, such as disruptions in global financial markets or negative trends in the biotechnology sector of the economy, even if our business is operating well.

Conversion of the Notes will dilute the ownership interest of existing stockholders, including holders who had previously converted their Notes, or may otherwise depress the price of our common stock.

The conversion of some or all of the Notes will dilute the ownership interests of existing stockholders. Any sales in the public market of the common stock issuable upon such conversion could adversely affect prevailing market prices of our common stock. In addition, the existence of the Notes may encourage short selling by market participants because the conversion of the Notes could be used to satisfy short positions, or anticipated conversion of the Notes into shares of our common stock could depress the price of our common stock.

The fundamental change repurchase feature of the Notes may delay or prevent an otherwise beneficial attempt to take us over.

The terms of the Notes require us to offer to repurchase the Notes in the event of a fundamental change (as defined in each indenture governing the Notes). A takeover of us would trigger options by the respective holders of the applicable Notes to

require us to repurchase such Notes. This may have the effect of delaying or preventing a takeover of us that would otherwise be beneficial to our stockholders or investors in the Notes.

Anti-takeover provisions in our charter documents and under Delaware law may make an acquisition of us, which may be beneficial to our stockholders, more difficult.

We are incorporated in Delaware. Certain anti-takeover provisions of Delaware law and our charter documents as currently in effect may make a change in control of us more difficult, even if a change in control would be beneficial to the stockholders. Our anti-takeover provisions include provisions in our restated certificate of incorporation and amended and restated bylaws providing that stockholders' meetings may only be called by our Chairman, the lead independent director or the majority of our Board of Directors and that the stockholders may not take action by written consent and requiring that stockholders that desire to nominate any person for election to our Board of Directors or to make any proposal with respect to business to be conducted at a meeting of our stockholders be submitted in appropriate form to our Secretary within a specified period of time in advance of any such meeting. Additionally, our Board of Directors has the authority to issue shares of preferred stock and to determine the terms of those shares of stock without any further action by our stockholders. The rights of holders of our common stock are subject to the rights of the holders of any preferred stock that may be issued. The issuance of preferred stock could make it more difficult for a third party to acquire a majority of our outstanding voting stock. Delaware law also prohibits corporations from engaging in a business combination with any holders of 15% or more of their capital stock until the holder has held the stock for three years unless, among other possibilities, our Board of Directors approves the transaction. Our Board of Directors may use these provisions to prevent changes in the management and control of us. Also, under applicable Delaware law, our Board of Directors may adopt additional anti-takeover measures in the future.

Our amended and restated bylaws designate the Court of Chancery of the State of Delaware and the federal district courts of the U.S. as the exclusive forums for the adjudication of certain disputes, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, or employees.

Our amended and restated bylaws provide that the Court of Chancery of the State of Delaware is the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law:

- any derivative claim or cause of action brought on our behalf;
- any claim or cause of action for breach of a fiduciary duty owed by any current or former director, officer or other employee of BioMarin to us or our stockholders;
- any claim or cause of action against us or any of our current or former directors, officers or other employees arising pursuant to any provision of the General Corporation Law of the State of Delaware, our restated certificate of incorporation or our amended and restated bylaws; any claim or cause of action seeking to interpret, apply, enforce or determine the validity of our restated certificate of incorporation or our amended and restated bylaws;
- any claim or cause of action as to which the General Corporation Law of the State of Delaware confers jurisdiction to the Court of Chancery of the State of Delaware; and
- any claim or cause of action against us or any of our current or former directors, officers or other employees that is governed by the internal affairs doctrine.

This exclusive-forum provision would not apply to suits brought to enforce a duty or liability created by the Securities Act of 1933, as amended, the Exchange Act or any other claim for which the U.S. federal courts have exclusive jurisdiction. In addition, our amended and restated bylaws provide that the federal district courts of the U.S. of America will be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act.

While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our amended and restated bylaws. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

These exclusive forum provisions may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers, and other employees. If a court were to find either of our exclusive forum provisions to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving the dispute in other jurisdictions, all of which could seriously harm our business. Our amended and restated bylaws further provide that any person or entity that acquires any interest in shares of our capital stock will be deemed to have notice of and consented to the provisions of such provisions.

General Risk Factors

We depend upon our key personnel and our ability to attract and retain qualified employees.

Our future growth and success will depend in large part on our continued ability to attract, retain, manage and motivate our employees. The loss of the services of a significant portion of our workforce or any member of our senior management or the inability to hire or retain qualified personnel could adversely affect our ability to execute our business plan and harm our operating results.

Because of the specialized nature of our business, we rely heavily on our ability to attract and retain qualified scientific, technical and managerial personnel. In particular, the loss of one or more of our senior executive officers could be detrimental to us if we do not have an adequate succession plan or if we cannot recruit suitable replacements in a timely manner. While our senior executive officers are parties to employment agreements with us, these agreements do not guarantee that they will remain employed with us in the future. In addition, in many cases, these agreements do not restrict our senior executive officers' ability to compete with us after their employment is terminated. In November 2023, we announced the retirement of Jean-Jacques Bienaimé, our then-current President and Chief Executive Officer, and the appointment of Alexander Hardy as President and Chief Executive Officer, each effective December 1, 2023. If Mr. Hardy's succession as President and Chief Executive Officer is not managed successfully, including his ability to lead a team that can effectively implement our strategic plans, it could disrupt our business and affect our financial condition and operating results. Additionally, on January 11, 2024, we announced that Jeffrey Ajer would step down as our Executive Vice President and Chief Commercial Officer effective July 1, 2024. The recent changes in our management team could cause retention and morale concerns among current employees, as well as operational risks.

The competition for qualified personnel in the pharmaceutical field is intense, and there is a limited pool of qualified potential employees to recruit. Recently, like many other employers in the U.S., we experienced increased employee turnover. Due to the intense competition for talent, we may be unable to continue to attract and retain qualified personnel necessary for the development of our business or to recruit suitable replacement personnel. Additionally, we cannot be sure that the compensation costs of doing so will not adversely affect our operating results, and we may not be able to hire and train employees quickly enough to meet our needs. If we fail to retain employees and effectively manage our hiring needs, our efficiency, ability to meet forecasts, employee morale, productivity, and the success of our strategic plans could suffer, which may have an adverse effect on our business, financial condition, and operating results.

Our success depends on our ability to manage our growth.

Our two newest products, VOXZOGO and ROCTAVIAN, address potentially larger patient populations than most of our other products, and product candidates that we are currently developing or may license or acquire in the future may be intended for similarly larger patient populations than we have historically targeted. In order to continue development of such product candidates and marketing of products with larger markets, we will need to continue expanding our operations. To manage expansion effectively, we need to continue to develop and improve our research and development capabilities, manufacturing and quality capacities, sales and marketing capabilities, financial and administrative systems and standard processes for global operations. For example, strong demand for VOXZOGO in certain markets has outpaced our projections in recent quarters, and we expect to face challenges meeting our current estimates of VOXZOGO demand through the first half of 2024. Our staff, financial resources, systems, procedures or controls may be inadequate to support our operations and may increase our exposure to regulatory, competitive, and corruption risks and our management may be unable to manage successfully current or future market opportunities or our relationships with customers and other third parties.

New tax laws or regulations that are enacted or existing tax laws and regulations that are interpreted, modified or applied adversely to us or our customers may have a material adverse effect on our business and financial condition.

New tax laws or regulations could be enacted at any time, and existing tax laws or regulations could be interpreted, modified or applied in a manner that is adverse to us or our customers, which could adversely affect our business and financial condition. For example, the Tax Cuts and Jobs Act, the Coronavirus Aid, Relief, and Economic Security Act and the Inflation Reduction Act enacted many significant changes to the U.S. tax laws. Future guidance from the Internal Revenue Service and other tax authorities with respect to such legislation may affect us, and certain aspects of such legislation could be repealed or modified in future legislation. Among other changes, the Tax Cuts and Jobs Act amended the Code to require that, for tax years beginning after December 31, 2021, certain research and experimental expenditures be capitalized and amortized over five years if incurred in the United States or fifteen years if incurred in foreign jurisdictions for tax years beginning after December 31, 2021. Although the U.S. Congress has considered legislation that would defer, modify, or repeal the capitalization and amortization requirement, there is no assurance that such changes will be made. If the requirement is not deferred, repealed or otherwise modified, it may increase our cash tax. In addition, it is uncertain if and to what extent various states will conform to federal tax laws. Any future tax legislation could increase our U.S. tax expense and could have a material adverse impact on our business and financial condition.

Moreover, changes in the tax laws of jurisdictions in which we conduct business could arise, including as a result of the base erosion and profit shifting (BEPS) project that is being led by the Organization for Economic Co-operation and Development

(OECD), and other initiatives led by the OECD or the EC. For example, the OECD, which represents a coalition of member countries including the U.S. and other countries in which we have operations, is working on proposals, commonly referred to as "BEPS 2.0", which, if and to the extent implemented, would make important changes to the international tax system. These proposals are based on two "pillars", Pillar One focuses on the allocation of taxing rights in respect of certain profits of multinational enterprises with annual global revenue above 20 billion euros and profitability above 10% to the jurisdictions within which they carry on business (based on the thresholds, we currently expect to be outside the scope of the Pillar One proposals, but could fall within their scope in the future) and Pillar Two imposes a minimum effective tax rate of 15% on certain multinational enterprises that have consolidated revenues of at least 750 million euros in at least two out of the last four years (based on the thresholds, we currently expect that we are likely to fall within the scope of the Pillar Two proposals). A number of countries in which we conduct business have enacted with effect from January 1, 2024, or are in the process of enacting, core elements of Pillar Two rules. The OECD has issued administrative guidance providing transition and safe harbor rules around the implementation of Pillar Two. We are monitoring developments and evaluating the impacts these new rules will have on our tax rate, including eligibility to qualify for these safe harbor rules. It is not uncommon for taxing authorities in different countries to have conflicting views, for instance, with respect to, among other things, the manner in which the arm's length standard is applied for transfer pricing purposes, or with respect to the valuation of intellectual property. If tax authorities successfully challenge our transfer prices as not reflecting arm's length transactions, they could require us to adjust our transfer prices and thereby reallocate our income to reflect these revised transfer prices, resulting in a higher tax liability. In addition, if a country from which income is reallocated does not agree with the reallocation, both that country and the other country to which the income was allocated could tax the same income, potentially resulting in double taxation. If tax authorities were to allocate income to a higher tax jurisdiction, subject our income to double taxation or assess interest and penalties, it would increase our consolidated tax liability, which could adversely affect our business, financial condition, results of operations and cash flows.

If we are found in violation of healthcare laws or privacy and data protection laws, we may be required to pay penalties, be subjected to scrutiny by regulators or governmental entities, or be suspended from participation in government healthcare programs, which may adversely affect our business, reputation, financial condition and results of operations.

We are subject to various healthcare laws and regulations in the U.S. and internationally, including anti-kickback laws, false claims laws, data privacy and security laws, and laws related to ensuring compliance. In the U.S., the federal Anti-Kickback Statute makes it illegal for any person or entity, including a pharmaceutical company, to knowingly and willfully offer, solicit, pay or receive any remuneration, directly or indirectly, in exchange for or to induce the referral of business, including the purchase, order or prescription of a particular drug, for which payment may be made under federal healthcare programs, such as Medicare and Medicaid. Under the federal Anti-Kickback Statute and related regulations, certain arrangements are deemed not to violate the federal Anti-Kickback Statute if they fit within a statutory exception or regulatory safe harbor. However, the exceptions and safe harbors are drawn narrowly, and practices that involve remuneration not intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exception or safe harbor. Our practices may not in all cases meet all of the criteria for safe harbor protection from Anti-Kickback liability, although we seek to comply with these safe harbors. Many states have adopted laws similar to the federal Anti-Kickback Statute, some of which apply to referral of patients for healthcare services reimbursed by any source, not just governmental payers. We recently received a subpoena from the U.S. Department of Justice requesting that we produce certain documents regarding our sponsored testing programs relating to VIMIZIM and NAGLAZYME. We have produced documents in response to the subpoena and are cooperating fully, but there is no assurance that such sponsored testing programs, or our other operations or programs, will not be found to violate such laws.

Federal and state false claims laws, including the civil False Claims Act and the Civil Monetary Penalties Law, prohibit any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid, or knowingly making, using, or causing to be made or used, a false record or statement to avoid, decrease or conceal an obligation to pay money to the federal government. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws.

Under the Health Insurance Portability and Accountability Act of 1996 (HIPAA), we also are prohibited from, among other things, knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private payers, or 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.

In addition, federal and state healthcare legislation have strengthened these laws in the U.S. For example, the PPACA, among other things, amends the intent requirement of the federal Anti-Kickback Statute and criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of these statutes or specific intent to violate them in order to commit a violation. Moreover, the PPACA provides that 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 civil False Claims Act.

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, also imposes obligations, including mandatory contractual terms, on certain types of individuals and entities, with respect to safeguarding the privacy, integrity, availability, security and transmission of individually identifiable health information. Many state and non-U.S. laws also govern the privacy and security of health information. They often differ from each other in

significant ways and often are not preempted by HIPAA, thus complicating compliance efforts. The global data protection landscape is rapidly evolving, and implementation standards and enforcement practices are likely to remain uncertain for the foreseeable future. In the U.S., state privacy laws and regulations impose restrictive requirements regulating the use and disclosure of health information and other sensitive personal information that is not subject to HIPAA. For example, California enacted the California Consumer Privacy Act (CCPA), which took effect on January 1, 2020. The CCPA gives California consumers expanded rights to access and delete their personal information, opt out of certain personal information sales, and receive detailed information about how their personal information is used. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. The CCPA was expanded substantially on January 1, 2023 when the California Privacy Rights Act of 2020 (CPRA) took effect and amended the CCPA. Following the CPRA amendments, the CCPA, among other things, gives consumers the ability to limit use of information deemed to be sensitive, increase the maximum penalties for violations concerning consumers under age 16, expands an individual's private right of action and establishes the California Privacy Protection Agency to implement and enforce the new law and impose administrative fines.

Other U.S. states have recently adopted consumer data protection and privacy laws, and more U.S. states may do so in the future. This creates the potential for a patchwork of overlapping but different state laws and could mark the beginning of a trend toward more stringent privacy legislation in the U.S., which could increase our potential liability and adversely affect our business, financial condition, and results of operations. Many other states are considering proposed comprehensive data privacy legislation and all 50 states have passed some form of legislation relating to privacy or cybersecurity.

Aspects of the CCPA, CPRA and similar laws in other states and their interpretation and enforcement remain uncertain. The potential effects of these laws are far-reaching and may require us to modify our data processing practices and policies and to incur substantial costs and expenses in an effort to comply. Complying with these or other similar laws, regulations, amendments to or re-interpretations of existing laws and regulations, and contractual or other obligations relating to privacy, data protection, data transfers, data localization, or information security may require us to make changes to our services to enable us or our customers to meet new legal requirements, incur substantial operational costs, modify our data practices and policies, and restrict our business operations. Any actual or perceived failure by us to comply with these laws, regulations, or other obligations may lead to significant fines, penalties, regulatory investigations, lawsuits, significant costs for remediation, damage to our reputation, or other liabilities.

The European Regulation 2016/679, known as the General Data Protection Regulation (GDPR), as well as EEA Member State legislations supplementing such regulation, apply to the processing of personal data of individuals located in the EEA, including health-related information, by companies located in the EEA, or in certain circumstances, by companies located outside of the EEA. These laws impose strict obligations on the ability to collect, record, store, disclose, use and transmit personal data, including health-related information. These include several requirements relating to (i) obtaining, in some situations, the informed consent of the individuals to whom the personal data relates, (ii) the information provided to the individuals about how their personal information is used, (iii) ensuring the security and confidentiality of the personal data, (iv) the obligation to notify regulatory authorities and affected individuals of personal data breaches, (v) extensive internal privacy governance obligations, and (vi) obligations to honor rights of individuals in relation to their personal data (for example, the right to access, correct and delete their data). Switzerland has adopted similar restrictions.

The GDPR and other European data protection laws generally restrict the transfer of personal information from Europe, including the EEA and Switzerland, to the U.S. and most other countries unless the U.S. companies participate in the EU-U.S. Data Privacy Framework in accordance with the EC's adequacy decision adopted on July 10, 2023, or have implemented specific safeguards to protect the transferred personal information. U.S. companies can join the EU-U.S. Data Privacy Framework by committing to comply with a detailed set of privacy obligations and U.S. companies that do not fall under the EU-U.S. Data Privacy Framework must implement certain specific safeguards. One of the primary safeguards allowing U.S. companies to import personal information from the EEA has been the EC's Standard Contractual Clauses (SCCs). However, the Court of Justice of the EU (CJEU) issued a decision that called into question whether the SCCs can lawfully be used for transfers of personal information from Europe to the United States or most other countries. After the mentioned CJEU judgment, new sets of SCCs were published on June 4, 2021. Most importantly, the use of SCCs does not any longer automatically ensure compliance with the GDPR. Instead, companies remain required to conduct a data transfer impact assessment for each transfer, which adds a compliance burden.

Potential pecuniary fines for noncompliance with the GDPR may be up to the greater of €20 million or 4% of annual global revenue. The GDPR has increased our responsibility and liability in relation to personal data that we process and has increased our compliance costs. The EU regulations that make certain materials we submit to the EMA in connection with our clinical trials subject to public disclosure have increased the risk that we may unintentionally disclose personal information protected under the GDPR and thereby incur associated penalties and suffer reputational damage.

In addition to the U.S. and European countries, other countries in which we operate have also enacted data privacy laws or may do so in the future. For example, Brazil's General Data Protection Law (LGPD), which is modeled on the GDPR, took effect in 2020.

Substantial new laws and regulations affecting compliance have also been adopted in the U.S. and certain non-U.S. countries, which may require us to modify our business practices with healthcare practitioners. For example, in the U.S., the

PPACA, through the Physician Payments Sunshine Act, requires certain drug, biologicals and medical supply manufacturers to collect and report to CMS information on payments or transfers of value to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), other health care professionals (such as physicians assistants and nurse practitioners), and teaching hospitals, as well as investment and ownership interests held by such physicians and their immediate family members during the preceding calendar year. In addition, there has been a recent trend of increased state regulation of payments made to physicians. Certain states and/or local jurisdictions mandate implementation of compliance programs, compliance with the Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers and the Pharmaceutical Research and Manufacturers of America (PhRMA) Code on Interactions with Healthcare Professionals, the registration of pharmaceutical sales representatives and/or the tracking and reporting of gifts, compensation and other remuneration to physicians, marketing expenditures, and drug pricing. Likewise, in many non-U.S. countries there is an increasing focus on the relationship between drug companies and healthcare practitioners. Recently enacted non-U.S. legislation creates reporting obligations on payments, gifts and benefits made to these professionals. Outside the U.S., interactions between pharmaceutical companies and health care professionals are also governed by strict laws, such as national anti-bribery laws of European countries, national sunshine rules, regulations, industry self-regulation codes of conduct and physicians' codes of professional conduct. The shifting regulatory environment and the need to implement systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the costs of maintaining compliance and the possibility that we may violate one or more of the requirements and be subject to fines or sanctions.

Due to the breadth of the healthcare and privacy and data protection laws described above, the narrowness of available statutory and regulatory exceptions and safe harbors and the increased focus by law enforcement authorities in enforcing such laws, our business activities could be subject to challenge under one or more of such laws. If we are found in violation of one of these laws, we may be subject to significant criminal, civil or administrative sanctions, including damages, fines, disgorgement, imprisonment, contractual damages, reputational harm, public reprimands, diminished profits and future earnings, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, curtailment of our operations, and debarment, suspension or exclusion from participation in government healthcare programs, any of which could adversely affect our business, financial condition and results of operations.

If product liability lawsuits are successfully brought against us, we may incur substantial liabilities.

We are exposed to the potential product liability risks inherent in the testing, manufacturing and marketing of human pharmaceuticals. We currently maintain insurance against product liability lawsuits for the commercial sale of our products and for the clinical trials of our product candidates. Pharmaceutical companies must balance the cost of insurance with the level of coverage based on estimates of potential liability. Historically, the potential liability associated with product liability lawsuits for pharmaceutical products has been unpredictable. Although we believe that our current insurance is a reasonable estimate of our potential liability and represents a commercially reasonable balancing of the level of coverage as compared to the cost of the insurance, we may be subject to claims in connection with our clinical trials and commercial use of our products and product candidates for which our insurance coverage may not be adequate and we may be unable to avoid significant liability if any product liability lawsuit is brought against us. If we are the subject of a successful product liability claim that exceeds the limits of any insurance coverage we obtain, we may incur substantial charges that would adversely affect our earnings and require the commitment of capital resources that might otherwise be available for the development and commercialization of our product programs.

In the EU, new rules on liability of defective products were proposed on September 28, 2022. If adopted, these rules will make it easier for patients to claim damages for defective products, for example by alleviating their burden of proof.

We rely significantly on information technology systems and any failure, inadequacy, interruption or security lapse of that technology, including any cybersecurity incidents, could harm our ability to operate our business effectively and have a material adverse effect on our business, reputation, financial condition, and results of operations.

We rely significantly on our information technology systems, including enterprise resource planning (ERP), production management, and other information systems, to effectively manage and maintain our operations, inventory and internal reports, to manufacture and ship products to customers and to timely invoice them. Any failure, inadequacy or interruption of that infrastructure or security lapse (whether intentional or inadvertent) of that technology, including cybersecurity incidents or attacks, could harm our ability to operate our business effectively.

We are currently preparing to implement a new global ERP system, which will replace existing operating and financial systems. The preparation and implementation of a new ERP system has, and will continue to, require significant investment of capital and human resources. Our results of operations could be adversely affected if we experience delays or cost overruns during the implementation process, or if the ERP system or associated process changes do not give rise to the benefits that we expect. Potential failure or flaws in the new ERP system may pose risks to our ability to operate successfully and efficiently and failure to implement the appropriate internal controls with respect to the new ERP system may result in the system producing inaccurate or unreliable information. Any disruptions, delays or deficiencies in the design or implementation of the new ERP system or related internal controls, or in the performance of legacy information technology systems, could result in potentially much higher costs than

we had incurred and adversely affect our ability to effectively fulfill contractual obligations, file related government reports in a timely manner, operate and manage our business or otherwise affect our controls environment. Any of these consequences could have an adverse effect on our results of operations and financial condition.

In addition, our technology systems, including our cloud technologies, continue to increase in multitude and complexity, making them potentially vulnerable to breakdown, cyberattack and other disruptions. Potential problems and interruptions associated with the implementation of new or upgraded technology systems or with maintenance or adequate support of existing systems could disrupt or reduce the efficiency of our operations and expose us to greater risk of security breaches. Cybersecurity incidents resulting in the failure of our enterprise resource planning system, production management or other systems to operate effectively or to integrate with other systems, or a breach in security or other unauthorized access or unavailability of these systems or those of any third parties in our supply chain or on whom we otherwise depend, have occurred in the past and may affect our ability in the future to manage and maintain our operations, inventory and internal reports, and result in delays in product fulfillment and reduced efficiency of our operations.

As part of our business, we collect, store, and transmit large amounts of confidential information, proprietary data, intellectual property, and personal data. The information and data processed and stored in our technology systems, and those of our research collaborators, CROs, contract manufacturers, suppliers, distributors, or other third parties on whom we depend to operate our business, may be vulnerable to loss, damage, denial-of-service, unauthorized access or misappropriation. Data security incidents may be the result of unauthorized or unintended activity (or lack of activity) by our employees, contractors, or others with authorized access to our network or malware, hacking, business email compromise, phishing, ransomware or other cyberattacks directed by third parties. While we have implemented measures to protect our information and data stored in our technology systems and those of the third parties that we rely on, our efforts may not be successful.

We have experienced and may continue to experience cybersecurity incidents, although to our knowledge we have not experienced any material incident or interruption to date. If such a significant event were to occur, it could result in a material disruption of our development programs and commercial operations, including due to a loss, corruption or unauthorized disclosure of our trade secrets, personal data or other proprietary or sensitive information. Further, these cybersecurity incidents can lead to the public disclosure of personal information (including sensitive personal information) of our employees, clinical trial patients and others and result in demands for ransom or other forms of blackmail. Such attacks, including phishing attacks and attempts to misappropriate or compromise confidential or proprietary information or sabotage enterprise IT systems, are of ever-increasing levels of sophistication and are made by groups and individuals with a wide range of motives (including industrial espionage) and expertise, including by organized criminal groups, “hacktivists”, nation states and others. Moreover, the costs to us to investigate and mitigate cybersecurity incidents could be significant. For example, the loss of clinical trial data could result in delays in our product development or regulatory approval efforts and significantly increase our costs to recover or reproduce the data. Any security breach that results in the unauthorized access, use or disclosure of personal data may require us to notify individuals, governmental authorities, credit reporting agencies, or other parties pursuant to privacy and security laws and regulations or other obligations. Such a security compromise could harm our reputation, erode confidence in our information security measures, and lead to regulatory scrutiny. To the extent that any disruption or security breach resulted in a loss of, or damage to, our data or systems, or inappropriate disclosure of confidential, proprietary or personal information, we could be exposed to a risk of loss, enforcement measures, penalties, fines, indemnification claims, litigation and potential civil or criminal liability, which could materially adversely affect our business, financial condition and results of operations.

Not all our contracts contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our data privacy and security obligations. We cannot be sure that our insurance coverage will be adequate or sufficient to protect us from or to mitigate liabilities arising out of our privacy and security practices, that such coverage will continue to be available on commercially reasonable terms or at all, or that such coverage will pay future claims.

Further, the SEC has adopted new rules that require us to provide greater disclosures around proactive security protections that we employ and reactive issues (e.g., security incidents). Any such disclosures, including those under state data breach notification laws, can be costly, and the disclosures we make to comply with, or the failure to comply with, such requirements could lead to adverse consequences.

If a natural disaster, terrorist or criminal activity or other unforeseen event caused significant damage to our facilities or those of our third-party manufacturers and suppliers or significantly disrupted our operations or those of our third-party manufacturers and suppliers, we may be unable to meet demand for our products and lose potential revenue, have reduced margins, or be forced to terminate a program.

The occurrence of an earthquake or other catastrophic disaster could cause damage to our facility and equipment, or that of our third-party manufacturers or single-source suppliers, which could materially impair the ability for us or our third-party manufacturers to manufacture our products and product candidates. Our Galli Drive facility, located in Novato, California, is currently our only manufacturing facility for ALDURAZYME, NAGLAZYME, VOXZOGO and PALYNZIQ and is one of two manufacturing facilities for BRINEURA and VIMIZIM. Our gene therapy manufacturing facility is also located in Novato, California, and it is currently our only manufacturing facility to support ongoing ROCTAVIAN clinical development activities and commercial

demand for ROCTAVIAN. These facilities are located in the San Francisco Bay Area near known earthquake fault zones and are vulnerable to significant damage from earthquakes. We, the third-party manufacturers with whom we contract and our single-source suppliers of raw materials, which include many of our critical raw materials, are also vulnerable to damage from other types of disasters, including fires, explosions, floods, and similar events. If any disaster were to occur, or any terrorist or criminal activity caused significant damage to our facilities or the facilities of our third-party manufacturers and suppliers, our ability to manufacture our products, or to have our products manufactured, could be seriously, or potentially completely, impaired, and our commercialization efforts and revenues could be seriously impaired.

Moreover, other unforeseen events, such as power outages, could significantly disrupt our operations or those of our third-party manufacturers and suppliers, which could result in damage to our facilities and significant delays in the manufacture of our products and adversely impact our commercial operations and revenues. The insurance that we carry, the inventory that we maintain and our risk mitigation plans may not be adequate to cover our losses resulting from disasters or other business interruptions.

Our business is affected by macroeconomic conditions.

Various macroeconomic factors could adversely affect our business and the results of our operations and financial condition, including changes in inflation, interest rates, or foreign currency exchange rates, natural disasters, geopolitical instability resulting from war, terrorism and other violence, such as the instability caused by Russia's invasion of Ukraine, effects of potential global public health threats and overall economic conditions and uncertainties, including those resulting from the current and future conditions in the global financial markets and volatility and disruptions in the equity and debt markets. For instance, COVID-19 previously adversely affected our ability to source materials and supplies. Inflation (such as that recently observed in the U.S. and elsewhere) has increased our business costs and could become more significant in the future, and it may not be feasible to pass price increases on to our customers due to the process by which healthcare providers are reimbursed for our products by the government. Interest rates, the liquidity of the credit markets and the volatility of the capital markets could also affect the value of our investments and our ability to liquidate our investments in order to fund our operations. We purchase or enter into a variety of financial instruments and transactions, including investments in commercial paper, the extension of credit to corporations, institutions and governments and hedging contracts. If any of the issuers or counterparties to these instruments were to default on their obligations, it could materially reduce the value of the transaction and adversely affect our cash flows.

We sell our products in countries that face economic volatility and weakness. Although we have historically collected receivables from customers in those countries, sustained weakness or further deterioration of the local economies and currencies may cause customers in those countries to be unable to pay for our products. Additionally, if one or more of these countries were unable to purchase our products, our revenues would be adversely affected.

Interest rates and the ability to access credit markets could also adversely affect the ability of our customers/distributors to purchase, pay for and effectively distribute our products, which could limit our ability to obtain sufficient materials and supplies necessary for production of our therapies. Similarly, these macroeconomic factors could affect the ability of our contract manufacturers, sole-source or single-source suppliers to remain in business or otherwise manufacture or supply product. Failure by any of them to remain a going concern could affect our ability to manufacture products.

Additionally, effects of any pandemic or other global public health threat on all aspects of our business and operations and the duration of such effects are highly uncertain and difficult to predict. For instance, a global pandemic could result in significant disruption of global financial markets, which could reduce our ability to access capital and could negatively affect our liquidity and the liquidity and stability of markets for our common stock and Notes. In addition, a recession, further market correction or depression resulting from a future global public health threat could materially adversely affect our business and the value of our common stock and Notes.

To the extent macroeconomic conditions continue to adversely affect our business and financial results, they may also have the effect of heightening many of the other risks described in this Risk Factors section, such as those relating to our conducting a significant amount of our sales and operations outside of the U.S., exposure to changes in foreign exchange rates, our need to generate sufficient cash flows to service our indebtedness and finance our operations and the volatility of our stock price.

Item 1B. Unresolved Staff Comments

None.

Item 1C. Cybersecurity

Risk Management and Strategy

We have implemented and maintain various information security processes designed to identify, assess and manage material risks from cybersecurity threats to our critical computer networks, third-party hosted services, communications systems, hardware and software, and our critical data, including, among other things, intellectual property, trade secrets, confidential information that is proprietary, strategic or competitive in nature, and personal data (collectively, Information Systems and Data).

Our cybersecurity risk management program leverages the National Institute of Standards and Technology (NIST) cybersecurity framework. Our cybersecurity operations team identifies and assesses risks from cybersecurity threats by monitoring and evaluating our threat environment and the Company's risk profile. We use various methods and security tools designed to help prevent, identify, protect, detect, escalate, respond, and recover from identified vulnerabilities and security incidents in a timely manner.

Depending on the technology environment, we implement and maintain various technical, physical, and organizational measures, in the form of policies, standards, processes, and technical capabilities, designed to manage and mitigate material risks from cybersecurity threats to our Information Systems and Data, including, among other things, internal reporting, annual and ongoing cybersecurity awareness training for employees, mechanisms to detect and monitor unusual network activity, as well as threat detection, containment, incident response and backup recovery tools.

Our assessment and management of material risks from cybersecurity threats are integrated into the Company's overall risk management processes. As part of such process, we conduct tests of our cybersecurity program on a regular basis that are designed to identify cybersecurity risks associated with our technology environment. We use third-party security service providers and cybersecurity consultants to assist us from time to time to identify, assess, and manage material risks from cybersecurity threats and review our cybersecurity program. Our internal audit team also conducts audits to evaluate the effectiveness of our cybersecurity program and improve our security measures and planning. The results of such reviews are reflected in the cybersecurity risk register and certain members of our senior management evaluates material risks from cybersecurity threats against our overall business objectives and reports to the Audit Committee (Audit Committee) of the Board of Directors (Board), which evaluates our overall enterprise risk, as well as to the full Board.

We use third-party service providers to perform a variety of functions throughout our business, such as research collaborators, contract research organizations, contract manufacturers, suppliers, and distributors. Depending on the nature of the services provided, certain providers are subject to cybersecurity risk assessments at the time of onboarding and upon contract renewal. We also use various inputs to assess the risk of our third-party service providers, including information supplied by them. Depending on the sensitivity of the Information Systems and Data at issue, and the identity of the provider, our vendor management process may involve various levels of assessment designed to help identify cybersecurity risks associated with a provider and impose contractual obligations related to cybersecurity on the provider.

While we have not, as of the date of this Annual Report on Form 10-K, experienced a cybersecurity incident that resulted in a material adverse impact to our business or operations, there can be no guarantee that we will not experience such an incident in the future. For a description of the risks from cybersecurity threats that may materially affect the Company and how they may do so, please see "Risk Factors" included in Part I, Item 1A of this Annual Report on Form 10-K, including "We rely significantly on information technology systems and any failure, inadequacy, interruption or security lapse of that technology, including any cybersecurity incidents, could harm our ability to operate our business effectively and have a material adverse effect on our business, reputation, financial condition, and results of operations."

Governance

Our Board has ultimate oversight of cybersecurity risk, which it manages as part of its general risk oversight function. The Board satisfies its responsibility to oversee cybersecurity risk through full reports by the Chair of the Audit Committee chair regarding such committee's considerations and actions, as well as through regular reports directly from officers responsible for oversight of risks. The Audit Committee is responsible for overseeing Company's cybersecurity risk management processes, including oversight and mitigation of risks from cybersecurity threats. The Board and the Audit Committee receives periodic reports, summaries, and presentations from our senior management, including the Chief Information Officer and Global Head of Cybersecurity, concerning the Company's significant cybersecurity threats and risk and the processes the Company has implemented to address them.

We recently established the Executive Cybersecurity Committee (ECC), which is comprised of our Chief Financial Officer, Chief Information Officer, Chief Legal Officer, Chief Accounting Officer, and Global Head of Cybersecurity, with the goal of providing oversight of the Company's cybersecurity program. The ECC is responsible for, among other things, evaluating and determining the materiality of cybersecurity incidents as well as reviewing and approving any public disclosures with respect to material cybersecurity incidents. Our cybersecurity incident response policy is designed for our cybersecurity operations team, which is led by our Global Head of Cybersecurity who works in conjunction with the cross-functional incident response team, to escalate certain cybersecurity incidents to the ECC depending on the circumstances. The ECC also has the responsibility of reporting to the Board and/or the Audit Committee.

We maintain a Cybersecurity Risk Committee (CRC) that is comprised of management level representatives from key organizations and functions within the Company and led by our Global Head of Cybersecurity. The CRC is responsible for our enterprise-wide cybersecurity risk management framework established by certain members of our senior management, including the review and approval of significant strategies, policies, procedures, processes, controls, and systems designed to identify, assess, monitor, and report the major risk factors facing the Company. In addition, the CRC provides guidance to senior management on risk appetite, risk profile and approves the effectiveness of the Company's enterprise-wide cybersecurity risk framework and such other duties as directed by the Board. The CRC also assists in the oversight of decisions that affect cybersecurity compliance with applicable laws, regulations, and corporate policies.

Our cybersecurity risk assessment and management processes are implemented and maintained by certain members of Company management, including the Chief Information Officer, who reports to the CFO. Our Chief Information Officer has nearly 25 years of industry experience and has been with us since 2008. Our Global Head of Cybersecurity has over 25 years of cybersecurity and privacy experience, including serving in similar roles leading and overseeing cybersecurity programs at other public companies.

Item 2. Properties

The following table contains information about our significant owned and leased properties as of December 31, 2023:

Location	Approximate Square Feet	Use	Lease Expiration Date
San Rafael facility, San Rafael, California	407,300	Corporate headquarters, laboratory and office	Owned property
Several facilities in Novato, California	293,300	Clinical and commercial manufacturing, laboratory and office	Owned property
Several leased facilities in Novato, California	158,600	Office and warehouse	2027
Shanbally facility, Cork, Ireland	260,700	Manufacturing, laboratory and office	Owned property

We expect that these properties, together with our other smaller leased office facilities in various countries, will be adequate for our operations for the foreseeable future.

Item 3. Legal Proceedings

On September 25, 2020, a purported shareholder class action lawsuit was filed against us, our Chief Executive Officer, our President of Worldwide Research and Development and our Chief Financial Officer in the United States District Court in the Northern District of California, alleging violations under Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 as amended (the Exchange Act). The complaint alleges that we made materially false or misleading statements regarding the clinical trials and Biologics License Application (BLA) for ROCTAVIAN (formerly known as valoctocogene roxaparvovec) by purportedly failing to disclose that differences between the Company's Phase 1/2 and Phase 3 clinical studies limited the ability of the Phase 1/2 study to support ROCTAVIAN's durability of effect and, as a result, that it was foreseeable that the Food and Drug Administration (FDA) would not approve the BLA without additional data. The complaint seeks an unspecified amount of damages, prejudgment and post-judgment interest, attorneys' fees, expert fees, and other costs. The lead plaintiff filed an amended complaint in February 2021, dropping our Chief Financial Officer as a defendant, and asserting that the Company misled investors about the progress of the FDA's review of our BLA for ROCTAVIAN. On April 22, 2021, we moved to dismiss the amended complaint. On January 6, 2022, the court denied our motion to dismiss. We answered the amended complaint on February 15, 2022. Plaintiff filed a motion for class certification on October 17, 2022. We filed an opposition to plaintiff's motion for class certification on January 27, 2023. On March 21, 2023, the Court entered an order staying all proceedings and vacating all deadlines because the parties

agreed to settle the case through a binding term sheet. The Court preliminarily approved the settlement on June 8, 2023. On November 14, 2023, the court granted final approval of the settlement and entered final judgment.

On October 22, 2021, a purported securities class action lawsuit was filed against us, our Chief Executive Officer, our current and prior Chief Financial Officers, and our President of Worldwide Research & Development in the United States District Court for the Northern District of California, alleging violations under Sections 10(b) and 20(a) of the Exchange Act. The complaint alleges that we made materially false or misleading statements regarding BMN 307 by purportedly failing to disclose information about BMN 307's safety profile, and by purportedly overstating BMN 307's clinical and commercial prospects. The complaint seeks an unspecified amount of damages, pre-judgment and post-judgment interest, attorneys' fees, expert fees, and other costs. The Court appointed lead plaintiffs and lead counsel on January 10, 2022. Lead plaintiffs filed an amended complaint on March 25, 2022. We filed a motion to dismiss the amended complaint on May 25, 2022. On January 19, 2023, the Court granted our motion to dismiss the complaint without prejudice. On February 21, 2023, the court dismissed the complaint with prejudice at plaintiffs' request. Plaintiffs appealed the court's January 19, 2023 order to the United State Court of Appeals for the Ninth Circuit and filed their opening brief on June 23, 2023. We filed our answering brief on August 23, 2023. Plaintiffs filed their reply brief on October 13, 2023. On February 15, 2024, the United States Court of Appeals for the Ninth Circuit affirmed the district court's dismissal.

On January 19, 2023 and May 30, 2023, certain of our officers and directors were named as defendants in two shareholder derivative actions filed in the Delaware Court of Chancery. The complaints assert, inter alia, breach of fiduciary duty claims arising from the facts underlying the securities class action related to ROCTAVIAN. The complaints seek unspecified monetary damages, internal governance reforms by the Company, attorneys fees and costs, and any other relief the court may deem just and proper. The parties in the derivative lawsuits have entered into a stipulation of settlement, that, subject to final approval by the Court of Chancery, will resolve the derivative lawsuits.

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 is listed under the symbol "BMRN" on the Nasdaq Global Select Market.

We have never paid any cash dividends on our common stock and we do not anticipate paying cash dividends in the foreseeable future.

Recent Sales of Unregistered Securities

None.

Issuer Purchases of Equity Securities

None.

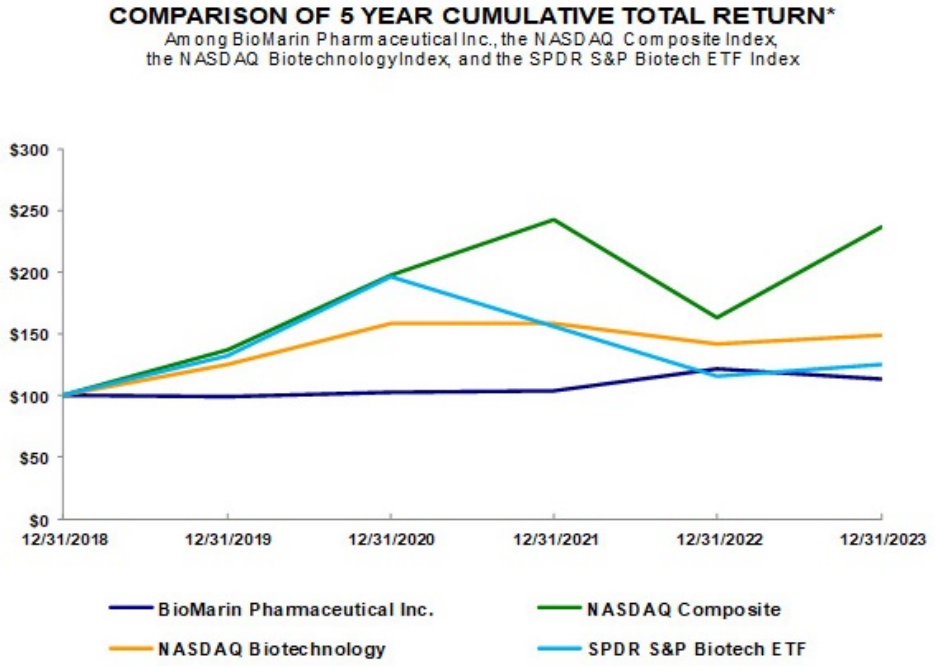
Holders

As of February 16, 2024, there were 34 holders of record of 188,675,622 outstanding shares of our common stock.

Performance Graph

The following is not deemed "filed" with the Securities and Exchange Commission and is not to be incorporated by reference into any filing we make under the Securities Act of 1933, as amended, whether made before or after the date hereof and irrespective of any general incorporation by reference language in such filing.

The following graph shows the value of an investment in BioMarin common stock, the Nasdaq Composite Index, the Nasdaq Biotechnology Index, and the Standard and Poor's (S&P) Depository Receipts S&P Biotech Exchange-traded Funds Index (SPDR S&P Biotech ETF), assuming the investment of $100.00 at the beginning of the period and the reinvestment of dividends, if any. Our common stock is traded on the Nasdaq Global Select Market and is a component of the Nasdaq Composite Index, the Nasdaq Biotechnology Index and the SPDR S&P Biotech ETF. The comparisons shown in the graph are based upon historical data and we caution that the stock price performance shown in the graph is not indicative of, nor intended to forecast, the potential future performance of our stock.



* $100 invested on December 31, 2018 in stock or index, including reinvestment of dividends

	2018	2019	2020	2021	2022	2023
BioMarin Pharmaceutical Inc.	$ 100.00	$ 99.30	$ 102.98	$ 103.76	$ 121.54	$ 113.24
Nasdaq Composite Index	$ 100.00	$ 136.69	$ 198.10	$ 242.03	$ 163.28	$ 236.17
Nasdaq Biotechnology	$ 100.00	$ 125.11	$ 158.17	$ 158.20	$ 142.19	$ 148.72
SPDR S&P Biotech ETF	$ 100.00	$ 132.56	$ 196.63	$ 156.42	$ 115.96	$ 124.77

Item 6. [Reserved]

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

The following Management's Discussion and Analysis of Financial Condition and Results of Operations (MD&A) is intended to help the reader understand our results of operations and financial condition. MD&A is provided as a supplement to, and should be read in conjunction with, our audited Consolidated Financial Statements and the accompanying notes to the Consolidated Financial Statements and other disclosures included in this Annual Report on Form 10-K, including the disclosures under "Risk Factors" in Part I, Item 1A of this Annual Report on Form 10-K. These risks and uncertainties could cause actual results to differ significantly from those projected in forward-looking statements contained in this report or implied by past results and trends. Forward-looking statements are statements that attempt to forecast or anticipate future developments in our business, financial condition or results of operations. See the section titled "Forward-Looking Statements" that appears at the beginning of this Annual Report on Form 10-K. These statements, like all statements in this report, speak only as of the date of this Annual Report on Form 10-K (unless another date is indicated), and, except as required by law, we undertake no obligation to update or revise these statements in light of future developments. Our Consolidated Financial Statements have been prepared in accordance with United States (U.S.) generally accepted accounting principles (GAAP) and are presented in U.S. Dollars (USD).

Overview

Founded in 1997, we are a global biotechnology company dedicated to transforming lives through genetic discovery. We develop and commercialize targeted therapies that address the root cause of genetic conditions. Our robust research and development capabilities have resulted in multiple innovative commercial therapies for patients with rare genetic disorders. Our distinctive approach to drug discovery has produced a diverse pipeline of commercial, clinical, and pre-clinical candidates that address a significant unmet medical need, have well-understood biology, and provide an opportunity to be first-to-market or offer a substantial benefit over existing treatment options. A summary of our commercial products, as of December 31, 2023, is provided below:

Commercial Products	Indication
Enzyme products:	
VIMIZIM (elosulfase alpha)	Mucopolysaccharidosis (MPS) IVA
NAGLAZYME (galsulfase)	MPS VI
PALYNZIQ (pegvaliase-pqpz)	Phenylketonuria (PKU)
BRINEURA (cerliponase alfa)	Neuronal ceroid lipofuscinosis type 2 (CLN2)
ALDURAZYME (laronidase)	MPS I
Other products:	
VOXZOGO (vosoritide)	Achondroplasia
KUVAN (sapropterin dihydrochloride)	PKU
ROCTAVIAN (valoctocogene roxaparvovec)	Severe Hemophilia A

(In millions of U.S. Dollars, except as otherwise disclosed)

2023 Financial Highlights

Key components of our results of operations include the following:

	Twelve Months Ended December 31,		
	2023	2022	2021
Total revenues	$ 2,419.2	$ 2,096.0	$ 1,846.3
Cost of sales	$ 514.9	$ 483.7	$ 470.5
Research and development (R&D) expense	$ 746.8	$ 649.6	$ 628.8
Selling, general and administrative (SG&A) expense	$ 937.3	$ 854.0	$ 759.4
Gain on sale of nonfinancial assets, net	$ —	$ 108.0	$ —
Provision for (benefit from) income taxes	$ 20.9	$ 8.0	$ (11.3)
Net income (loss)	$ 167.6	$ 141.6	$ (64.1)

See "Results of Operations" below for discussion of our results for the periods presented.

Uncertainty Relating to Macroeconomic Environment

Conditions in the current macroeconomic environment, such as inflation, changes in interest and foreign currency exchange rates, natural disasters, and supply chain disruptions, could impact our global revenue sources and our overall business operations. The extent and duration of such effects remain uncertain and difficult to predict. We are actively monitoring and managing our response and assessing actual and potential impacts to our operating results and financial condition, as well as developments in our business, which could further impact the developments, trends and expectations described below. See the risk factor, "Our business is affected by macroeconomic conditions." described in "Risk Factors" in Part I, Item 1A of this Annual Report on Form 10-K.

Business Developments

We continued to grow our commercial business and advance our product candidate pipeline during 2023. We believe that the combination of our internal research programs and partnerships will allow us to continue to develop and commercialize innovative therapies for people with serious and life-threatening rare diseases and medical conditions.

In 2023, we achieved $2.4 billion in total revenues, including a significant contribution from our ongoing expansion of VOXZOGO, and we continued making important advancements in our product development pipeline. Our key business developments since the beginning of 2023 include U.S. Food and Drug Administration (FDA) approval of VOXZOGO for children with achondroplasia of all ages with open growth plates in the U.S., European Commission approval to expand the indication for VOXZOGO to treat children with achondroplasia aged four months and older with open growth plates in the European Union (EU), and FDA approval of ROCTAVIAN in the U.S. We also continued progress in our earlier stage clinical programs. Please see the disclosures in Part I Item I in this Annual Report on Form 10-K for further discussion of these recent developments.

(In millions of U.S. Dollars, except as otherwise disclosed)

Results of Operations

Net Product Revenues

Net Product Revenues consisted of the following:

	Twelve Months Ended December 31,				
	2023	2022	2021	2023 vs. 2022	2022 vs. 2021
Enzyme products:					
VIMIZIM	$ 701.0	$ 663.8	$ 623.1	$ 37.2	$ 40.7
NAGLAZYME	420.3	443.8	380.4	(23.5)	63.4
PALYNZIQ	303.9	255.0	237.5	48.9	17.5
BRINEURA	161.9	154.3	128.0	7.6	26.3
ALDURAZYME	131.2	128.4	122.8	2.8	5.6
Total enzyme product revenues	$ 1,718.3	$ 1,645.3	$ 1,491.8	$ 73.0	$ 153.5
Other products:					
VOXZOGO	469.9	169.1	5.9	300.8	163.2
KUVAN	180.8	227.6	285.8	(46.8)	(58.2)
ROCTAVIAN	3.5	—	—	3.5	—
Total net product revenues	$ 2,372.5	$ 2,042.0	$ 1,783.5	$ 330.5	$ 258.5

The increase in Net Product Revenues in 2023 as compared to 2022 was primarily attributed to the following:

- VOXZOGO: higher sales volume due to new patients initiating therapy across all regions;
- PALYNZIQ: higher sales volume from new patients initiating therapy, particularly in the U.S.; and
- VIMIZIM: higher sales volume primarily due to new patients initiating therapy, particularly in the U.S. and Europe, timing of orders in countries that place large government orders, particularly in the Middle East and Latin America; partially offset by
- KUVAN: lower sales primarily attributed to increasing generic competition as a result of the loss of exclusivity in the U.S. that occurred in October 2020 and
- NAGLAZYME: lower sales volume primarily due to timing of orders in countries that place large government orders, particularly in the Middle East.

In certain countries, governments place large periodic orders for our products. We expect that the timing of these large government orders will continue to be inconsistent, which has created and may continue to create significant period to period variation in our revenues.

Strong demand for VOXZOGO in certain markets has outpaced our projections in recent quarters, and we expect to face challenges meeting our current estimates of VOXZOGO demand through the first half of 2024. These demand challenges will result in modest reduction of our revenue growth for VOXZOGO during the supply-constrained period. The projected temporary supply constraint could result in postponement of planned entry into additional markets or delayed clinical development activities until VOXZOGO inventory levels increase. When the expected increases in supply become available during 2024, while overall inventory and ability to supply the market will increase, if actual demand continues to exceed our estimates, the supply constraint could be prolonged. We are working to increase fill-finish capacity to meet this increased demand while also implementing actions to manage growth and minimize patient impact. For example, in 2023 we secured increased supply commitments beginning in mid-2024. We do not expect a material impact on our revenues if we successfully execute our manufacturing plans. See "Risk Factors" in Part I, Item 1A of this Annual Report for additional information on risk factors that could impact our business and operations.

See also the risk factor "The sale of generic versions of KUVAN by generic manufacturers has adversely affected and will continue to adversely affect our revenues and may cause a decline in KUVAN revenues faster than expected" in "Risk Factors" included in Part I, Item 1A of this Annual Report for additional information on risks we face.

(In millions of U.S. Dollars, except as otherwise disclosed)

We face exposure to movements in foreign currency exchange rates, which we expect to continue in future periods. We use foreign currency exchange forward contracts to hedge a percentage of our foreign currency exposure, primarily the Euro. The following table shows our Net Product Revenues denominated in USD and foreign currencies:

	Twelve Months Ended December 31,				
	2023	2022	2021	2023 vs. 2022	2022 vs. 2021
Sales denominated in USD	$ 1,137.8	$ 1,008.8	$ 961.1	$ 129.0	$ 47.7
Sales denominated in foreign currencies	1,234.7	1,033.2	822.4	201.5	210.8
Total net product revenues	$ 2,372.5	$ 2,042.0	$ 1,783.5	$ 330.5	$ 258.5

	Twelve Months Ended December 31,				
	2023	2022	2021	2023 vs. 2022	2022 vs. 2021
Favorable (unfavorable) impact of foreign currency exchange rates on product sales denominated in currencies other than USD	$ (100.0)	$ (59.0)	$ 2.3	$ (41.0)	$ (61.3)

The unfavorable impact of foreign currency exchange rates on USD reported results in 2023 was primarily driven by the Argentine Peso, Euro, Japanese Yen and Russian Ruble.

See "Quantitative and Qualitative Disclosures about Market Risk" in Part II, Item 7A of this Annual Report on Form 10-K and the risk factor "Our international operations pose currency risks, which may adversely affect our operating and net income" in "Risk Factors" included in Part I, Item 1A of this Annual Report for information on currency exchange rate risk related to our Net Product Revenues.

Royalty and Other Revenues

Royalty and Other Revenues include royalties earned on net sales of products sold by third parties, up-front licensing fees, milestones achieved by licensees or sublicensees and rental income associated with the tenants in our facilities.

	Twelve Months Ended December 31,				
	2023	2022	2021	2023 vs. 2022	2022 vs. 2021
Royalty and other revenues	$ 46.7	$ 54.0	$ 62.8	$ (7.3)	$ (8.8)

The decrease in Royalty and Other Revenues in 2023 as compared to 2022 was primarily due to lower royalty revenues earned from third parties.

We expect to continue to earn royalties from third parties in the future.

Cost of Sales and Gross Margin

Cost of Sales includes raw materials, personnel, facility and other costs associated with manufacturing our commercial products. These costs include production materials, production costs at our manufacturing facilities, third-party manufacturing

(In millions of U.S. Dollars, except as otherwise disclosed)

costs, amortization of technology transfer intangible assets and internal and external final formulation and packaging costs. Cost of Sales also includes royalties payable to third parties based on sales of our products and charges for inventory valuation reserves.

The following table summarizes our Cost of Sales and gross margin:

	Twelve Months Ended December 31,				
	2023	2022	2021	2023 vs. 2022	2022 vs. 2021
Total revenues	$ 2,419.2	$ 2,096.0	$ 1,846.3	$ 323.2	$ 249.7
Cost of sales	$ 514.9	$ 483.7	$ 470.5	$ 31.2	$ 13.2
Gross margin	78.7 %	76.9 %	74.5 %	1.8 %	2.4 %

Cost of Sales increased for 2023 compared to 2022 primarily due to higher sales volumes as noted above. Gross margin for 2023 increased compared to 2022 primarily due to higher sales volume of products with higher margins, predominately related to VOXZOGO, and lower per unit manufacturing costs for our enzyme products.

We expect gross margin to increase modestly in future periods as the product mix is expected to shift to reflect an increase of sales volumes for higher margin commercial products.

Research and Development

We group all of our R&D activities and related expense into three categories: (i) research and early pipeline, (ii) later-stage clinical programs and (iii) marketed products as follows:

Category	Description
Research and early pipeline	R&D expense incurred in activities substantially in support of early research through the completion of phase 2 clinical trials, including drug discovery, toxicology, pharmacokinetics and drug metabolism and process development.
Later-stage clinical programs	R&D expense incurred in or related to phase 3 clinical programs intended to result in registration of a new product or a new indication for an existing product primarily in the U.S. or the EU.
Marketed products	R&D expense incurred in support of our marketed products that are authorized to be sold primarily in the U.S. or the EU. Includes clinical trials designed to gather information on product safety (certain of which may be required by regulatory authorities) and their product characteristics after regulatory approval has been obtained, as well as the costs of obtaining regulatory approval of a product in a new market after approval in either the U.S. or EU has been obtained.

We manage our R&D expense by identifying the R&D activities we anticipate will be performed during a given period and then prioritizing efforts based on scientific data, probability of successful development, market potential, available human and capital resources and other similar considerations. We continually review our product pipeline and the development status of product candidates and, as necessary, reallocate resources among the research and development portfolio that we believe will best support the future growth of our business.

We continuously evaluate the recoverability of costs associated with pre-launch or pre-qualification manufacturing activities, if any, and capitalize the costs incurred related to those activities if we determine that recoverability is highly likely and therefore future revenues are expected. If the related product candidate's marketing application is rejected by the applicable regulators and the likelihood of future revenues for a product candidate become uncertain, the related manufacturing costs are expensed as R&D expenses.

(In millions of U.S. Dollars, except as otherwise disclosed)

R&D expense consisted of the following:

	Twelve Months Ended December 31,				
	2023	2022	2021	2023 vs. 2022	2022 vs. 2021
Research and early pipeline	$ 393.1	$ 313.9	$ 275.9	$ 79.2	$ 38.0
Later-stage clinical programs	62.6	119.0	244.4	(56.4)	(125.4)
Marketed Products	291.1	216.7	108.5	74.4	108.2
Total R&D expense	$ 746.8	$ 649.6	$ 628.8	$ 97.2	$ 20.8

R&D expense increased for 2023 compared to 2022 primarily due to higher spend in research and early pipeline attributable to increased pre-clinical activities, including studies for planned clinical trial application submissions in the U.S. and EU. Higher spend on R&D activities related to our marketed products was partially offset by the decrease in later-stage clinical program spend due to the marketing approval of ROCTAVIAN in mid-2023.

We expect R&D expense to increase in future periods compared to 2023, primarily due to higher spend on early pipeline and later-stage clinical programs.

Selling, General and Administrative

Sales and marketing (S&M) expense primarily consists of employee-related expenses for our sales group, brand marketing, patient support groups and pre-commercialization expenses related to our product candidates. General and administrative (G&A) expense primarily consists of corporate support and other administrative expenses, including employee-related expenses.

SG&A expenses consisted of the following:

	Twelve Months Ended December 31,				
	2023	2022	2021	2023 vs. 2022	2022 vs. 2021
S&M expense	$ 488.4	$ 450.3	$ 405.1	$ 38.1	$ 45.2
G&A expense	448.9	403.7	354.3	45.2	49.4
Total SG&A expense	$ 937.3	$ 854.0	$ 759.4	$ 83.3	$ 94.6

S&M expenses by product were as follows:

	Twelve Months Ended December 31,				
	2023	2022	2021	2023 vs. 2022	2022 vs. 2021
Enzyme Products	$ 225.3	$ 225.7	$ 222.1	$ (0.4)	$ 3.6
VOXZOGO	108.9	102.3	75.1	6.6	27.2
ROCTAVIAN	104.5	73.6	54.0	30.9	19.6
Other	49.7	48.7	53.9	1.0	(5.2)
Total S&M expense	$ 488.4	$ 450.3	$ 405.1	$ 38.1	$ 45.2

The increase in S&M expense for 2023 compared to 2022 was primarily a result of increased activities in support of the European and U.S. commercial launch of ROCTAVIAN.

The increase in G&A expense was primarily due to increased costs related to costs associated with our enterprise resource planning (ERP) system and other strategic initiatives, an impairment charge recorded in 2023 and unfavorable fluctuations of unhedged currencies. Partially offsetting the increases was a decrease in severance and employee termination benefits related to the 2022 reorganization plan that did not recur in 2023. In 2023, we decided to cease development of the first generation VOXZOGO pen device and impaired the related capitalized tooling and fixed assets that had not been placed in service. See Note 4 to our accompanying Consolidated Financial Statements for additional details.

(In millions of U.S. Dollars, except as otherwise disclosed)

We expect SG&A expense to increase in future periods as a result of the continued market expansion of our commercial products and support of our global business as it grows.

Intangible Asset Amortization and Contingent Consideration and Gain on Sale of Nonfinancial Assets

Changes during the periods presented for Intangible Asset Amortization and Contingent Consideration and Gain on Sale of Nonfinancial Assets were as follows:

	Twelve Months Ended December 31, 2023				
	2023	2022	2021	2023 vs. 2022	2022 vs. 2021
Amortization of intangible assets	$ 62.2	$ 62.8	$ 61.9	$ (0.6)	$ 0.9
Changes in the fair value of contingent consideration	—	4.4	8.0	(4.4)	(3.6)
Total intangible asset amortization and contingent consideration	$ 62.2	$ 67.2	$ 69.9	$ (5.0)	$ (2.7)
Gain on sale of nonfinancial assets	$ —	$ 108.0	$ —	$ (108.0)	$ 108.0

Amortization of intangible assets: the expense in 2023 as compared to 2022 was relatively flat.

Changes in the fair value of contingent consideration: the 2023 decrease in expense as compared to 2022 was attributable to the attainment of final commercial milestones in 2022.

Gain on Sale of Nonfinancial Assets: the decrease in 2023 as compared to 2022 was due to the sale in 2022 of a Priority Review Voucher (PRV) with no similar transaction in 2023.

Interest Income

We invest our cash equivalents and investments in U.S. government securities and other high credit quality debt securities in order to limit default and market risk.

	Twelve Months Ended December 31,				
	2023	2022	2021	2023 vs. 2022	2022 vs. 2021
Interest income	$ 58.3	$ 18.0	$ 10.5	$ 40.3	$ 7.5

The increase in Interest Income during 2023 compared to 2022 was primarily due to higher money market and available-for-sale debt securities balances and higher yields on our investment portfolio. We do not expect Interest Income to fluctuate significantly over the next 12 months due to anticipated interest rates and yields on our cash equivalents and investments.

Interest Expense

We incur interest expense primarily on our convertible debt. Interest Expense for the periods presented was as follows:

	Twelve Months Ended December 31,				
	2023	2022	2021	2023 vs. 2022	2022 vs. 2021
Interest expense	$ 17.3	$ 16.0	$ 15.3	$ 1.3	$ 0.7

Interest Expense in 2023 as compared to 2022 was relatively flat. We expect Interest Expense to decrease over the next 12 months due to the settlement of our convertible debt that matures in August 2024. See Note 10 to our accompanying Consolidated Financial Statements for additional information regarding our convertible debt.

(In millions of U.S. Dollars, except as otherwise disclosed)

Other Income (Expense), Net

Other Income (Expense), Net for the periods presented was as follows:

	Twelve Months Ended December 31,				
	2023	2022	2021	2023 vs. 2022	2022 vs. 2021
Other income (expense), net	$ (10.5)	$ (2.1)	$ 11.8	$ (8.4)	$ (13.9)

The change in Other Income (Expense), Net, in 2023 compared to 2022 was primarily due to impairment losses on an equity investment and a convertible note, partially offset by the gain on the fair value of assets held in our nonqualified deferred compensation plan and gains related to refundable tax credits recorded in 2023.

Provision for (Benefit from) Income Taxes

Provision for (Benefit from) Income Taxes for the periods presented was as follows:

	Twelve Months Ended December 31,				
	2023	2022	2021	2023 vs. 2022	2022 vs. 2021
Provision for (benefit from) income taxes	$ 20.9	$ 8.0	$ (11.3)	$ 12.9	$ 19.3

Provision for income taxes in 2023 increased compared to 2022, primarily due to taxes on higher earnings and foreign-source income taxed in the U.S. partially offset by an additional benefit from an increase in R&D credits and the release of a valuation allowance related to future royalty earnings. Our Provision for income taxes in 2023 and 2022 consisted of state, federal and foreign current tax expense which was offset by tax benefits related to stock option exercises, foreign tax credits, and deferred tax benefits from federal orphan drug credits and federal R&D credits. See Note 15 to our accompanying Consolidated Financial Statements for additional information.

In the third quarter of 2023, we determined that it is more likely than not that the deferred tax assets related to a future royalty stream will be realized. In making this determination, we analyzed both the consistent historical royalty earnings and the forecast of future royalty earnings and reached the conclusion that it was appropriate to release the valuation allowance reserve.

Certain countries in which we have operations, including Ireland, have adopted Pillar Two rules, recently released from the Organisation for Economic Co-operation and Development (OECD), including a minimum tax rate of 15%. It is uncertain whether the United States will enact legislation to adopt the Pillar Two framework. We do not expect the adoption of the Pillar Two framework to have a material impact on our effective tax rate and we plan to continue evaluating additional guidance released by the OECD, along with the pending legislative adoption by additional individual countries.

Results of Operations 2022 Compared to 2021

For a discussion of our results of operations pertaining to 2022 as compared to 2021 see Item 7, "Management's Discussion and Analysis of Financial Condition and Results of Operations" in our Annual Report on Form 10-K for the year ended December 31, 2022 (filed with the Securities and Exchange Commission (SEC) on February 27, 2023).

(In millions of U.S. Dollars, except as otherwise disclosed)

Financial Condition, Liquidity and Capital Resources

Our cash, cash equivalents, and investments were as follows:

	December 31, 2023	December 31, 2022	Change
Cash and cash equivalents	$ 755.1	$ 724.5	$ 30.6
Short-term investments	318.7	567.0	(248.3)
Long-term investments	611.1	333.9	277.2
Total cash, cash equivalents and investments	$ 1,684.9	$ 1,625.4	$ 59.5

We believe our cash generated from sales of our commercial products, in addition to our cash, cash equivalents and investments, will be sufficient to satisfy our liquidity requirements for at least the next 12 months. We believe we will meet longer-term expected future cash requirements and obligations through a combination of cash flows from operating activities and available cash and investments balances. We will need to raise additional funds from equity or debt securities, loans or collaborative agreements if we are unable to satisfy our liquidity requirements. For example, we may require additional financing to fund the repayment of our convertible debt, future milestone payments and our future operations, including the commercialization of our products and product candidates currently under development, preclinical studies and clinical trials, and potential licenses and acquisitions. The timing and mix of our funding alternatives could change depending on many factors, including how much we elect to spend on our development programs, potential licenses and acquisitions of complementary technologies, products and companies or if we settle our convertible debt in cash.

We are mindful that conditions in the current macroeconomic environment, such as inflation, changes in interest and foreign currency exchange rates, natural disasters, and supply chain disruptions, could affect our ability to achieve our goals. In addition, we sell our products in certain countries that face economic volatility and weakness. Although we have historically collected receivables from customers in such countries, sustained weakness or further deterioration of the local economies and currencies may cause customers in those countries to be unable to pay for our products. We will continue to monitor these conditions and will attempt to adjust our business processes, as appropriate, to mitigate macroeconomic risks to our business.

Our cash flows for each of the years ended December 31, 2023 and 2022 were as follows:

	2023	2022	2023 vs. 2022
Net cash provided by operating activities	$ 159.3	$ 175.9	$ (16.6)
Net cash used in investing activities	$ (111.2)	$ (20.0)	$ (91.2)
Net cash used in financing activities	$ (18.7)	$ (18.7)	$ —

The decrease in net cash provided by operating activities in 2023 compared to 2022 was primarily attributed to the timing of cash receipts from our customers and increased payments for inventory purchases, income taxes and increased payments related to implementation of our ERP system, partially offset by timing of cash payments to other vendors.

The increase in net cash used in investing activities in 2023 compared to 2022 was primarily attributable to the absence of $110.0 million gross proceeds from the sale of PRV in 2022, partially offset by a decrease in purchases of fixed assets.

Net cash used by financing activities in 2023 compared to 2022 was flat but was driven by higher taxes paid for net settlement of shares under our equity incentive plans offset by a decrease in milestone payments to a third party that had been contingent upon PKU sales milestones achieved in 2022.

Financing and Credit Facilities

Our $1.1 billion (undiscounted) of total convertible debt as of December 31, 2023 will impact our liquidity due to the semi-annual cash interest payments as well as the repayment of the principal amount, if not converted. As of December 31, 2023, our indebtedness consisted of our 1.250% senior subordinated convertible notes due in 2027 (the 2027 Notes) and our 0.599% senior subordinated convertible notes due in 2024 (the 2024 Notes and together with the 2027 Notes, the Notes), which, if not converted, will be required to be repaid in cash at maturity in May 2027 and August 2024, respectively. We have reclassified all of the outstanding principal of the 2024 Notes as a current liability as there are less than twelve months remaining until maturity.

In October 2018, we entered into an unsecured revolving credit facility of up to $200.0 million that included a letter of credit subfacility and a swingline loan subfacility. The credit facility was intended to finance ongoing working capital needs and for other general corporate purposes. In May 2021, the credit facility was amended to extend the original maturity date from October 19, 2021 to May 28, 2024. The credit facility was terminated on August 4, 2023 and, therefore there were no amounts outstanding under the terminated credit facility as of December 31, 2023.

See Note 10 to our accompanying Consolidated Financial Statements for additional discussion on our convertible debt and credit facility.

Material Cash Requirements

Purchase and Lease Obligations

As of December 31, 2023, we had purchase obligations of approximately $354.1 million, of which $325.9 million is expected to be paid in 2024. Our purchase obligations are primarily related to firm purchase commitments entered into in the normal course of business to procure active pharmaceutical ingredients, certain inventory-related items, certain third-party R&D services, production services and facility construction services. The amount also includes hosting fees and other ERP system implementation costs for which we are committed.

As of December 31, 2023, we had lease payment obligations of $58.7 million, of which $11.4 million is payable in 2024. See Note 9 to our accompanying Consolidated Financial Statements for details on our lease liabilities.

Contingent Obligations

As of December 31, 2023, we were subject to contingent payments considered reasonably possible of $763.3 million, of this amount we may pay up to $30.1 million in 2024 if certain contingencies are met. See Note 18 to our accompanying Consolidated Financial Statements for additional discussion on our contingent obligations.

Unrecognized Tax Benefits

As of December 31, 2023, our liability for unrecognized tax benefits was $277.5 million. Due to their nature, we cannot reasonably estimate the timing of future payments. See Note 15 to our accompanying Consolidated Financial Statements for a full discussion on our income taxes.

Critical Accounting Estimates

In preparing our Consolidated Financial Statements in accordance with U.S. GAAP and pursuant to the rules and regulations promulgated by the SEC, we make assumptions, judgments and estimates that can have a significant impact on our net income/loss and affect the reported amounts of certain assets, liabilities, revenue and expenses, and related disclosures. On an ongoing basis, we evaluate our estimates and discuss our critical accounting policies and estimates with the Audit Committee of our Board of Directors. We base our estimates on historical experience and various other assumptions that we believe to be reasonable under the circumstances. Actual results could differ materially from these estimates under different assumptions or conditions. Historically, our assumptions, judgments and estimates relative to our critical accounting policies have not differed materially from actual results.

Our significant accounting policies are described in Note 1 to our accompanying Consolidated Financial Statements included in this Annual Report on Form 10-K. We believe the critical accounting estimates below reflect the most critical judgments and estimates used in the preparation of our Consolidated Financial Statements.

Revenue Recognition and Related Allowances

Net Product Revenues – We recognize revenue when the customer obtains control of promised goods or services, in an amount that reflects the consideration which we expect to receive in exchange for those goods or services. For ALDURAZYME revenues, we receive a payment ranging from 39.5% to 50% on worldwide net ALDURAZYME sales by Sanofi depending on sales volume, which is included in Net Product Revenues in our Consolidated Statements of Operations. We recognize our best estimate of the entire revenue that we expect to receive when the product is released and control is transferred to Sanofi. We record ALDURAZYME net product revenues based on the estimated variable consideration payable when the product is sold through by Sanofi. Differences between the estimated variable consideration to be received and actual payments received are not expected to be material. If actual results vary from our estimates, we will make adjustments, which would affect Net Product Revenues and earnings in the period such variances become known.

(In millions of U.S. Dollars, except as otherwise disclosed)

Gross-to-Net Sales Adjustments – We record product sales net of estimated mandatory and supplemental discounts to government payers, discounts to private payers and other related charges. Rebates, cash discounts and distributor fees represent the majority of our gross-to-net deductions and are recorded in the same period the related sales occur. Rebates may include amounts paid to Medicaid or other U.S. or foreign government programs, certain managed care providers, or other payers. Rebates, branded co-pay assistance programs, cash discounts and distributor fees are estimates based on contractual arrangements or statutory obligations, which may vary by product and payer. Estimation requires evaluation of our actual historical experience, customer and payer mix, current contractual and statutory obligations, patient outcomes, specific known market events and trends and industry data. We evaluate our customer and payer mix to estimate which sales will be subject to these revenue dilutive items and consider changes to government program guidelines or contractual obligations that would impact the actual rebates and/or our estimates of which sales qualify for such rebates. Any necessary adjustments to our reserves are made each quarter to reflect current information. We believe the methodologies that we use to estimate allowances are reasonable and appropriate given the facts and circumstances. However, actual results may differ significantly from our estimates.

The following table summarizes the consolidated activities and ending balances of all our gross-to-net sales adjustments:

	Balance at Beginning of Year	Provision for Current Period Sales	Payments	Balance at End of Year
Year ended December 31, 2023	$ 115.0	$ 370.7	$ (333.6)	$ 152.1
Year ended December 31, 2022	$ 85.6	$ 282.5	$ (253.1)	$ 115.0
Year ended December 31, 2021	$ 104.4	$ 252.9	$ (271.7)	$ 85.6

Income Taxes

We calculate and provide for income taxes in each of the tax jurisdictions in which we operate. Our Consolidated Balance Sheets reflect net deferred tax assets and liabilities, which are measured using enacted tax rates. The net deferred tax assets primarily represent the tax benefit of tax credits and timing differences between book and tax recognition of certain revenue and expense items, net of a valuation allowance. When it is more likely than not that all or some portion of deferred tax assets may not be realized, we establish a valuation allowance for the amount that may not be realized. We utilize financial projections to support our net deferred tax assets, which contain significant assumptions and estimates of future operations. If such assumptions were to differ significantly, it may have a material impact on our ability to realize our net deferred tax assets. Changes in our valuation allowance will result in a change to tax expense.

We establish liabilities or reduce assets for certain tax positions when we believe those certain tax positions are not more likely than not to be sustained if challenged. Each quarter, we evaluate these uncertain tax positions and adjust the related tax assets and liabilities in light of changing facts and circumstances.

We are subject to income taxes in the U.S. and various foreign jurisdictions, including Ireland. Due to economic and political conditions, various countries are actively considering changes to existing tax laws. We cannot predict the form or timing of potential legislative changes that could have a material adverse impact on our results of operations. Management is not aware of any potential changes that would have a material effect on our Consolidated Financial Statements. See Note 15 to our accompanying Consolidated Financial Statements for additional discussion.

Impairments of Long-Lived Assets

We assess changes in economic, regulatory and legal conditions and make assumptions regarding estimated future cash flows in evaluating the value of our property, plant and equipment, goodwill and other long-lived assets. We periodically evaluate whether current facts or circumstances indicate that the carrying values of our long-lived assets may not be recoverable. Should there be an indication of impairment, we test for recoverability by comparing the estimated undiscounted future cash flows expected to result from the use of the asset or asset group and its eventual disposition to the carrying amount of the asset or asset group. Any excess of the carrying value of the asset or asset group over its estimated fair value is recognized as an impairment loss.

Recent Accounting Pronouncements

See Note 1 to our accompanying Consolidated Financial Statements for a full description of recent accounting pronouncements and our expectation of their impact on our results of operations and financial condition.

Item 7A. Quantitative and Qualitative Disclosure About Market Risk

We are exposed to market risks that may result from changes in foreign currency exchange rates, interest rates and credit risks. To reduce certain of these risks, we enter into foreign currency derivative hedging transactions, follow investment guidelines and monitor outstanding trade receivables as part of our risk management program.

Foreign Currency Exchange Rate Risk

Our operations include manufacturing activities in the U.S. and Ireland and sales activities in the U.S. as well as in regions outside the U.S, including Europe, Latin America, the Middle East and Asia Pacific. As a result, our financial results may be significantly affected by factors such as changes in foreign currency exchange rates or weak economic conditions in the foreign markets in which we sell our products. Our operating results are exposed to changes in foreign currency exchange rates between the U.S. Dollar (USD) and various foreign currencies, primarily the Euro. When the USD strengthens against these currencies, the relative value of the sales and operating expenses made in the respective foreign currency decreases. Conversely, when the USD weakens against these currencies, the relative value of such sales and operating expenses increases. Overall, we are a net receiver of foreign currencies and, therefore, benefit from a weaker USD and are adversely affected by a stronger USD relative to those foreign currencies in which we transact significant business.

During 2023, approximately 52% of our net product sales were denominated in foreign currencies and 24% of our operating expenses, excluding Cost of Sales, were denominated in foreign currencies. To partially mitigate the impact of changes in currency exchange rates on net cash flows from our foreign currency denominated sales and operating expenses, we enter into foreign currency exchange forward contracts (forward contracts). We also hedge certain monetary assets and liabilities, primarily those denominated in Euros, using forward contracts, which reduces but does not eliminate our exposure to currency fluctuations between the date the transaction is recorded and the date the cash is collected or paid. Generally, the market risks of these contracts are offset by the corresponding gains and losses on the transactions being hedged.

We do not use derivative financial instruments for speculative trading purposes, nor do we hedge foreign currency exchange rate exposure in a manner that entirely offsets the effects of changes in foreign currency exchange rates. The counterparties to these forward contracts are creditworthy multinational commercial banks, which minimizes the risk of counterparty nonperformance. We regularly review our hedging program and may, as part of this review, make changes to the program.

As of December 31, 2023, we had open forward contracts with net notional amounts of $1.3 billion. A hypothetical 10% adverse movement in foreign currency exchange rates compared with the USD relative to exchange rates as of December 31, 2023 would have resulted in a reduction in the value received over the remaining life of these contracts by approximately $134.4 million on this date and, if realized, would negatively affect earnings during the remaining life of the contracts. The estimated fair value change was determined by measuring the impact of the hypothetical exchange rate movement on outstanding forward contracts. This analysis does not consider the impact of the hypothetical changes in foreign currency rates would have on the forecasted transactions that these foreign currency sensitive instruments were designated to offset. Our use of this methodology to quantify the market risk of such instruments is subject to assumptions and actual impact could be significantly different.

Based on our overall foreign currency denominated exposures as of December 31, 2023, we believe that a near-term 10% fluctuation of the USD exchange rate could result in a potential change in the fair value of our net foreign currency denominated assets and liabilities, excluding our investments and open forward contracts, by approximately $27.4 million. We expect to continue to enter into transactions based in foreign currencies that could be impacted by changes in exchange rates.

Interest Rate Market Risk

Our exposure to market risk for changes in interest rates relates primarily to our investment portfolio, which includes our cash equivalents and marketable debt securities. By policy, we place our investments with highly rated credit issuers and limit the amount of credit exposure to any one issuer. As stated in our investment policy, we seek to improve the safety and likelihood of preservation of our invested funds by limiting default risk and market risk.

We mitigate default risk by investing in high credit quality securities and by positioning our portfolio to respond appropriately to a significant reduction in a credit rating of any investment issuer or guarantor. The portfolio includes only marketable securities with active secondary or resale markets to ensure portfolio liquidity.

We have outstanding $495.0 million (undiscounted) of the 2024 Notes and $600.0 million (undiscounted) of the 2027 Notes. The interest rates on these notes are fixed and therefore they do not expose us to risk related to rising interest rates. As of December 31, 2023, the fair value of our convertible debt was $1.1 billion.

As of December 31, 2023, our investment portfolio did not include any investments with significant exposure to countries that face economic volatility and weakness. Although not predictive in nature, based on our investment portfolio and interest rates

for the period ending December 31, 2023, we believe a 100 basis point increase in interest rates could result in a potential loss in fair value of our investment portfolio of approximately $10.8 million. Changes in interest rates may affect the fair value of our investment portfolio. However, we will not recognize such gains or losses in our Consolidated Statements of Operations unless the investments are sold or we determine that the declines in the investment's fair values below the cost basis are a result of a credit loss, which, if any, are reported in Other Income (Expense), Net in the current period through an allowance for credit losses.

The table below summarizes the expected maturities and average interest rates of our interest-generating investments as of December 31, 2023 (in millions of U.S. Dollars):

	Expected Maturity					
	2024	2025	2026	2027	2028	Total
Available-for-sale debt securities	$ 344.7	$ 291.4	$ 269.8	$ 45.5	$ 4.4	$ 955.8
Average interest rate	5.0 %	5.0 %	4.7 %	4.8 %	5.4 %	4.9 %

Counterparty Credit Risks

Our financial instruments, including derivatives, are subject to counterparty credit risk that we consider as part of the overall fair value measurement. Our financial risk management policy limits derivative transactions by requiring transactions to be with institutions with minimum credit ratings of A- or equivalent by Standards & Poor's, Moody's or Fitch. In addition, we have an investment policy that limits investments to certain types of debt and money market instruments issued by institutions primarily with investment grade credit ratings and places restriction on maturities and concentrations by asset class and issuer.

Item 8. Financial Statements and Supplementary Data

The information required to be filed in this item appears under "Exhibits, Financial Statement Schedules" in Part IV, Item 15 of this Annual Report on Form 10-K.

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

None.

Item 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

An evaluation was carried out, under the supervision of and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, of the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of the end of the period covered by this report. Based on the evaluation, our Chief Executive Officer and our Chief Financial Officer have concluded that our disclosure controls and procedures were effective as of December 31, 2023.

Management's Annual Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining an adequate internal control structure and procedures for financial reporting. Under the supervision of and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, our management has assessed the effectiveness of our internal control over financial reporting as defined in Rule 13a-15(f) under the Exchange Act as of December 31, 2023. Our management's assessment was based on criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO), Internal Control-Integrated Framework (2013).

Based on the COSO criteria, our management has concluded that our internal control over financial reporting as of December 31, 2023 was effective at the reasonable assurance level.

Our independent registered public accounting firm, KPMG LLP, has audited the financial statements included in this Annual Report on Form 10-K and has issued a report on the effectiveness of our internal control over financial reporting. The report of KPMG LLP is incorporated by reference to Item 8 of this Annual Report on Form 10-K.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act, during our most recently completed quarter that have materially affected or are reasonably likely to materially affect our internal control over financial reporting. We continue to utilize the Committee of Sponsoring Organizations of the Treadway Commission (COSO) 2013 Framework on internal control. We rely extensively on information systems and technology to manage our business, including integrated supply chain operations, and global consolidated financial results. We are currently preparing to implement a new global enterprise resource planning (ERP) system, which will replace existing operating and financial systems. The ERP system is designed to accurately maintain our financial records, support integrated supply chain and other operational functionality, and provide timely information to our management team related to the operation of the business. We are currently implementing in phases through 2025, with post-implementation activities following thereafter. As the implementation and post-implementation activities take place, we will have changes to certain of our processes and procedures, and we will evaluate quarterly whether the changes materially affect our internal control over financial reporting.

Scope of the Effectiveness of Controls

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 GAAP. Our internal control over financial reporting includes those policies and procedures that:

- pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect our transactions and dispositions of our assets;
- provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with GAAP, and that our receipts and expenditures are being made only in accordance with authorizations of our management and our board of directors; and
- provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of our assets that could have a material effect on our financial statements.

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.

Item 9B. Other Information

Rule 10b5-1 Trading Plans

During the three months ended December 31, 2023, our directors and officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted or terminated the contracts, instructions or written plans for the purchase or sale of BioMarin securities set forth in the table below.

				Type of Trading Arrangement			
Name	**Position**	**Action**	**Adoption/Termination Date**	**Rule 10b5-1[(1)]**	**Non-Rule 10b5-1[(2)]**	**Total Shares of Common Stock to be Sold[(3)]**	**Expiration Date**
Erin Burkhart	Group Vice President and Chief Accounting Officer	Termination	November 29, 2023	X		up to 2,782	March 8, 2024
Erin Burkhart	Group Vice President and Chief Accounting Officer	Adoption	November 29, 2023	X		up to 5,709	November 29, 2024

(1) Contract, instruction or written plan intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) under the Exchange Act.

(2) "Non-Rule 10b5-1 trading arrangement" as defined in Item 408(c) of Regulation S-K under the Exchange Act.

(3) Represents the maximum number of shares that may be sold pursuant to the 10b5-1 arrangement. The number of shares sold will be dependent on the satisfaction of certain conditions as set forth in the written plan.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections

Not applicable.

Part III

Item 10. Directors, Executive Officers and Corporate Governance

The information required by this Item regarding our directors, executive officers and corporate governance is incorporated into this section by reference to the sections captioned “Election of Directors” and “Executive Officers” in the proxy statement for our 2024 annual meeting of stockholders.

Item 11. Executive Compensation

The information required by this Item regarding executive compensation is incorporated into this section by reference to the section captioned “Executive Compensation” in the proxy statement for our 2024 annual meeting of stockholders.

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

The information required by this Item regarding security ownership of our beneficial owners, management and related stockholder matters is incorporated into this section by reference to the section captioned “Security Ownership of Certain Beneficial Owners and Management” in the proxy statement for our 2024 annual meeting of stockholders.

The information required by this Item regarding the securities authorized for issuance under our equity compensation plans is incorporated into this section by reference to the section captioned “Equity Compensation Plan Information” in the proxy statement for our 2024 annual meeting of stockholders.

Item 13. Certain Relationships and Related Transactions, and Director Independence

The information required by this Item regarding certain relationships, related transactions and director independence is incorporated into this section by reference to the sections captioned “Other Board Governance Information — Transactions with Related Persons, Promoters and Certain Control Persons,” “Other Board Governance Information — Review, Approval and Ratification of Transactions with Related Parties” and “Director Independence” in the proxy statement for our 2024 annual meeting of stockholders.

Item 14. Principal Accountant Fees and Services

The information required by this Item regarding our principal accountant fees and services is incorporated into this section by reference to the section captioned “Independent Registered Public Accounting Firm” in the proxy statement for our 2024 annual meeting of stockholders.

Part IV

Item 15. Exhibits, Financial Statement Schedules

Exhibit Index

Exhibit Number	Description
2.1	Amended and Restated Termination and Transition Agreement, dated as of December 23, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on January 7, 2016 as Exhibit 2.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC.
2.2	Termination and Transition Agreement, dated as of October 1, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on January 7, 2016 as Exhibit 2.3 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC.
2.3	First Amendment, dated as of December 12, 2016, to the Amended and Restated Termination and Transition Agreement, dated as of December 23, 2015 and effective as of October 1, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on February 27, 2017 as Exhibit 2.6 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC.
3.1	Restated Certificate of Incorporation of BioMarin Pharmaceutical Inc., previously filed with the SEC on June 12, 2017 as Exhibit 3.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
3.2	Amended and Restated Bylaws of BioMarin Pharmaceutical Inc., previously filed with the SEC on December 21, 2022 as Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
4.1	Base Indenture, dated August 11, 2017, between the Company and Wilmington Trust, National Association, as Trustee, previously filed with the SEC on August 11, 2017 as Exhibit 4.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
4.2	First Supplemental Indenture, dated August 11, 2017, between the Company and Wilmington Trust, National Association, as Trustee (including the form of 0.599% Senior Subordinated Convertible Note due 2024), previously filed with the SEC on August 11, 2017 as Exhibit 4.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
4.3	Indenture, dated as of May 14, 2020, between BioMarin Pharmaceutical Inc. and U.S. Bank National Association, as trustee, including the Form of Global Note representing BioMarin Pharmaceutical, Inc.'s 1.25% Senior Subordinated Convertible Notes due 2027 as Exhibit A thereto, previously filed with the SEC on May 14, 2020 as Exhibit 4.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
4.4	Description of Capital Stock, previously filed with the SEC on February 27, 2020 as Exhibit 4.6 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference.
10.1†	Form of Indemnification Agreement for Directors and Officers, previously filed with the SEC on December 19, 2016 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
10.2†	BioMarin Pharmaceutical Inc. Amended and Restated 2006 Employee Stock Purchase Plan, as amended and restated April 12, 2019, previously filed with the SEC on August 2, 2019 as Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference.
10.3†	BioMarin Pharmaceutical Inc. Amended and Restated 2006 Share Incentive Plan, as adopted on May 2, 2006 and as amended and restated on April 16, 2015, previously filed with the SEC on June 15, 2015 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
10.4†	Form of Agreement Regarding Restricted Share Units for the BioMarin Pharmaceutical Inc. 2006 Share Incentive Plan, previously filed with the SEC on May 16, 2013 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
10.5†	Form of Amendment to Agreement Regarding Restricted Share Units for the BioMarin Pharmaceutical Inc. 2006 Share Incentive Plan, previously filed with the SEC on December 9, 2016 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.

10.6†	Amended and Restated BioMarin Pharmaceutical Inc. Nonqualified Deferred Compensation Plan, as adopted on December 1, 2005 and as amended and restated on January 1, 2009 and further amended and restated on December 19, 2013 and October 7, 2014, previously filed with the SEC on October 14, 2014 as Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
10.7†	Amended and Restated Employment Agreement with Jean-Jacques Bienaimé effective December 13, 2016 previously filed with the SEC on December 19, 2016 as Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
10.8	License Agreement dated July 30, 2004, between BioMarin Pharmaceutical Inc. and Daiichi Suntory Pharma Co., Ltd., as amended by Amendment No. 1 to License Agreement dated November 19, 2004, previously filed with the SEC on March 16, 2005 as Exhibit 10.25 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.
10.9	Operating Agreement with Genzyme Corporation, previously filed with the SEC on July 6, 1999 as Exhibit 10.30 to the Company's Amendment No. 2 to Registration Statement on Form S-1 (File No. 333-77701), which is incorporated herein by reference.
10.10	Manufacturing, Marketing and Sales Agreement dated as of January 1, 2008, by and among BioMarin Pharmaceutical Inc., Genzyme Corporation and BioMarin/Genzyme LLC previously filed with the SEC on February 28, 2008 as Exhibit 10.30 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.
10.11	Amended and Restated Collaboration Agreement dated as of January 1, 2008, by and among BioMarin Pharmaceutical Inc., Genzyme Corporation and BioMarin/Genzyme LLC previously filed with the SEC on February 28, 2008 as Exhibit 10.31 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.
10.12	Members Agreement dated as of January 1, 2008 by and among BioMarin Pharmaceutical Inc., Genzyme Corporation, BioMarin Genetics Inc., and BioMarin/Genzyme LLC previously filed with the SEC on February 28, 2008 as Exhibit 10.32 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.
10.13†	Form of Stock Options Agreement for the BioMarin Pharmaceutical Inc. 2006 Share Incentive Plan, (as Amended and Restated 2010), previously filed with the SEC on August 2, 2012 as Exhibit 10.11 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference.
10.14†	Form of Amended and Restated Employment Agreement for the Company's Executive Officers (other than the Company's Chief Executive Officer) previously filed with the SEC on June 15, 2015 as Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
10.15	Settlement and License Agreement among BioMarin Pharmaceutical Inc., Merck & Cie, Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd., dated September 14, 2015, previously filed with the SEC on November 2, 2015 as Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.
10.16	Settlement and License Agreement among BioMarin Pharmaceutical Inc., Merck & Cie and Par Pharmaceutical, Inc., dated as of April 12, 2017, previously filed with the SEC on November 13, 2017 as Exhibit 10.1 to the Company's Amendment No. 1 to Quarterly Report on Form 10-Q/A (File No. 000-26727), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.
10.17†	Form of Agreement Regarding Performance Stock Award in the Form of Restricted Stock Units for the BioMarin Pharmaceutical Inc. 2006 Share Incentive Plan, previously filed with the SEC on February 27, 2017 as Exhibit 10.50 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference.
10.18†	BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, as amended on April 3, 2023, previously filed with the SEC on August 2, 2023 as Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q (File No.000-26727), which is incorporated herein by reference.
10.19†	Form of Stock Options Agreement for the BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on June 12, 2017 as Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
10.20†	Form of Agreement Regarding Restricted Stock Units for the BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on June 12, 2017 as Exhibit 10.3 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.

10.21†	Form of Agreement Regarding Performance Stock Award in the Form of Restricted Stock Units for the BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on June 12, 2017 as Exhibit 10.4 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
10.22†	BioMarin Pharmaceutical Inc. Summary of Independent Director Compensation, previously filed with the SEC on October 28, 2022 as Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference.
10.23†	First Amendment to the Amended and Restated BioMarin Pharmaceutical Inc. Nonqualified Deferred Compensation Plan, as adopted June 4, 2019, previously filed with the SEC on August 2, 2019 as Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference.
10.24†	Second amendment to the Amended and Restated BioMarin Pharmaceutical Inc. Nonqualified Deferred Compensation Plan, as adopted on October 5, 2021, previously filed with the SEC on February 25, 2022 as Exhibit 10.32 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference.
10.25	Asset Purchase Agreement by and between Eli Lilly and Company, BioMarin Pharmaceutical Inc., and BioMarin International Ltd., dated February 8, 2022, previously filed with the SEC on April 29, 2022 as Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit have been omitted because they are not material and the type that the registrant treats as private or confidential.
10.26†	Third Amendment to the Amended and Restated BioMarin Pharmaceutical Inc. Nonqualified Deferred Compensation Plan, as adopted October 4, 2022, previously filed with the SEC on October 28, 2022 as Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference.
10.27†	Form of Agreement Regarding Non-Employee Director Restricted Stock Units for the BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on February 27, 2023 as Exhibit 10.35 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference.
10.28†	Form of Agreement Regarding Restricted Stock Units for the BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on February 27, 2023 as Exhibit 10.36 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference.
10.29†	Form of Stock Options Agreement for the BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on February 27, 2023 as Exhibit 10.37 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference.
10.30†	Separation Agreement and General Release by and between BioMarin Pharmaceutical Inc. and Jean-Jacques Bienaimé, dated October 30, 2023, previously filed with the SEC on November 3, 2023 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
10.31†	Consulting Agreement by and between BioMarin Pharmaceutical Inc. and Jean-Jacques Bienaimé, dated October 30, 2023, previously filed with the SEC on November 3, 2023 as Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
10.32†	Employment Agreement by and between BioMarin Pharmaceutical Inc. and Alexander Hardy, dated October 30, 2023, previously filed with the SEC on November 3, 2023 as Exhibit 10.3 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
10.33	Cooperation Agreement, dated as of December 20, 2023, by and among the Company, Elliott Investment Management L.P., Elliott Associates, L.P. and Elliott International, L.P., previously filed with the SEC on December 20, 2023 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
21.1*	Subsidiaries of BioMarin Pharmaceutical Inc.
23.1*	Consent of KPMG LLP, Independent Registered Public Accounting Firm for BioMarin Pharmaceutical Inc.
24.1*	Power of Attorney (Included in Signature Page to this Report)
31.1*	Certification of Chief Executive Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
31.2*	Certification of Chief Financial Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.

32.1*	Certification of Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. This Certification accompanies this report and shall not, except to the extent required by the Sarbanes-Oxley Act of 2002, be deemed filed for purposes of §18 of the Securities Exchange Act of 1934, as amended.
97.1*	Dodd-Frank Incentive Compensation Recoupment Policy, as adopted on October 4, 2023.
101.INS	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.
101.SCH	Inline XBRL Taxonomy Extension Schema Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase
101.LAB	Inline XBRL Taxonomy Extension Labels Linkbase Document
101.PRE	Inline XBRL Taxonomy Extension Presentation Link Document
104	XBRL tags for the cover page from the Company's Annual Report on Form 10-K for the year ended December 31, 2023, are embedded within the Inline XBRL document.

* Filed herewith

† Management contract or compensatory plan or arrangement

Attached as Exhibit 101 to this report are documents formatted in XBRL (Extensible Business Reporting Language): (i) Consolidated Balance Sheets as of December 31, 2023 and December 31, 2022, (ii) Consolidated Statements of Operations for the years ended December 31, 2023, 2022 and 2021, (iii) Consolidated Statements of Comprehensive Income (Loss) for the years ended December 31, 2023, 2022 and 2021, (iv) Consolidated Statements of Stockholders' Equity for the years ended December 31, 2023, 2022 and 2021, (v) Consolidated Statements of Cash Flows for the years ended December 31, 2023, 2022 and 2021, and (vi) Notes to Consolidated Financial Statements.

Item 16. Form 10-K Summary

None.

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.

BIOMARIN PHARMACEUTICAL INC.

Dated: February 26, 2024

By: /S/ BRIAN R. MUELLER

Brian R. Mueller
Executive Vice President, Finance & Chief Financial Officer

Exhibit 24.1

POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Alexander Hardy and Brian R. Mueller, his or her attorney-in-fact, with the power of substitution, for him or her in any and all capacities, to sign any amendments to the Annual Report on Form 10-K and to file the same, with exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, hereby ratifying and confirming all that each of said attorneys-in-fact, or his substitute or substitutes, may do or cause to be done by virtue hereof.

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:

Signature	Title	Date
/S/ ALEXANDER HARDY **Alexander Hardy**	President and Chief Executive Officer (Principal Executive Officer), Director	February 26, 2024
/S/ BRIAN R. MUELLER **Brian R. Mueller**	Executive Vice President, Finance & Chief Financial Officer (Principal Financial Officer)	February 26, 2024
/S/ ERIN BURKHART **Erin Burkhart**	Group Vice President, Chief Accounting Officer (Principal Accounting Officer)	February 26, 2024
/S/ RICHARD A. MEIER **Richard A. Meier**	Chair of the Board of Directors	February 26, 2024
/S/ MARK J. ALLES **Mark J. Alles**	Director	February 26, 2024
/S/ ELIZABETH MCKEE ANDERSON **Elizabeth McKee Anderson**	Director	February 26, 2024
/S/ JEAN-JACQUES BIENAIMÉ **Jean-Jacques Bienaimé**	Director	February 26, 2024
/S/ BARBARA BODEM **Barbara Bodem**	Director	February 26, 2024
/S/ ATHENA COUNTOURIOTIS, M.D. **Athena Countouriotis, M.D.**	Director	February 26, 2024
/S/ MARK ENYEDY **Mark Enyedy**	Director	February 26, 2024
/S/ ELAINE J. HERON **Elaine J. Heron**	Director	February 26, 2024
/S/ MAYKIN HO **Maykin Ho**	Director	February 26, 2024
/S/ ROBERT J. HOMBACH **Robert J. Hombach**	Director	February 26, 2024
/S/ V. BRYAN LAWLIS **V. Bryan Lawlis**	Director	February 26, 2024
/S/ DAVID PYOTT **David Pyott**	Director	February 26, 2024
/S/ DENNIS J. SLAMON **Dennis J. Slamon**	Director	February 26, 2024

BIOMARIN PHARMACEUTICAL INC.
INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

Report of Independent Registered Public Accounting Firm

To the Stockholders and Board of Directors
BioMarin Pharmaceutical Inc.:

Opinions on the Consolidated Financial Statements and Internal Control Over Financial Reporting

We have audited the accompanying consolidated balance sheets of BioMarin Pharmaceutical Inc. and subsidiaries (the Company) as of December 31, 2023 and December 31, 2022, the related consolidated statements of operations, comprehensive income (loss), stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2023, and the related notes (collectively, the consolidated financial statements). We also have audited the Company's internal control over financial reporting as of December 31, 2023, based on criteria established in *Internal Control – Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission.

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the financial position of the Company as of December 31, 2023 and December 31, 2022, and the results of its operations and its cash flows for each of the years in the three-year period ended December 31, 2023, in conformity with U.S. generally accepted accounting principles. Also in our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2023 based on criteria established in *Internal Control – Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission.

Basis for Opinion

The Company's management is responsible for these consolidated financial statements, for maintaining effective internal control over financial reporting, and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying Management's Annual Report on Internal Control Over Financial Reporting. Our responsibility is to express an opinion on the Company's consolidated financial statements and an opinion on the Company's internal control over financial reporting 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 audits to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud, and whether effective internal control over financial reporting was maintained in all material respects. Our audit of the consolidated financial statements included performing procedures to assess the risks of material misstatement of the consolidated 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 consolidated financial statements. Our audit also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. Our audit of internal control over financial reporting included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audits also included performing such other procedures as we considered necessary in the circumstances. We believe that our audits provide a reasonable basis for our opinions.

Definition and Limitations of Internal Control Over Financial Reporting

A company's 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. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

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.

Report of Independent Registered Public Accounting Firm

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current period audit of the consolidated financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the consolidated financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of a critical audit matter 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 matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Evaluation of variable consideration relating to ALDURAZYME product sales

As described in Notes 1 and 12 to the consolidated financial statements, during the year ended December 31, 2023 the Company recognized $131 million in ALDURAZYME net product revenue. Under its arrangement with Sanofi, the Company receives payments ranging from 39.5% to 50% on worldwide net ALDURAZYME sales by Sanofi, depending on Sanofi's sales volume. The Company estimates this variable consideration based on the amount that it expects to be entitled to from Sanofi's sales of ALDURAZYME. The Company recognizes this revenue upon satisfying the product performance obligation, which is when the product is shipped to Sanofi and all required quality control certificates are complete.

We identified the evaluation of variable consideration relating to ALDURAZYME net product revenue as a critical audit matter. Evaluating the key assumptions of forecasted Sanofi sales volume and average price per vial involved a high degree of subjective auditor judgment due to the nature of available supporting evidence being limited to Sanofi sales forecasts and historical sales and price data. Changes in these key assumptions could have had a significant impact on ALDURAZYME net product revenue.

The following are the primary procedures we performed to address this critical audit matter. We evaluated the design and tested the operating effectiveness of certain internal controls related to the Company's process for recognizing ALDURAZYME net product revenue. This included controls over forecasting Sanofi's sales volume and average price per vial used to estimate variable consideration. We evaluated the Company's ability to estimate the variable consideration by comparing historical estimates of sales volume and price per vial to actual current-period sales volume and price per vial of product sold by Sanofi. We also compared the Company's forecasts of future Sanofi sales volume and average price per vial to Sanofi's historical sales volume and price per vial.

/s/ KPMG LLP

We have served as the Company's auditor since 2002.

San Francisco, California
February 26, 2024

BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED BALANCE SHEETS

December 31, 2023 and 2022

(In thousands of U.S. Dollars, except share and per share amounts)

	December 31, 2023	December 31, 2022
ASSETS		
Current assets:		
Cash and cash equivalents	$ 755,127	$ 724,531
Short-term investments	318,683	567,006
Accounts receivable, net	633,704	461,316
Inventory	1,107,183	894,083
Other current assets	141,391	104,521
Total current assets	2,956,088	2,751,457
Noncurrent assets:		
Long-term investments	611,135	333,835
Property, plant and equipment, net	1,066,133	1,073,366
Intangible assets, net	294,701	338,569
Goodwill	196,199	196,199
Deferred tax assets	1,545,809	1,505,412
Other assets	171,538	176,236
Total assets	$ 6,841,603	$ 6,375,074
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 683,147	$ 572,959
Short-term convertible debt, net	493,877	—
Short-term contingent consideration	—	15,925
Total current liabilities	1,177,024	588,884
Noncurrent liabilities:		
Long-term convertible debt, net	593,095	1,083,019
Other long-term liabilities	119,935	100,015
Total liabilities	1,890,054	1,771,918
Stockholders' equity:		
Common stock, $0.001 par value: 500,000,000 shares authorized; 188,598,154 and 186,250,719 shares issued and outstanding, respectively	189	186
Additional paid-in capital	5,611,562	5,404,895
Company common stock held by the Nonqualified Deferred Compensation Plan	(9,860)	(8,859)
Accumulated other comprehensive loss	(28,788)	(3,867)
Accumulated deficit	(621,554)	(789,199)
Total stockholders' equity	4,951,549	4,603,156
Total liabilities and stockholders' equity	$ 6,841,603	$ 6,375,074

The accompanying notes are an integral part of these Consolidated Financial Statements.

BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF OPERATIONS

Years Ended December 31, 2023, 2022 and 2021

(In thousands of U.S. Dollars, except per share amounts)

	2023	2022	2021
REVENUES:			
Net product revenues	$ 2,372,538	$ 2,042,025	$ 1,783,498
Royalty and other revenues	46,688	54,014	62,777
Total revenues	2,419,226	2,096,039	1,846,275
OPERATING EXPENSES:			
Cost of sales	514,854	483,669	470,515
Research and development	746,773	649,606	628,793
Selling, general and administrative	937,291	854,009	759,375
Intangible asset amortization and contingent consideration	62,211	67,193	69,933
Gain on sale of nonfinancial assets, net	—	(108,000)	—
Total operating expenses	2,261,129	1,946,477	1,928,616
INCOME (LOSS) FROM OPERATIONS	158,097	149,562	(82,341)
Interest income	58,339	18,034	10,482
Interest expense	(17,335)	(15,970)	(15,337)
Other income (expense), net	(10,538)	(2,050)	11,846
INCOME (LOSS) BEFORE INCOME TAXES	188,563	149,576	(75,350)
Provision for (benefit from) income taxes	20,918	8,015	(11,270)
NET INCOME (LOSS)	$ 167,645	$ 141,561	$ (64,080)
EARNINGS (LOSS) PER SHARE, BASIC	$ 0.89	$ 0.76	$ (0.35)
EARNINGS (LOSS) PER SHARE, DILUTED	$ 0.87	$ 0.75	$ (0.35)
Weighted average common shares outstanding, basic	187,834	185,266	182,852
Weighted average common shares outstanding, diluted	191,595	188,963	182,852

The accompanying notes are an integral part of these Consolidated Financial Statements.

BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME (LOSS)

Years Ended December 31, 2023, 2022 and 2021

(In thousands of U.S. Dollars)

	2023	2022	2021
NET INCOME (LOSS)	$ 167,645	$ 141,561	$ (64,080)
OTHER COMPREHENSIVE INCOME (LOSS):			
Available-for-sale debt securities:			
Unrealized holding gain (loss) arising during the period, net of tax impact of $(3,922), $3,247 and $1,596, respectively	12,963	(10,720)	(5,262)
Cash flow hedges:			
Unrealized holding gain (loss) arising during the period, net of tax impact of $0 for all periods presented	(37,720)	29,045	34,379
Less: reclassifications to net income (loss), net of tax impact of $0 for all periods presented	164	36,624	(1,454)
Net change in unrealized holding gain (loss), net of tax	(37,884)	(7,579)	35,833
OTHER COMPREHENSIVE INCOME (LOSS), NET OF TAX	(24,921)	(18,299)	30,571
COMPREHENSIVE INCOME (LOSS)	$ 142,724	$ 123,262	$ (33,509)

The accompanying notes are an integral part of these Consolidated Financial Statements.

BIOMARIN PHARMACEUTICAL INC.
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
Years Ended December 31, 2023, 2022 and 2021
(In thousands of U.S. Dollars and share amounts in thousands)

	2023	2022	2021
Shares of Common Stock, beginning balances	186,251	183,913	181,741
Issuances under equity incentive plans	2,347	2,338	2,172
Shares of Common Stock, ending balances	188,598	186,251	183,913
Total stockholders' equity, beginning balances	$ 4,603,156	$ 4,265,669	$ 4,100,931
Common stock:			
Beginning balances	186	184	182
Issuances under equity incentive plans, net of tax	3	2	2
Ending balances	189	186	184
Additional paid-in capital:			
Beginning balances	5,404,895	5,191,502	4,993,407
Issuances under equity incentive plans, net of tax	(7,162)	14,328	3,389
Stock-based compensation	212,828	199,895	194,856
Change in Common stock held by the Nonqualified Deferred Compensation plan (NQDC)	1,001	(830)	(150)
Ending balances	5,611,562	5,404,895	5,191,502
Company common stock held by the NQDC:			
Beginning balances	(8,859)	(9,689)	(9,839)
Change in Common stock held by the NQDC	(1,001)	830	150
Ending balances	(9,860)	(8,859)	(9,689)
Accumulated other comprehensive income (loss):			
Beginning balances	(3,867)	14,432	(16,139)
Other comprehensive income (loss)	(24,921)	(18,299)	30,571
Ending balances	(28,788)	(3,867)	14,432
Accumulated deficit:			
Beginning balances	(789,199)	(930,760)	(866,680)
Net income (loss)	167,645	141,561	(64,080)
Ending balances	(621,554)	(789,199)	(930,760)
Total stockholders' equity, ending balances	$ 4,951,549	$ 4,603,156	$ 4,265,669

The accompanying notes are an integral part of these Consolidated Financial Statements.

BIOMARIN PHARMACEUTICAL INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
Years Ended December 31, 2023, 2022 and 2021
(In thousands of U.S. dollars)

	2023	2022	2021
CASH FLOWS FROM OPERATING ACTIVITIES:			
Net income (loss)	$ 167,645	$ 141,561	$ (64,080)
Adjustments to reconcile net income (loss) to net cash used in operating activities:			
Depreciation and amortization	104,386	101,969	108,039
Non-cash interest expense	4,188	4,117	4,146
Amortization of premium (accretion of discount) on investments	(9,228)	3,043	5,155
Stock-based compensation	207,099	196,308	197,263
Gain on sale of nonfinancial assets, net	—	(108,000)	—
Impairment of assets	38,608	—	—
Deferred income taxes	(44,981)	(52,087)	(15,608)
Unrealized foreign exchange loss (gain)	28,446	(14,287)	(1,810)
Non-cash changes in the fair value of contingent consideration	—	1,704	8,026
Other	(365)	(2,043)	(2,629)
Changes in operating assets and liabilities:			
Accounts receivable, net	(190,435)	(82,033)	65,574
Inventory	(157,058)	(68,264)	(35,060)
Other current assets	(50,335)	7,822	29,760
Other assets	(31,149)	(19,859)	(6,593)
Accounts payable and other short-term liabilities	68,853	59,018	15,689
Other long-term liabilities	23,585	6,933	(3,336)
Net cash provided by operating activities	159,259	175,902	304,536
CASH FLOWS FROM INVESTING ACTIVITIES:			
Purchases of property, plant and equipment	(96,691)	(120,959)	(95,578)
Maturities and sales of investments	864,863	619,995	691,049
Purchases of investments	(868,496)	(611,809)	(937,143)
Proceeds from sale of nonfinancial assets	—	103,325	—
Purchase of intangible assets	(10,920)	(10,581)	(23,647)
Other	—	—	(994)
Net cash used in investing activities	(111,244)	(20,029)	(366,313)
CASH FLOWS FROM FINANCING ACTIVITIES:			
Proceeds from exercises of awards under equity incentive plans	69,353	69,333	49,194
Taxes paid related to net share settlement of equity awards	(76,319)	(54,283)	(45,805)
Payments of contingent consideration	(9,475)	(31,095)	—
Principal repayments of financing leases	(2,286)	(2,605)	(3,039)
Other	—	—	(398)
Net cash used in financing activities	(18,727)	(18,650)	(48)
Effect of exchange rate changes on cash	1,308	32	(57)
NET INCREASE IN CASH AND CASH EQUIVALENTS	30,596	137,255	(61,882)
Cash and cash equivalents:			
Beginning of period	724,531	587,276	649,158
End of period	$ 755,127	$ 724,531	$ 587,276
SUPPLEMENTAL CASH FLOW DISCLOSURES:			
Cash paid for interest	$ 10,303	$ 10,281	$ 10,395
Cash paid for income taxes	$ 73,312	$ 54,372	$ 18,153
SUPPLEMENTAL CASH FLOW DISCLOSURES FOR NON-CASH INVESTING AND FINANCING ACTIVITIES:			
Increase (decrease) in accounts payable and accrued liabilities related to fixed assets	$ 164	$ (1,482)	$ (4,749)
Increase in accounts payable and accrued liabilities related to intangible assets	$ 6,904	$ 742	$ 9,428

The accompanying notes are an integral part of these Consolidated Financial Statements.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

(1) BUSINESS OVERVIEW AND SIGNIFICANT ACCOUNTING POLICIES

Nature of Operations

Founded in 1997, BioMarin Pharmaceutical Inc. (the Company or BioMarin) is a global biotechnology company dedicated to transforming lives through genetic discovery. The Company develops and commercializes targeted therapies that address the root cause of genetic conditions. The Company's robust research and development capabilities have resulted in multiple innovative commercial therapies for patients with rare genetic disorders. The Company's distinctive approach to drug discovery has produced a diverse pipeline of commercial, clinical, and pre-clinical candidates that address a significant unmet medical need, have well-understood biology, and provide an opportunity to be first-to-market or offer a substantial benefit over existing treatment options.

Basis of Presentation

These Consolidated Financial Statements have been prepared pursuant to United States generally accepted accounting principles (U.S. GAAP) and the rules and regulations of the Securities and Exchange Commission (the SEC) for Annual Reports on Form 10-K and include the accounts of BioMarin and its wholly owned subsidiaries. All intercompany transactions have been eliminated. Management performed an evaluation of the Company's activities through the date of filing of this Annual Report on Form 10-K, and has concluded that there were no subsequent events or transactions that occurred subsequent to the balance sheet date and prior to the filing of this Annual Report on Form 10-K.

Use of Estimates

U.S. GAAP requires management to make estimates and assumptions that affect amounts reported on the Company's Consolidated Financial Statements and accompanying disclosures. Although these estimates are based on management's best knowledge of current events and actions that the Company may undertake in the future, actual results may be different from those estimates. The Consolidated Financial Statements reflect all adjustments of a normal, recurring nature that are, in the opinion of management, necessary for a fair presentation of results.

Significant Accounting Policies

Cash and Cash Equivalents

The Company treats highly liquid investments, readily convertible to cash, with original maturities of three months or less on the purchase date as cash equivalents.

Marketable and Non-Marketable Securities

Marketable Securities

The Company determines the appropriate classification of its investments in debt and equity securities at the time of purchase and reevaluates such designations at each reporting period. The Company classifies its debt and equity securities with original maturities greater than three months when purchased as either short-term or long-term investments based on each instrument's underlying contractual maturity date and its availability for use in current operations.

All marketable securities are classified as available-for-sale. Available-for-sale debt securities are measured and recorded at fair market value with unrealized gains and losses included in Accumulated Other Comprehensive Income (AOCI) on the Company's Consolidated Balance Sheets, with the exception of any declines in fair value below the cost basis that are a result of a credit loss, which, if any, are reported in Other Income (Expense), Net in the current period through an allowance for credit losses. Impairment assessments are made at the individual security level each reporting period. When the fair value of an investment is less than its cost at the balance sheet date, a determination is made as to whether the impairment is related to a credit loss and, if so, an impairment loss is recognized in earnings equal to the difference between the investment's amortized cost and fair value at such date.

Non-Marketable Equity Securities

The Company records investments in equity securities, other than equity method investments, at fair market value, if fair value is readily determinable. Equity securities with no readily determinable fair values are recorded using the measurement alternative of cost adjusted for observable price changes in orderly transactions for identical or similar investments of the same

issuer less impairment, if any. Investments in equity securities are recorded in Other Assets on the Company's Consolidated Balance Sheets. Unrealized gains and losses are reported in Other Income (Expense), Net. The Company regularly reviews its non-marketable equity securities for indicators of impairment.

Inventory

Commercial Inventory

The Company values inventory at the lower of cost and net realizable value and determines the cost of inventory using the average-cost approach on the first-in, first out (FIFO) method. The Company analyzes its inventory levels quarterly for obsolescence and, if required, adjusts inventory to its net realizable value if the cost basis of inventory is in excess of its expected net realizable value, or for quantities in excess of expected demand. If the Company determines cost exceeds its net realizable value, the resulting adjustments are recognized as Cost of Sales in the Consolidated Statements of Operations.

Inventory Produced Prior to Regulatory Approval

When future commercialization for a product candidate is considered probable and management believes that material uncertainties related to the ultimate regulatory approval have been significantly reduced and the Company expects to realize economic benefit in the future, the Company capitalizes pre-launch or pre-qualification manufacturing costs prior to regulatory approval. For inventories that are capitalized in preparation of product launch, a number of factors are taken into consideration based on information available at the time, including the product candidate's current status in the drug development and regulatory approval process, results from the related pivotal clinical trial, results from meetings with the relevant regulatory authorities prior to the filing of regulatory applications, historical experience, as well as potential impediments to the approval process such as product safety or efficacy, as well as commercialization and market trends. If additional requirements are subsequently presented by the regulatory authorities, prior to their final decision thus extending anticipated regulatory approval timelines resulting in expiration of the product prior to revised demand forecasts, the pre-launch inventory costs are expensed to Cost of Sales. If the marketing application is ultimately rejected by the applicable regulators and the pre-launch inventory cannot be sold for commercial use, the pre-launch inventory costs are expensed to Research and Development (R&D).

Property, Plant and Equipment

Property, plant and equipment are stated at historical cost net of accumulated depreciation. Depreciation is computed using the straight-line method over the related estimated useful lives, as presented in the table below. Significant additions and improvements are capitalized, whereas repairs and maintenance are expensed as incurred. Depreciation of property, plant and equipment are included in Cost of Sales, R&D and Selling, General and Administrative (SG&A), as appropriate, in the Consolidated Statements of Operations. Property and equipment purchased for specific R&D projects with no alternative future uses are expensed as incurred and recorded to R&D in the Consolidated Statements of Operations.

Leasehold improvements	Shorter of life of asset or lease term
Building and improvements	20 to 50 years
Manufacturing and laboratory equipment	5 to 15 years
Computer hardware and software	3 to 7 years
Office furniture and equipment	5 years
Land improvements	10 to 20 years
Land	Not applicable
Construction-in-progress	Not applicable

Leases

The Company's lease portfolio primarily consists of leases for properties and equipment for administrative, manufacturing and R&D activities. The Company determines if an arrangement is a lease at contract inception. For leases where the Company is the lessee, Right of Use (ROU) assets represent the Company's right to use the underlying asset for the term of the lease and the lease liabilities represent the lease payment obligation. ROU assets and lease liabilities are recognized at the lease commencement date based on the present value of the future lease payments over the lease term. The Company uses its incremental borrowing rate based on the information available at the commencement date of the underlying lease arrangement to determine the present value of lease payments. The ROU asset also includes any prepaid lease payments and any lease incentives received. The lease term to calculate the ROU asset and related lease liability includes options to extend or terminate

the lease when it is reasonably certain that the Company will exercise the option. The Company's lease agreements generally do not contain any material variable lease payments, residual value guarantees or restrictive covenants.

Lease expense for operating leases is recognized on a straight-line basis over the lease term as an operating expense while expense for financing leases is recognized as depreciation expense and interest expense using the accelerated interest method of recognition. When an arrangement requires payments for lease and non-lease components, the Company has elected to account for lease and non-lease components separately. Lease expense for leases with a term of twelve months or less is recognized on a straight-line basis and are not included in the recognized ROU assets and lease liabilities.

Goodwill and Intangible Assets

The Company records goodwill in a business combination when the total consideration exceeds the fair value of the assets acquired.

Intangible assets with indefinite useful lives are related to purchased in-process research and development (IPR&D) projects and are measured at their respective fair values as of the acquisition date. Intangible assets related to IPR&D projects are considered to be indefinite-lived until the completion or abandonment of the associated R&D efforts. If and when development is complete, which generally occurs if and when regulatory approval to market a product is obtained, the associated assets are considered finite-lived and are amortized using the straight-line method based on their respective estimated useful lives at that point in time. The amortization of these intangible assets is included in Intangible Asset Amortization and Contingent Consideration in the Consolidated Statements of Operations.

Intangible assets with finite useful lives primarily consist of acquired intellectual property and royalty rights, regulatory approval and first commercial sales milestone payments as well as costs associated with technology transfer to qualify third-party manufacturing facilities for commercial production. Intangible assets are recorded at cost, net of accumulated amortization, and amortize over their estimated useful lives on a straight-line basis. Amortization expense is recorded in Intangible Asset Amortization and Contingent Consideration on the Company's Consolidated Statements of Operations, except for amortization expense related to the technology transfer, which is recorded in Cost of Sales.

Impairment

The Company assesses goodwill and indefinite-lived intangible assets for impairment annually in the fourth quarter, or more frequently as warranted by events or changes in circumstances that indicate that the carrying amount may not be recoverable.

Goodwill is assessed for impairment by comparing the fair value of the Company's reporting unit with its carrying amount. If the carrying value of the reporting unit exceeds its fair value, an impairment loss equal to the difference would be recorded.

Indefinite-lived intangible assets are assessed for impairment first by performing a qualitative assessment. If the qualitative assessment indicates that it is more likely than not that the fair value of an indefinite-lived intangible asset is less than its carrying amount, then the Company will perform a quantitative assessment and record an impairment loss.

Long-lived Asset Impairment

The Company's long-lived assets consist of property, plant and equipment, leased ROU assets and finite-lived intangible assets, which includes costs associated with technology transfer to qualify manufacturing facilities for commercial production. Should there be an indication of impairment, the Company tests for recoverability by comparing the estimated undiscounted future cash flows expected to result from the use of the asset or asset group and its eventual disposition to the carrying amount of the asset or asset group. Any excess of the carrying value of the asset or asset group over its estimated fair value is recognized as an impairment loss. Impairment charges related to property, plant or equipment that are not material are recorded to depreciation expense and presented in SG&A in the Consolidated Statements of Operations. Impairment charges for finite-lived intangible assets associated with technology transfer costs that are not material are recorded to Cost of Sales in the Consolidated Statements of Operations. Impairment charges related to all other finite-lived intangible assets that are not material are recorded to Intangible Asset Amortization and Contingent Consideration in the Consolidated Statements of Operations.

Capitalized Software

The Company capitalizes software development costs associated with internal use software, including external direct costs of materials and services and payroll costs for employees devoting time to a software project. Costs incurred during the preliminary project stage, as well as costs for maintenance and training, are expensed as incurred. When placed in service, implementation costs are subsequently amortized on a straight-line basis over the expected useful life of the asset. As of

December 31, 2023, $30.6 million of capitalized costs associated with cloud computing arrangements were included in Other Assets on the Company's Consolidated Balance Sheets.

Revenue Recognition

The Company recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the Company expects to receive in exchange for those goods or services. To determine revenue recognition for contracts with customers, the Company performs the following five steps:

(i) identification of the promised goods or services in the contract;

(ii) determination of whether the promised goods or services are performance obligations including whether they are distinct in the context of the contract;

(iii) measurement of the transaction price, including the constraint on variable consideration;

(iv) allocation of the transaction price to the performance obligations based on estimated selling prices; and

(v) recognition of revenue when (or as) the Company satisfies each performance obligation. A performance obligation is a promise in a contract to transfer a distinct good or service to the customer and is the unit of account.

Net Product Revenues

In the U.S., the Company's commercial products, except for PALYNZIQ and ALDURAZYME, are generally sold to specialty pharmacies or end-users, such as hospitals, which act as retailers. PALYNZIQ is distributed in the U.S. through certain certified specialty pharmacies under the PALYNZIQ Risk Evaluation and Mitigation Strategy (REMS) and ALDURAZYME is marketed world-wide by Sanofi. Outside the U.S., the Company's commercial products are sold to its authorized distributors or directly to government purchasers or hospitals, which act as the end-users. Revenues from product sales are recognized when the customer obtains control of the Company's product, which occurs at a point in time, typically upon shipment to the customer. The Company's payment terms vary by customer, jurisdiction or, in some instances, by product. With the exception of Sanofi and certain outcomes-based contracts, most of the Company's payment terms are based on customary commercial terms and are generally less than one year after the customer obtains control. The Company does not adjust revenue for the effects of a significant financing component for contracts if the period between the transfer of control and corresponding payment is expected to be one year or less. Amounts collected from customers and remitted to governmental authorities, which primarily consist of value-added taxes related to product sales in foreign jurisdictions, are presented on a net basis on the Company's Consolidated Statements of Operations, in that taxes billed to customers are not included as a component of Net Product Revenues.

For ALDURAZYME revenues, the Company receives a payment ranging from 39.5% to 50% on worldwide net ALDURAZYME sales by Sanofi depending on sales volume, which is included in Net Product Revenues on the Company's Consolidated Statements of Operations. The Company recognizes its best estimate of the revenue it expects to earn when the product is released and control is transferred to Sanofi. The Company records ALDURAZYME net product revenues based on the estimated variable consideration payable when the product is sold through by Sanofi. Actual amounts of consideration ultimately received may differ from the Company's estimates. Differences between the estimated variable consideration to be received from Sanofi and actual payments received are not expected to be material. If actual results vary from the Company's estimates, the Company will make adjustments, which would affect Net Product Revenues and earnings in the period such variances become known.

Revenue Reserves

Revenues from product sales are recorded at the net sales price (transaction price), which includes estimates of variable consideration for which reserves are established and which result from government and commercial rebates, chargebacks, sales returns, and other incentives that are offered within contracts between the Company and its customers, such as specialty pharmacies, hospitals, authorized distributors and government purchasers. These reserves are based on the amounts earned or to be claimed on the related sales and are classified as reductions of accounts receivable (if the amount is payable to the customer) or a current liability (if the amount is payable to a party other than a customer). Where appropriate, these estimates take into consideration a range of possible outcomes that are probability-weighted for relevant factors such as the Company's historical experience, current contractual and statutory requirements, specific known market events and trends, patient outcomes, industry data and forecasted customer buying and payment patterns. Overall, these reserves reflect the Company's best estimates of the amount of consideration to which it is entitled based on the terms of the contract. The amount of variable consideration that is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Actual amounts of consideration ultimately received may differ from the Company's estimates, however the Company does not expect any such

difference to be material. If actual results in the future vary from the Company's estimates, the Company will adjust its estimates, which would affect net product revenue and earnings in the period such variances become known.

Government and Commercial Rebates: The Company records reserves for rebates payable under government programs, such as Medicaid, and commercial arrangements, such as managed care rebates, as a reduction of revenue at the time product revenues are recorded. The Company's reserve calculations require estimates, including estimates of customer mix and patient outcomes, to determine which sales will be subject to rebates and the amount of such rebates. The Company updates its estimates and assumptions on a quarterly basis and records any necessary adjustments to its reserves.

Sales Returns: The Company records allowances for product returns, if appropriate, as a reduction of revenue at the time product sales are recorded. Several factors are considered in determining whether an allowance for product returns is required, including market exclusivity of the products based on their orphan drug status, the patient population, the customers' limited return rights and the Company's historical experience with returns. Because of the pricing of the Company's commercial products, the limited number of patients and the customers' limited return rights, most customers and retailers carry a limited inventory. The Company relies on historical return rates to estimate a reserve for returns. Based on these factors and the fact that the Company has not experienced significant product returns to date, return allowances are not material.

Other Incentives: Other incentives include fees paid to the Company's distributors and discounts for prompt payment. The Company also offers a branded co-pay assistance program for eligible patients with commercial insurance in the U.S. who are on an eligible BioMarin product. The branded co-pay assistance programs assist commercially insured patients who have coverage for an eligible BioMarin product and are intended to reduce each participating patient's portion of the financial responsibility of the purchase price up to a specified dollar amount of assistance. The Company records fees paid to distributors, cash discounts and amounts paid under the brand specific co-pay assistance program for each patient as a reduction of revenue.

Royalty and Other Revenues

Royalties: For arrangements that include the receipt of sales-based royalties, including milestone payments based on the level of sales when the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (a) when the related sales occur, or (b) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied).

Licenses of intellectual property: If the license to the Company's intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenues from non-refundable, up-front fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license. For licenses that are bundled with other promises, the Company uses judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue from non-refundable, up-front fees. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition.

Milestone payments: At the inception of each arrangement that includes developmental, regulatory or commercial milestone payments, the Company evaluates whether achieving the milestones is considered probable and estimates 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 value of the associated milestone (such as a regulatory submission by the Company) is included in the transaction price. Milestone payments that are not within the control of the Company, such as approvals from regulators or where attainment of the specified event is dependent on the development activities of a third party, are not considered probable of being achieved until those approvals are received or the specified event occurs. Revenue is recognized from the satisfaction of performance obligations in the amount billable to the customer.

Research and Development

R&D costs are generally expensed as incurred. These expenses include contract R&D services provided by third parties, preclinical and clinical studies, raw materials costs associated with manufacturing clinical product, quality control and assurance, other R&D activities, facilities and regulatory costs and R&D-related personnel costs including salaries, benefits and stock-based compensation. Upfront and milestone payments made to third parties in connection with licensed intellectual property, which does not have an alternative future use or does not reach technological feasibility, are expensed as incurred up to the point of regulatory

approval. Advance payments for goods or services for use in research and development activities are capitalized and recorded in other current assets, and then expensed as the related goods are delivered or the services are performed.

Advertising Expenses

The costs of advertising are presented in SG&A in the Consolidated Statements of Operations and are expensed as incurred. Advertising expenses were $27.8 million, $25.2 million and $30.2 million in 2023, 2022 and 2021, respectively.

Earnings (Loss) Per Common Share

Basic earnings (loss) per share is calculated by dividing Net Income (Loss) by the weighted average shares of common stock outstanding during the period. Diluted earnings (loss) per share reflects the potential dilution that would occur if securities or other contracts to issue common stock were exercised or converted into common stock; however, potential common equivalent shares are excluded if their effect is anti-dilutive.

Stock-Based Compensation

The Company has equity incentive plans under which various types of equity-based awards may be granted to employees. Stock-based compensation expense is recognized on a straight-line basis over the requisite service period, which is generally the vesting period required to obtain full vesting, and is classified as Cost of Sales, R&D or SG&A, as appropriate, in the Consolidated Statements of Operations. The Company accounts for forfeitures as they occur.

Restricted Stock Units

The fair value of restricted stock units (RSUs) with service-based vesting conditions and RSUs with performance conditions is determined to be the fair market value of the Company's underlying common stock on the date of grant. The stock-based compensation expense for RSUs with service-based vesting is recognized over the period during which the vesting restrictions lapse. Stock-based compensation expense for RSUs with performance conditions is recognized beginning in the period the Company determines it is probable that the performance condition will be achieved. Management expectations related to the achievement of performance goals associated with RSUs with performance conditions are assessed regularly to determine whether such grants are expected to vest. The fair value for RSUs with market conditions is estimated using the Monte Carlo valuation model, utilizing expected volatility rates derived from those of the Company and the members of the referenced peer group. Related stock-based compensation is recognized, beginning on the grant date, on a straight-line basis regardless of whether the market condition is met unless the required service is not performed.

Stock Options and Purchase Rights

The fair value of each stock option award and purchase rights under the Company's Employee Stock Purchase Plan (ESPP) are estimated on the date of grant using the Black-Scholes valuation model and the following assumptions: expected term, expected volatility, risk-free interest rate and expected dividend yield. The dividend yield reflects that the Company has not paid any cash dividends since inception and does not intend to pay any cash dividends in the foreseeable future. The expected term of stock options is based on observed historical exercise patterns. In estimating the life of stock options, the Company has identified two employee groups with distinctly different historical exercise patterns: executive and non-executive. The executive employee group has a history of holding stock options for longer periods than non-executive employees. The expected term of purchase rights for ESPP is based on each tranche of an offering period, which is four tranches in a twenty-four-month period.

The determination of the fair value of stock-based payment awards using an option-pricing model is affected by the Company's stock price and may use assumptions regarding a number of complex and subjective variables.

Income Taxes

The Company calculates and provides for income taxes in each of the tax jurisdictions in which it operates. Deferred tax assets and liabilities, measured using enacted tax rates, are recognized for the future tax consequences of temporary differences between the tax and financial statement basis of assets and liabilities. A valuation allowance reduces the deferred tax assets to the amount that is more likely than not to be realized. The Company establishes liabilities or reduces assets for uncertain tax positions when the Company believes certain tax positions are not more likely than not of being sustained if challenged. Each quarter, the

Company evaluates these uncertain tax positions and adjusts the related tax assets and liabilities in light of changing facts and circumstances.

The Company uses financial projections to support its net deferred tax assets, which contain significant assumptions and estimates of future operations. If such assumptions were to differ significantly, it may have a material impact on the Company's ability to realize its deferred tax assets. At the end of each period, the Company will reassess the ability to realize its deferred tax benefits. If it is more likely than not that the Company would not realize the deferred tax benefits, a valuation allowance may need to be established against all or a portion of the deferred tax assets, which will result in a charge to tax expense.

Foreign Currency

For the Company and its subsidiaries, the functional currency has been determined to be the U.S. Dollar (USD). Assets and liabilities denominated in foreign currency are remeasured at period-end exchange rates for monetary assets and liabilities. Non-monetary assets and liabilities denominated in foreign currencies are remeasured at historical rates. Foreign currency transaction losses resulting from remeasurement recognized in SG&A in the Consolidated Statements of Operations totaled $27.7 million, $11.4 million and $11.7 million in 2023, 2022 and 2021, respectively.

Derivatives and Hedging Activities

The Company uses foreign currency exchange forward contracts (forward contracts) to hedge certain operational exposures resulting from potential changes in foreign currency exchange rates. Such exposures result from portions of the Company's forecasted revenues and operating expenses being denominated in currencies other than the USD, primarily the Euro. The Company designates certain of these forward contracts as hedging instruments and also enters into forward contracts that are considered to be economic hedges that are not designated as hedging instruments. Whether designated or undesignated, these forward contracts protect against the reduction in value of forecasted foreign currency cash flows resulting from gross product revenues, operating expenses and monetary asset or liability positions designated in currencies other than the USD. To receive hedge accounting treatment, cash flow hedges must be highly effective in offsetting changes to expected future cash flows on hedged transactions. The Company does not hold or issue derivative instruments for trading or speculative purposes.

The Company is exposed to counterparty credit risk on its derivatives. The Company has established and maintains strict counterparty credit guidelines and enters into hedging agreements with financial institutions that are investment grade or better to minimize the Company's exposure to potential defaults. The Company is not required to pledge collateral under these agreements.

The Company accounts for its derivative instruments as either assets or liabilities on its Consolidated Balance Sheets and measures them at fair value, which is estimated using current exchange and interest rates and takes into consideration the current creditworthiness of the counterparties or the Company, as applicable. For derivatives designated as hedging instruments, the entire change in the fair value of qualifying derivative instruments is recorded in AOCI and amounts deferred in AOCI are reclassified to earnings in the same line item in which the earnings effect of the hedged item is reported. Derivatives not designated as hedging instruments are adjusted to fair value through earnings in SG&A in the Consolidated Statements of Operations.

Fair Value of Financial Instruments

The Company applies fair value accounting for all financial assets and liabilities and non-financial assets and liabilities that are recognized or disclosed at fair value in the financial statements on a recurring basis. The Company defines fair value as the price that would be received from selling an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. When determining the fair value measurements for assets and liabilities that are required to be recorded at fair value, the Company considers the principal or most advantageous market in which the Company would transact and the market-based risk measurements or assumptions that market participants would use to price the asset or liability, such as risks inherent in valuation techniques, transfer restrictions and credit risk. When estimating fair value, depending on the nature and complexity of the asset or liability, the Company may use the following techniques:

- Income approach, which is based on the present value of a future stream of net cash flows
- Market approach, which is based on market prices and other information from market transactions involving identical or comparable assets or liabilities.

The Company's fair value methodologies depend on the following types of inputs:

- Quoted prices for identical assets or liabilities in active markets (Level 1 inputs)
- Quoted prices for similar assets or liabilities in active markets, or quoted prices for identical or similar assets or liabilities that are not active, or inputs other than quoted process that are directly or indirectly observable, or

inputs that are derived principally from, or corroborated by, observable market data by correlation or other means (Level 2 inputs)

- Unobservable inputs that reflect estimates and assumptions (Level 3 inputs)

The Company's Level 2 instruments are valued using third-party pricing sources. The pricing services 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 reported trades of and broker/dealer quotes on the same or similar securities, issuer credit spreads, benchmark securities, prepayment/default projections based on historical data and other observable inputs. The Company validates the prices provided by its third-party pricing services by understanding the models used, obtaining market values from other pricing sources, analyzing pricing data in certain instances and confirming those securities traded in active markets.

The Company's Level 3 financial assets and liabilities include acquired intangible assets and contingent consideration resulting from business acquisitions. The estimated fair value of acquired finite-lived and indefinite-lived intangible assets and contingent consideration are measured by applying a probability-based income approach utilizing an appropriate discount rate as of the acquisition date. Key assumptions used by management to estimate the fair value of contingent consideration include estimated probabilities, the estimated timing of when a milestone may be attained and assumed discount periods and rates. Changes in the fair value of contingent consideration can result from changes to one or more inputs, including the estimated probability with respect to regulatory approval, changes in the assumed timing of when milestones are likely to be achieved and changes in assumed discount periods and rates. Contingent consideration is remeasured on a recurring basis and resulting changes in the fair value, due to the revision of key assumptions, are recorded in Intangible Asset Amortization and Contingent Consideration on the Company's Consolidated Statements of Operations.

See Notes 2, 7, 8, and 10 to these Consolidated Financial Statements for further information on the nature of these financial instruments.

Segment Information

The Company currently operates in one segment focused on the development and commercialization of innovative therapies for people with serious and life-threatening rare diseases and medical conditions. A single management team reports to the chief operating decision maker who comprehensively manages the entire business. All products are included in one operating segment because the majority of the Company's products have similar economic and other characteristics, including the nature of the products and production processes, type of customers, distribution methods and regulatory environment. The Company is not organized by market and is managed and operated as one business. The Company does not operate any separate lines of business or separate business entities with respect to its products. Accordingly, the Company does not accumulate discrete financial information with respect to separate products, other than revenues, cost of sales and certain other operating expenses.

Recent Accounting Pronouncements

There have been no new accounting pronouncements adopted by the Company during 2023. The following paragraphs discuss new accounting pronouncements issued by the Financial Accounting Standards Board (FASB), but not yet adopted by the Company.

Segment Reporting

In November 2023, the FASB issued Accounting Standards Update (ASU) 2023-07, Segment Reporting Topic 280, *Improvements to Reportable Segment Disclosures*, to improve reportable segment disclosure requirements through enhanced disclosures about significant segment expenses. ASU 2023-07 expands public entities' segment disclosures by requiring disclosure of significant segment expenses that are regularly provided to the chief operating decision maker and included within each reported measure of segment profit or loss, an amount and description of its composition for other segment items and interim disclosures of a reportable segment's profit or loss and assets. The effective date for the update is for fiscal years beginning after December 15, 2023 and interim periods within fiscal years beginning after December 15, 2024 and should be applied on a retrospective basis to all periods presented. The Company is currently evaluating the effect of adopting the update on the Company's disclosures.

Income Taxes

In December 2023, the FASB issued ASU 2023-09, Income Taxes Topic 740, *Improvements to Income Tax Disclosures*. The guidance requires disclosure of disaggregated information about the Company's effective tax rate reconciliation as well as information on income taxes paid. The disclosure requirements will be applied on a prospective basis, with the option to apply it

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

retrospectively. The effective date for the update is for fiscal years beginning after December 15, 2024. The Company is currently evaluating the effect of the update on the Company's related disclosures.

(2) FINANCIAL INSTRUMENTS

The following tables show the Company's cash, cash equivalents and available-for-sale securities by significant investment category as of December 31, 2023 and 2022, respectively:

	December 31, 2023						
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Aggregate Fair Value	Cash and Cash Equivalents	Short-term Marketable Securities [1]	Long-term Marketable Securities [2]
Level 1:							
Cash	$ 229,676	$ —	$ —	$ 229,676	$ 229,676	$ —	$ —
Level 2:							
Money market instruments	499,483	—	—	499,483	499,483	—	—
Corporate debt securities	587,896	3,476	(1,996)	589,376	—	193,251	396,125
U.S. government agency securities	251,952	556	(1,140)	251,368	19,976	111,343	120,049
Commercial paper	20,076	5	—	20,081	5,992	14,089	—
Asset-backed securities	94,744	351	(134)	94,961	—	—	94,961
Subtotal	1,454,151	4,388	(3,270)	1,455,269	525,451	318,683	611,135
Total	$ 1,683,827	$ 4,388	$ (3,270)	$ 1,684,945	$ 755,127	$ 318,683	$ 611,135

	December 31, 2022						
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Aggregate Fair Value	Cash and Cash Equivalents	Short-term Marketable Securities [1]	Long-term Marketable Securities [2]
Level 1:							
Cash	$ 463,248	$ —	$ —	$ 463,248	$ 463,248	$ —	$ —
Level 2:							
Money market instruments	248,933	—	—	248,933	248,933	—	—
Corporate debt securities	504,984	34	(11,541)	493,477	1,881	299,153	192,443
U.S. government agency securities	312,720	45	(3,771)	308,994	—	229,846	79,148
Commercial paper	48,103	11	(22)	48,092	10,469	37,623	—
Asset-backed securities	63,151	69	(592)	62,628	—	384	62,244
Subtotal	1,177,891	159	(15,926)	1,162,124	261,283	567,006	333,835
Total	$ 1,641,139	$ 159	$ (15,926)	$ 1,625,372	$ 724,531	$ 567,006	$ 333,835

(1) The Company's short-term marketable securities mature in one year or less.

(2) The Company's long-term marketable securities mature between one and five years.

As of December 31, 2023, the Company had the ability and intent to hold all investments that were in an unrealized loss position until maturity. The Company considered its intent and ability to hold the securities until recovery of amortized cost basis, the extent to which fair value is less than amortized cost basis, conditions specifically related to the security's industry and

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

geography, payment structure and history and changes to the ratings (if any) in determining that the decline in fair value compared to carrying value is not related to a credit loss.

The Company has certain investments in non-marketable equity securities, measured using unobservable valuation inputs and remeasured on a nonrecurring basis, which are collectively considered strategic investments. As of December 31, 2023 and December 31, 2022, the fair value of the Company's strategic investments was $11.3 million and $23.9 million, respectively. These investments were recorded in Other Assets on the Company's Consolidated Balance Sheets. In 2023, based on new developments, the Company concluded that factors existed indicating it would no longer realize a $12.6 million equity investment in its non-marketable securities. The loss on the equity investment due to impairment was recorded to Other Income (Expense), Net on the Company's Consolidated Statements of Operations.

See Note 1 to these Consolidated Financial Statements for additional discussion regarding the Company's fair value measurements.

(3) INTANGIBLE ASSETS

Intangible Assets, Net consisted of the following:

	December 31,	
	2023	2022
Finite-lived intangible assets	$ 710,011	$ 690,871
Accumulated amortization	(415,310)	(352,302)
Net carrying value	$ 294,701	$ 338,569

The following table summarizes the carrying value and estimated remaining life of the Company's finite-lived intangible assets as of December 31, 2023:

	Net Balance	Average Remaining Life
Acquired intellectual property	$ 185,344	4.4 years
Technology transfer	89,221	4.4 years[1]
License payments [2]	20,136	9.8 years
Total	$ 294,701	

(1) Certain technology transfer assets have not yet been placed into service. The average remaining life presented is only for those placed into service.

(2) License payments include finite-lived intangible assets due to the Company's achievement in 2023 of first commercial sale milestones related to ROCTAVIAN.

As of December 31, 2023, the estimated future amortization expense associated with the Company's finite-lived intangible assets that have been placed into service, was as follows:

Fiscal Year	Amount
2024	$ 58,200
2025	38,924
2026	38,924
2027	38,924
2028	23,174
Thereafter	7,293
	$ 205,439

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

(4) PROPERTY, PLANT AND EQUIPMENT

Property, Plant and Equipment, Net, consisted of the following:

	December 31	
	2023	2022
Building and improvements	$ 860,807	$ 819,100
Manufacturing and laboratory equipment	538,677	475,663
Computer hardware and software	211,482	214,829
Land	90,781	90,786
Leasehold improvements	58,230	59,532
Furniture and equipment	46,453	45,762
Land improvements	26,779	26,455
Construction-in-progress [1]	100,013	143,384
	1,933,222	1,875,511
Accumulated depreciation	(867,089)	(802,145)
Total property, plant and equipment, net	$ 1,066,133	$ 1,073,366

(1) In the fourth quarter of 2023, the Company decided to cease development of the first generation VOXZOGO pen device and impaired $14.0 million of capitalized tooling and fixed assets that had not been placed in service.

Depreciation expense, net of amounts capitalized into inventory, was $40.3 million, $38.6 million and $46.1 million for the years ended December 31, 2023, 2022 and 2021, respectively.

(5) INVENTORY

Inventory consisted of the following:

	December 31	
	2023	2022
Raw materials	$ 155,704	$ 131,071
Work-in-process	571,107	410,656
Finished goods	380,372	352,356
Total inventory	$ 1,107,183	$ 894,083

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

(6) SUPPLEMENTAL FINANCIAL STATEMENT INFORMATION

Accounts Payable and Accrued Liabilities consisted of the following:

	December 31,	
	2023	2022
Accounts payable and accrued operating expenses	$ 315,509	$ 231,238
Accrued compensation expense	201,067	207,573
Accrued rebates payable	96,179	72,654
Foreign currency exchange forward contracts	33,853	12,601
Accrued royalties payable	14,299	13,306
Lease liability	8,779	10,375
Accrued income taxes	2,651	16,213
Deferred revenue	4,620	711
Other	6,190	8,288
Total accounts payable and accrued liabilities	$ 683,147	$ 572,959

Reorganization Plan Costs

On October 6, 2022, the Company announced a plan to simplify its organizational design, which included a planned reduction in headcount. The Company recorded costs of $23.0 million in 2022 and negligible adjustments in 2023 related to one-time termination severance and employee termination benefits within SG&A expense. As of December 31, 2022, $11.1 million was included in Accounts Payable and Accrued Liabilities on the Company's Consolidated Balance Sheet. As of December 31, 2023, all accrued costs have been paid.

Significant Revenue Rebates and Reserves for Cash Discounts

The roll forward of significant estimated accrued rebates and reserve for cash discounts for the years ended December 31, 2023, 2022 and 2021, were as follows:

	Balance at Beginning of Period	Provision for Current Period Sales	Payments	Balance at End of Period
Year ended December 31, 2023:				
Accrued rebates	$ 72,654	$ 196,864	$ (173,339)	$ 96,179
Reserve for cash discounts	$ 3,639	$ 21,081	$ (19,330)	$ 5,390
Year ended December 31, 2022:				
Accrued rebates	$ 47,987	$ 140,260	$ (115,593)	$ 72,654
Reserve for cash discounts	$ 2,013	$ 20,351	$ (18,725)	$ 3,639
Year ended December 31, 2021:				
Accrued rebates	$ 65,526	$ 116,691	$ (134,230)	$ 47,987
Reserve for cash discounts	$ 1,716	$ 16,003	$ (15,706)	$ 2,013

(7) FAIR VALUE MEASUREMENTS

The Company measures certain financial assets and liabilities at fair value in accordance with the policy described in Note 1 to these Consolidated Financial Statements.

The following tables present the classification within the fair value hierarchy of financial assets and liabilities not disclosed elsewhere in these Consolidated Financial Statements that are remeasured on a recurring basis as of December 31, 2023 and 2022. Other than the Company's fixed-rate convertible debt disclosed in Note 10 to these Consolidated Financial Statements, there

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

were no financial assets or liabilities that were remeasured using a quoted price in active markets for identical assets (Level 1) as of December 31, 2023 and 2022. Refer to Notes 2 and 8 to these Consolidated Financial Statements for other financial assets and liabilities measured at fair value.

	Fair Value Measurements as of December 31, 2023		
	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:			
Other current assets:			
NQDC Plan assets	$ 2,026	$ —	$ 2,026
Other assets:			
NQDC Plan assets	28,119	—	28,119
Restricted investments (1)	2,393	—	2,393
Total other assets	30,512	—	30,512
Total assets	$ 32,538	$ —	$ 32,538
Liabilities:			
Current liabilities:			
NQDC Plan liability	$ 2,026	$ —	$ 2,026
Other long-term liabilities:			
NQDC Plan liability	28,119	—	28,119
Total liabilities	$ 30,145	$ —	$ 30,145

	Fair Value Measurements as of December 31, 2022		
	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:			
Other current assets:			
NQDC Plan assets	$ 2,654	$ —	$ 2,654
Other assets:			
NQDC Plan assets	19,867	—	19,867
Restricted investments (1)	2,429	—	2,429
Total other assets	22,296	—	22,296
Total assets	$ 24,950	$ —	$ 24,950
Liabilities:			
Current liabilities:			
NQDC Plan liability	$ 2,654	$ —	$ 2,654
Contingent consideration	—	15,925	15,925
Total current liabilities	2,654	15,925	18,579
Other long-term liabilities:			
NQDC Plan liability	19,867	—	19,867
Total other long-term liabilities	19,867	—	19,867
Total liabilities	$ 22,521	$ 15,925	$ 38,446

(1) The restricted investments as of December 31, 2023 and 2022 secure the Company's irrevocable standby letters of credit obtained in connection with certain commercial agreements.

There were no transfers between levels during the periods presented.

Liabilities measured at fair value using Level 3 inputs consisted of contingent consideration. The following tables represent a roll-forward of contingent consideration.

Contingent consideration as of December 31, 2022	$	15,925
Milestone payments to Ares Trading S.A. (Merck Serono)		(16,255)
Realized foreign exchange gain on settlement of contingent consideration		330
Contingent consideration as of December 31, 2023	$	—

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

(8) DERIVATIVE INSTRUMENTS AND HEDGING STRATEGIES

The Company's forward contracts designated as hedging instruments have maturities up to two years. The Company's forward contracts that are considered to be economic hedges that are not designated as hedging instruments have maturities up to three months.

The following table summarizes the aggregate notional amounts for the Company's derivatives outstanding as of the periods presented.

Forward Contracts	December 31, 2023	December 31, 2022
Derivatives designated as hedging instruments:		
Sell	$ 1,249,662	$ 808,635
Purchase	$ 198,408	$ 177,393
Derivatives not designated as hedging instruments:		
Sell	$ 350,269	$ 218,903
Purchase	$ 90,102	$ 6,785

The fair value carrying amounts of the Company's derivatives, as classified within the fair value hierarchy, were as follows:

Balance Sheet Location	December 31, 2023	December 31, 2022
Derivatives designated as hedging instruments:		
Asset Derivatives - Level 2 (1)		
Other current assets	$ 6,663	$ 19,464
Other assets	1,855	2,059
Subtotal	$ 8,518	$ 21,523
Liability Derivatives - Level 2 (1)		
Accounts payable and accrued liabilities	$ 30,005	$ 12,130
Other long-term liabilities	8,171	1,074
Subtotal	$ 38,176	$ 13,204
Derivatives not designated as hedging instruments:		
Asset Derivatives - Level 2 (1)		
Other current assets	$ 547	$ 1,472
Liability Derivatives - Level 2 (1)		
Accounts payable and accrued liabilities	$ 3,848	$ 471
Total Derivatives Assets	$ 9,065	$ 22,995
Total Derivatives Liabilities	$ 42,024	$ 13,675

(1) Refer to Note 1 to these Consolidated Financial Statements for additional information related to the Company's fair value measurements.

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

The following tables summarize the impact of gains and losses from the Company's derivatives on its Consolidated Statements of Operations for the periods presented.

	Years Ended December 31,	
	2023	2022
Derivatives Designated as Cash Flow Hedging Instruments	**Cash Flow Hedging Gains (Losses) Reclassified into Earnings**	**Cash Flow Hedging Gains (Losses) Reclassified into Earnings**
Net product revenues	$ (186)	$ 48,541
Operating expenses	$ 350	$ (11,917)
Derivatives Not Designated as Hedging Instruments	**Gains (Losses) Recognized in Earnings**	**Gains (Losses) Recognized in Earnings**
Operating expenses	$ (8,808)	$ 872

As of December 31, 2023, the Company expects to reclassify unrealized losses of $23.3 million from AOCI to earnings as the forecasted revenue and operating expense transactions occur over the next twelve months. For additional discussion of balances in AOCI see Note 11 to these Consolidated Financial Statements.

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

(9) LEASES

The following table presents the Company's ROU assets and lease liabilities for the periods presented.

Lease Classification	Classification	December 31, 2023	December 31, 2022
Assets:			
Operating	Other assets	$ 42,731	$ 34,935
Financing	Other assets	3,343	6,021
Total ROU assets		$ 46,074	$ 40,956
Liabilities:			
Current:			
Operating	Accounts payable and accrued liabilities	$ 8,640	$ 8,088
Financing	Accounts payable and accrued liabilities	139	2,287
Noncurrent:			
Operating	Other long-term liabilities	38,047	26,329
Financing	Other long-term liabilities	77	184
Total lease liabilities		$ 46,903	$ 36,888

Maturities of lease liabilities as of December 31, 2023 by fiscal year were as follows:

Maturity of Lease Liabilities	Operating	Financing	Total
2024	$ 11,217	$ 142	$ 11,359
2025	9,861	75	9,936
2026	7,966	6	7,972
2027	7,064	—	7,064
2028	6,508	—	6,508
Thereafter	15,811	—	15,811
Total lease payments	58,427	223	58,650
Less: Interest	(11,740)	(7)	(11,747)
Present value of lease liabilities	$ 46,687	$ 216	$ 46,903

Lease costs associated with payments under the Company's leases for the periods presented were as follows:

Lease Cost	Classification	Years Ended December 31, 2023	Years Ended December 31, 2022
Operating [1]	Operating expenses	$ 14,197	$ 13,669
Financing:			
Amortization	Operating expenses	3,360	2,858
Interest expense	Interest expense	2,648	163
Total lease costs		$ 20,205	$ 16,690

(1) Includes short-term leases and variable lease costs, both of which were not material in the periods presented.

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

The following table includes the weighted average remaining lease terms and the weighted average discount rate used to calculate the present value of the Company's lease liabilities:

	Years Ended December 31,	
Other Information	**2023**	**2022**
Weighted average remaining lease term (in years):		
Operating leases	6.8	5.2
Financing leases	1.6	1.7
Weighted average discount rate:		
Operating leases	5.9 %	5.1 %
Financing leases	3.1 %	5.4 %

As of December 31, 2023, no leases were expected to commence that would create significant rights and obligations for the Company.

	Years Ended December 31,	
Supplemental Cash Flow Information	**2023**	**2022**
Cash paid for amounts included in the measurement of lease liabilities:		
Cash used in operating activities:		
Operating leases	$ 9,980	$ 10,760
Financing leases	$ 51	$ 165
Cash used in financing activities:		
Financing leases	$ 2,286	$ 2,605
ROU assets obtained in exchange for lease obligations:		
Operating leases	$ 16,321	$ 5,252
Financing leases	$ 68	$ 878

(10) DEBT

Convertible Notes

As of December 31, 2023, the Company had outstanding fixed-rate notes with varying maturities for an undiscounted aggregate principal amount of $1.1 billion (collectively the Notes). The Notes are senior subordinated convertible obligations, and

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

interest is payable in arrears, semi-annually. The following table summarizes information regarding the Company's convertible debt:

	December 31,	
	2023	2022
0.599% senior subordinated convertible notes due in August 2024 (the 2024 Notes)	$ 495,000	$ 495,000
Unamortized discount net of deferred offering costs	(1,123)	(3,040)
2024 Notes, net [1]	493,877	491,960
1.250% senior subordinated convertible notes due in May 2027 (the 2027 Notes)	600,000	600,000
Unamortized discount net of deferred offering costs	(6,905)	(8,941)
2027 Notes, net	593,095	591,059
Total convertible debt, net	$ 1,086,972	$ 1,083,019
Fair value of fixed-rate convertible debt [2]:		
2024 Notes	$ 488,288	$ 526,230
2027 Notes	619,260	647,370
Total fair value of fixed-rate convertible debt	$ 1,107,548	$ 1,173,600

(1) As the 2024 Notes mature in August 2024, the outstanding principal of the 2024 Notes is classified as a current liability as of December 31, 2023.

(2) The fair value of the Company's fixed-rate convertible debt is based on open market trades and is classified as Level 1 in the fair value hierarchy. See Note 1 to these Consolidated Financial Statements for additional discussion of fair value measurements.

Interest expense on the Company's fixed-rate convertible debt consisted of the following:

	Years Ended December 31,		
	2023	2022	2021
Coupon interest expense	$ 10,465	$ 10,465	$ 10,465
Accretion of discount on convertible notes	3,359	3,349	3,339
Amortization of debt issuance costs	594	593	593
Total interest expense on convertible debt	$ 14,418	$ 14,407	$ 14,397

2024 Notes

In August 2017, the Company issued $495.0 million in aggregate principal amount of senior subordinated convertible notes with a maturity date of August 1, 2024. The 2024 Notes were issued to the public at 98% of face value and bear interest at the rate of 0.599% per annum. Interest is payable semi-annually in cash in arrears on February 1 and August 1 of each year, beginning February 1, 2018. The 2024 Notes are convertible, at the option of the holder into shares of the Company's common stock. The initial conversion rate for the 2024 Notes is 8.0212 shares per $1,000 principal amount of the 2024 Notes, which represents a conversion price of approximately $124.67 per share, subject to adjustment under certain conditions. Following certain corporate transactions, the Company will, in certain circumstances, increase the conversion rate for a holder that elects to convert its 2024 Notes in connection with such corporate transactions by a number of additional shares of the Company's common stock. A holder may convert fewer than all of such holder's 2024 Notes so long as the amount of the 2024 Notes converted is an integral multiple of $1,000 principal amount. Net proceeds from the offering were $481.7 million. In connection with the issuance of the 2024 Notes, the Company recorded a discount on the 2024 Notes of $9.9 million, which will be accreted and recorded as additional interest expense over the life of the 2024 Notes.

The 2024 Notes are senior subordinated, unsecured obligations, and rank (i) subordinated in right of payment to the prior payment in full of any of the Company's existing and future senior debt, (ii) equal in right of payment to any of the Company's existing and future senior subordinated debt, (iii) senior in right of payment to any of the Company's existing and future indebtedness that is expressly subordinated in right of payment to the 2024 Notes, and (iv) effectively subordinated to any of the Company's existing and future secured indebtedness, to the extent of the value of the collateral securing that indebtedness and

structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables, and (to the extent the Company is not a holder thereof) preferred equity, if any, of the Company's subsidiaries. Upon the occurrence of a "fundamental change," as defined in the indenture governing the 2024 Notes, the holders may require the Company to repurchase all or a portion of such holder's 2024 Notes for cash at 100% of the principal amount of the 2024 Notes being purchased, plus any accrued and unpaid interest.

2027 Notes

In May 2020, the Company issued $600.0 million in aggregate principal amount of senior subordinated unsecured convertible notes with a maturity date of May 15, 2027. The 2027 Notes were issued to the public at par value and bear interest at the rate of 1.25% per annum. Interest is payable semi-annually in cash in arrears on May 15 and November 15 of each year, beginning November 15, 2020. The 2027 Notes are convertible, at the option of the holder into shares of the Company's common stock. The initial conversion rate for the 2027 Notes is 7.2743 shares per $1,000 principal amount of the 2027 Notes, which represents a conversion price of approximately $137.47 per share, subject to adjustment under certain conditions. Following certain corporate transactions, the Company will, in certain circumstances, increase the conversion rate for a holder that elects to convert its 2027 Notes in connection with such corporate transactions by a number of additional shares of the Company's common stock. A holder may convert fewer than all of such holder's 2027 Notes so long as the amount of the 2027 Notes converted is an integral multiple of $1,000 principal amount. Net proceeds from the offering were $585.8 million. In connection with the issuance of the 2027 Notes, the Company recorded a discount on the 2027 Notes of $13.5 million, which will be accreted and recorded as additional interest expense over the life of the 2027 Notes.

The 2027 Notes are senior subordinated, unsecured obligations, and rank (i) subordinated in right of payment to the prior payment in full of all of the Company's existing and future senior debt, (ii) equal in right of payment with the Company's existing and future senior subordinated debt, (iii) senior in right of payment to the Company's existing and future indebtedness that is expressly subordinated in right of payment to the notes, (vi) effectively subordinated to the Company's existing and future secured indebtedness, to the extent of the value of the collateral securing that indebtedness, and (v) structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables, and (to the extent the Company is not a holder thereof) preferred equity, if any, of the Company's subsidiaries. Upon the occurrence of a "fundamental change," as defined in the indenture governing the 2027 Notes, the holders may require the Company to repurchase all or a portion of such holder's 2027 Notes for cash at 100% of the principal amount of the 2027 Notes being purchased, plus any accrued and unpaid interest.

The offer and sale of the 2027 Notes and the shares of the Company's common stock issuable upon conversion of the 2027 Notes have not been registered under the Securities Act or any state securities laws and the 2027 Notes were offered only to qualified institutional buyers as defined in Rule 144A under the Securities Act.

See Note 16 to these Consolidated Financial Statements for further discussion of the effect of conversion of the Company's convertible debt on earnings (loss) per common share.

Revolving Credit Facility

In October 2018, the Company entered into an unsecured revolving credit facility of up to $200.0 million, which included a letter of credit subfacility and a swingline loan subfacility. The credit facility was intended to finance ongoing working capital needs and for other general corporate purposes. In May 2021, the credit facility was amended to extend the original maturity date from October 19, 2021 to May 28, 2024. The credit facility was terminated on August 4, 2023 and there were no amounts outstanding under the credit facility as of December 31, 2023.

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

(11) ACCUMULATED OTHER COMPREHENSIVE INCOME (LOSS)

The following table summarizes changes in the accumulated balances for each component of AOCI, including current period other comprehensive income (loss) and reclassifications out of AOCI, for the periods presented.

	Unrealized Gains (Losses) on Cash Flow Hedges	Unrealized Gains (Losses) on Available-for-Sale Debt Securities	Total
AOCI balance at December 31, 2020	$ (20,028)	$ 3,889	$ (16,139)
Other comprehensive income (loss) before reclassifications	34,379	(6,858)	27,521
Less: gain (loss) reclassified from AOCI	(1,454)	—	(1,454)
Tax effect	—	1,596	1,596
Net current period other comprehensive income (loss)	35,833	(5,262)	30,571
AOCI balance at December 31, 2021	$ 15,805	$ (1,373)	$ 14,432
Other comprehensive income (loss) before reclassifications	29,045	(13,967)	15,078
Less: gain (loss) reclassified from AOCI	36,624	—	36,624
Tax effect	—	3,247	3,247
Net current period other comprehensive income (loss)	(7,579)	(10,720)	(18,299)
AOCI balance at December 31, 2022	$ 8,226	$ (12,093)	$ (3,867)
Other comprehensive income (loss) before reclassifications	(37,720)	16,885	(20,835)
Less: gain (loss) reclassified from AOCI	164	—	164
Tax effect	—	(3,922)	(3,922)
Net current period other comprehensive income (loss)	(37,884)	12,963	(24,921)
AOCI balance at December 31, 2023	$ (29,658)	$ 870	$ (28,788)

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

(12) REVENUE, CREDIT CONCENTRATIONS AND GEOGRAPHIC INFORMATION

The following table presents Total Revenues and disaggregates Net Product Revenues by product.

	Years Ended December 31,		
	2023	2022	2021
Enzyme product revenues:			
VIMIZIM	$ 701,053	$ 663,739	$ 623,145
NAGLAZYME	420,292	443,794	380,449
PALYNZIQ	303,919	255,032	237,474
BRINEURA	161,889	154,333	128,034
ALDURAZYME	131,248	128,422	122,765
Total enzyme product revenues	1,718,401	1,645,320	1,491,867
VOXZOGO	469,881	169,128	5,855
KUVAN	180,767	227,577	285,776
ROCTAVIAN	3,489	—	—
Total net product revenues	2,372,538	2,042,025	1,783,498
Royalty and other revenues	46,688	54,014	62,777
Total revenues	$ 2,419,226	$ 2,096,039	$ 1,846,275

The Company considers there to be revenue concentration risks for regions where Net Product Revenues exceed 10% of consolidated Net Product Revenues. The concentration of the Company's Net Product Revenues within the regions below may have a material adverse effect on the Company's revenues and results of operations if sales in the respective regions experience difficulties. The table below disaggregates total Net Product Revenues by geographic region, which is based on patient location for Company's commercial products sold directly by the Company, except for ALDURAZYME, which is distributed, marketed and sold exclusively by Sanofi worldwide.

	Years Ended December 31,		
	2023	2022	2021
United States	$ 771,314	$ 684,284	$ 657,700
Europe	669,331	650,952	558,952
Latin America	332,437	266,801	191,151
Rest of world	468,208	311,566	252,930
Total net product revenues marketed by the Company	2,241,290	1,913,603	1,660,733
ALDURAZYME net product revenues marketed by Sanofi	131,248	128,422	122,765
Total net product revenues	$ 2,372,538	$ 2,042,025	$ 1,783,498

The following table illustrates the percentage of the Company's total Net Product Revenues attributed to the Company's largest customers for the periods presented.

	Years Ended December 31,		
	2023	2022	2021
Customer A	14 %	16 %	18 %
Customer B	12 %	12 %	14 %
Customer C	10 %	9 %	10 %
Total	36 %	37 %	42 %

On a consolidated basis, two customers accounted for 15% and 12% of the Company's December 31, 2023 accounts receivable balance, respectively, compared to December 31, 2022 when two customers accounted for 22% and 15% of the

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

accounts receivable balance, respectively. As of December 31, 2023 and 2022, the accounts receivable balance for Sanofi included $63.4 million and $68.8 million, respectively, of unbilled accounts receivable, which becomes payable to the Company when the product is sold through by Sanofi. The Company does not require collateral from its customers, but does perform periodic credit evaluations of its customers' financial condition and requires prepayments in certain circumstances.

The Company is mindful that conditions in the current macroeconomic environment, such as inflation, changes in interest and foreign currency exchange rates, natural disasters and supply chain disruptions, could affect the Company's ability to achieve its goals. In addition, the Company sells its products in countries that face economic volatility and weakness. Although the Company has historically collected receivables from customers in certain countries, sustained weakness or further deterioration of the local economies and currencies may cause customers in those countries to delay payment or be unable to pay for the Company's products. The Company believes that the allowances for doubtful accounts related to these countries, if any, are adequate based on its analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries. The Company will continue to monitor these conditions and will attempt to adjust its business processes, as appropriate, to mitigate macroeconomic risks to its business.

Long-lived assets, which consist of net property, plant and equipment and ROU assets are summarized by geographic region in the following table.

	December 31,	
	2023	2022
Long-lived assets by geography:		
United States	$ 788,590	$ 786,509
Ireland	306,542	309,180
Rest of world	17,075	18,633
Total long-lived assets	$ 1,112,207	$ 1,114,322

(13) EQUITY COMPENSATION PLANS AND STOCK-BASED COMPENSATION

Equity Compensation Plans

Shares Available Under Equity Compensation Plans

As of December 31, 2023, an aggregate of approximately 52.3 million unissued shares were authorized for future issuance under the Company's stock plans, which primarily includes shares issuable under the 2017 Equity Incentive Plan (2017 EIP) and the ESPP. Under the 2017 EIP, shares issued and outstanding under the Amended and Restated 2006 Share Incentive Plan (the 2006 Share Incentive Plan) and the 2017 EIP that expire or are forfeited generally become available for future issuance under the 2017 EIP. No additional awards will be granted under the 2006 Share Incentive Plan; however, there are vested and unvested awards outstanding under the 2006 Share Incentive Plan. The Company's stock-based compensation plans are administered by the Company's Board of Directors (the Board), or designated Committee thereof, which selects persons to receive awards and determines the number of shares subject to each award and the terms, conditions, performance measures and other provisions of the awards. See Note 1 to these Consolidated Financial Statements for discussion regarding the valuation of equity awards.

2017 Equity Incentive Plan

The 2017 EIP provides for awards of RSUs and stock options as well as other forms of equity compensation. RSUs granted to employees generally vest annually over a straight-line four-year period after the grant date. RSUs with Performance-based Vesting Conditions (PRSUs) generally vest over a three-year period on a cliff basis three years after the grant date. Stock option awards granted to employees generally vest over a four-year period on a cliff basis 12 months after the grant date and then

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monthly thereafter. The contractual term of stock option awards is generally 10 years from the grant date. As of December 31, 2023, approximately 39.5 million shares were authorized and reserved for future issuance under the 2017 EIP.

Employee Stock Purchase Plan

The ESPP was initially approved in June 2006, replacing the Company's previous plan, and was most recently amended in June 2019. Under BioMarin's ESPP, employees meeting specific employment qualifications are eligible to participate and can purchase shares on established dates (each purchase date) semi-annually through payroll deductions at the lower of 85% of the fair market value of the stock at the commencement of the offering period or each purchase date of the offering period. Each offering period will span up to two years. The ESPP permits eligible employees to purchase common stock through payroll deductions for up to 10% of qualified compensation, up to an annual limit of $25,000. The ESPP is intended to qualify as an "employee stock purchase plan" under Section 423 of the Internal Revenue Code. During the year ended December 31, 2023, the Company issued 0.3 million shares under the ESPP. As of December 31, 2023, approximately 7.0 million shares were authorized and 2.5 million shares reserved for future issuance under the ESPP.

Board of Director Grants

On the date of the Company's annual meeting of stockholders for a given year, each re-elected Independent Director receives an RSU grant valued at $400,000, with the number of RSUs to be granted calculated based on the thirty-day trailing average closing price of the Company's common stock on the Nasdaq Global Select Market. The annual RSU grant for a director who has served for less than a year is prorated to the nearest quarter of the calendar year. The RSUs subject to the annual award vest in full on the one-year anniversary of the grant date, subject to each respective Director providing service to the Company through such vesting date. Upon election or appointment, a new Independent Director will receive an RSU grant on the same terms as the annual award, pro-rated for amount and vesting to the nearest quarter for the time such new Independent Director will serve prior to the Company's next annual meeting of stockholders.

Stock-based Compensation

Stock-based compensation expense included on the Company's Consolidated Statements of Operations for all stock-based compensation arrangements was as follows:

	Years Ended December 31,		
	2023	2022	2021
Cost of sales	$ 17,604	$ 17,709	$ 22,357
Research and development	65,714	61,702	67,196
Selling, general and administrative	123,781	116,897	107,710
Total stock-based compensation expense	$ 207,099	$ 196,308	$ 197,263

Stock-based compensation of $21.7 million, $21.3 million and $20.0 million was capitalized into inventory for the years ended December 31, 2023, 2022 and 2021, respectively. Capitalized stock-based compensation is recognized in Cost of Sales when the related product is sold.

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

Restricted Stock Units

Restricted Stock Unit Awards with Service-Based Vesting Conditions

Below is a summary of activity related to RSUs with service-based vesting conditions for the year ended December 31, 2023:

	Shares	Weighted Average Grant Date Fair Value
Non-vested units as of December 31, 2022	4,519,746	$ 79.55
Granted	2,150,561	$ 88.96
Vested	(1,666,477)	$ 80.68
Forfeited	(300,005)	$ 81.35
Non-vested units as of December 31, 2023	4,703,825	$ 83.39

The weighted-average grant date fair values per share of RSUs with service-based vesting granted during the years ended December 31, 2023, 2022 and 2021, was $88.96, $79.43 and $78.46, respectively. The total intrinsic values of restricted stock that vested and released in the years ended December 31, 2023, 2022 and 2021, was $149.8 million, $130.1 million and $117.2 million respectively.

As of December 31, 2023, total unrecognized compensation cost related to unvested RSUs with service-based vesting conditions of $274.7 million was expected to be recognized over a weighted average period of 2.6 years.

Restricted Stock Unit Awards with Performance-based Vesting Conditions

Below is a summary of activity related to RSUs with vesting conditions based on performance targets for the year ended December 31, 2023:

	Shares	Weighted Average Grant Date Fair Value
Non-vested units as of December 31, 2022	447,662	$ 78.78
Granted	230,019	$ 89.22
Vested	(231,346)	$ 78.30
Forfeited	(10,235)	$ 78.36
Non-vested units as of December 31, 2023	436,100	$ 83.33

The weighted-average grant date fair value of the PRSUs for the years ended December 31, 2023, 2022 and 2021, was $89.22, $78.27 and $78.09, respectively.

Non-vested PRSUs included grants with vesting contingent upon the achievement of three-year performance targets for strategic goals, Non-GAAP income or other internal financial measures and a regulatory milestone. The awarded PRSUs generally vest over a three-year service period on a cliff basis. The Company evaluated the targets in the context of its current long-range financial plan, its product candidate development pipeline and planned regulatory activity to determine when attainment of each grant target was probable for accounting purposes. The number of shares that may be earned range between 50% and 200% of the base PRSUs granted.

As of December 31, 2023, total unrecognized compensation expense related to non-vested PRSUs of $10.9 million was expected to be recognized over a weighted average period of 1.3 years.

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

Restricted Stock Unit Awards with Market-based Vesting Conditions

The Compensation Committee and Board may grant RSUs with market-based vesting conditions (base TSR-RSUs) to certain executives. These base TSR-RSUs vest, if at all, in full following a three-year service period only if certain total shareholder return (TSR) results relative to the Nasdaq Biotechnology Index comparative companies are achieved. The number of shares that may be earned range between zero percent and 200% of the base TSR-RSUs with a ceiling achievement level of 100% of the base TSR-RSUs in the event the Company's TSR is above the 50th percentile but negative on an absolute basis.

Below is a summary of activity related to RSUs with market-based vesting conditions for the year ended December 31, 2023:

	Shares	Weighted Average Grant Date Fair Value
Non-vested units as of December 31, 2022	396,120	$ 119.61
Granted	261,570	$ 132.56
Vested	(248,940)	$ 116.05
Forfeited	(4,850)	$ 121.04
Non-vested units as of December 31, 2023	403,900	$ 128.95

The grant date fair values and assumptions used to determine the fair value of TSR-RSUs on grant date during the periods presented were as follows:

	Years Ended December 31,		
	2023	2022	2021
Grant date fair value	$132.56	124.67	$117.52
Expected volatility	22.4 – 152.1%	24.5 – 157.6%	24.7 – 161.7%
Dividend yield	0.0%	0.0%	0.0%
Expected term	2.8 years	2.8 years	2.8 years
Risk-free interest rate	3.8%	2.0%	0.3%

As of December 31, 2023, total unrecognized compensation expense of $18.0 million related to base TSR-RSUs was expected to be recognized over a weighted average period of 1.4 years.

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

Stock Options and Purchase Rights

Stock Options

The following table summarizes activity under the Company's stock option plans for the year ended December 31, 2023. All stock option grants presented in the table had exercise prices not less than the fair value of the underlying common stock on the grant date:

	Shares	Weighted Average Exercise Price	Weighted Average Remaining Years	Aggregate Intrinsic Value [1]
Options outstanding as of December 31, 2022	5,943,535	$ 82.41		$ 132,395
Granted	704,589	$ 89.47		
Exercised	(837,623)	$ 68.82		
Expired and forfeited	(90,370)	$ 82.69		
Options outstanding as of December 31, 2023	5,720,131	$ 85.26	5.0	$ 74,704
Options unvested as of December 31, 2023	1,260,219	$ 84.63	8.7	$ 15,139
Exercisable at December 31, 2023	4,459,912	$ 85.44	4.0	$ 59,566

(1) The aggregate intrinsic value for outstanding options is calculated as the difference between the exercise price of the underlying awards and the quoted price of the Company's common stock on the Nasdaq Global Select Market as of the last trading day for the respective year. The aggregate intrinsic value of options outstanding and exercisable includes options with an exercise price below $96.42, the closing price of the Company's common stock on the Nasdaq Global Select Market on December 29, 2023.

The weighted-average fair values per stock options granted in the years ended December 31, 2023, 2022 and 2021, were $39.30, $32.45 and $31.61, respectively. The total intrinsic values of options exercised during the years ended December 31, 2023, 2022 and 2021, were $25.9 million, $32.1 million and $40.7 million, respectively, determined as of the date of option exercise. Upon the exercise of the options, the Company issues new common stock from its authorized shares.

The assumptions used to estimate the per share fair value of stock options granted during the periods presented were as follows:

	Years Ended December 31,		
	2023	2022	2021
Expected volatility	37.8 – 40.3%	38.1 – 40.5%	39.4 – 41.6%
Dividend yield	0.0%	0.0%	0.0%
Expected term	4.7 – 6.2 years	4.7 – 6.1 years	4.7 – 6.0 years
Risk-free interest rate	3.5 – 4.6%	2.1 – 4.2%	0.7 – 1.3%

As of December 31, 2023, total unrecognized compensation cost related to unvested stock options of $36.5 million was expected to be recognized over a weighted average period of 2.7 years. The net tax benefit from stock options exercised during the year ended December 31, 2023 was $2.3 million.

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

Stock Purchase Rights

The assumptions used to estimate the per share fair value of stock purchase rights granted under the ESPP were as follows:

	Years Ended December 31,		
	2023	2022	2021
Expected volatility	24.0 – 48.0%	28.6 – 69.2%	23.7 – 69.2%
Dividend yield	0.0%	0.0%	0.0%
Expected term	0.5 – 2.0 years	0.5 – 2.0 years	0.5 – 2.0 years
Risk-free interest rate	0.06 – 5.5%	0.04 – 4.8%	0.04 – 2.4%

As of December 31, 2023, total unrecognized compensation cost related to unvested stock purchase rights under the ESPP of $14.2 million was expected to be recognized over a weighted average period of 1.4 years.

(14) OTHER EMPLOYEE BENEFITS

401(k) Plan

The Company sponsors the BioMarin Retirement Savings Plan (the 401(k) Plan) for eligible U.S. employees. The Company pays the direct expenses of the 401(k) Plan and matches 100% of each participating employee's eligible contributions, up to a maximum of the lesser of 6% of the employee's annual compensation or the annual statutory contribution limit. The Company's matching contribution vests immediately and was approximately $32.7 million, $30.8 million and $31.6 million for the years ended December 31, 2023, 2022 and 2021, respectively.

Deferred Compensation Plan

The Company maintains the NQDC under which eligible directors and key employees may defer compensation. The NQDC prohibits the diversification of deferrals of Company stock. Company stock issued and held by the NQDC is accounted for similarly to treasury stock in that the fair value of the employer stock was determined on the grant date and the shares are issued into the NQDC when the restricted stock vests. The corresponding deferred compensation obligation is classified as equity with no changes in the fair value of Company stock held in the NQDC recognized in earnings. Other contributions held in the NQDC are classified as trading securities, recorded at fair value with the corresponding deferred compensation obligation classified as a liability and subsequent changes in the fair value of these non-BioMarin investments are recognized in earnings in the period they occur.

See Note 7 to these Consolidated Financial Statements for additional discussion on the fair value and presentation of the NQDC assets and liabilities.

(15) INCOME TAXES

The Provision for (Benefit from) Income Taxes was based on Income (Loss) before Income Taxes as follows:

	Years Ended December 31,		
	2023	2022	2021
U.S. Source	$ (453,840)	$ (299,403)	$ (259,258)
Non-U.S. Source	642,403	448,979	183,908
Income (loss) before income taxes	$ 188,563	$ 149,576	$ (75,350)

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

The U.S. and foreign components of the Provision for (Benefit from) Income Taxes were as follows:

	Years Ended December 31,		
	2023	2022	2021
Provision for (benefit from) income taxes			
Federal	$ 25,120	$ 12,798	$ (2,038)
State and local	5,098	5,058	1,339
Foreign	35,681	42,246	5,037
	65,899	60,102	4,338
Provision for (benefit from) deferred income taxes:			
Federal	(70,754)	(79,270)	(29,895)
State and local	(8,030)	(5,143)	(1,230)
Foreign	33,803	32,326	15,517
	(44,981)	(52,087)	(15,608)
Provision for (benefit from) income taxes	$ 20,918	$ 8,015	$ (11,270)

The following is a reconciliation of the statutory federal income tax benefit to the Company's effective tax rate:

	December 31,		
	2023	2022	2021
Federal statutory income tax rate	$ 39,598	$ 31,412	$ (15,824)
State and local taxes	(3,614)	(1,017)	509
Orphan Drug & General Business Credit	(39,535)	(35,674)	(29,363)
Stock compensation expense	2,209	6,433	7,859
Foreign Source Income Subject to US Tax	47,721	(5,644)	16,878
Foreign tax rate differential [(1)]	(69,987)	(4,051)	(16,971)
Section 162(m) limitation	9,699	6,577	6,304
Tax Reserves	27,296	18,043	15,530
Intra-entity transfer of assets	5,019	(18,752)	(3,920)
Valuation allowance/deferred benefit	3,723	7,851	6,821
Other	(1,211)	2,837	907
Effective income tax rate	$ 20,918	$ 8,015	$ (11,270)

(1) For the year ended December 31, 2023, the foreign rate differential included foreign local tax expense which was at an effective rate lower than the U.S. statutory rate. For the year ended December 31, 2022, the foreign rate differential included foreign local tax expense which was at an effective rate lower than the U.S. statutory rate and was offset by the income from the sale of a priority review voucher that was taxed at a higher tax rate. For the year ended December 31, 2021, the foreign rate differential included foreign local tax expense which was at an effective rate lower than the U.S. statutory rate and includes the recognition of the valuation allowance against a portion of the deferred tax assets of the Company's Dutch subsidiary of $9.3 million.

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

The significant components of the Company's net deferred tax assets were as follows:

	December 31,	
	2023	2022
Net deferred tax assets:		
Net operating loss carryforwards	$ 19,025	$ 20,657
Tax credit carryforwards	524,652	555,319
Accrued expenses, reserves, and prepaids	107,485	88,697
Intangible assets	789,479	836,402
Capitalized research and development expenses	216,975	103,212
Stock-based compensation	48,744	49,472
Lease liabilities	7,857	5,757
Inventory	18,914	22,726
Other	1,113	5,596
Valuation allowance	(119,230)	(116,299)
Total deferred tax assets	1,615,014	1,571,539
Joint venture basis difference	(1,111)	(745)
Acquired intangibles	(1,026)	(1,138)
ROU Assets	(6,917)	(5,347)
Property, plant and equipment	(60,151)	(58,897)
Total deferred tax liabilities	(69,205)	(66,127)
Net deferred tax assets	$ 1,545,809	$ 1,505,412

The increase in net deferred tax assets is primarily related to capitalization of research and development expenses offset by a decrease in intangible assets and tax credits utilized.

Valuation allowances are provided to reduce the amounts of the Company's deferred tax assets to an amount that is more likely than not to be realized based on an assessment of positive and negative evidence, including estimates of future taxable income necessary to realize future deductible amounts. At the end of each period, the Company will reassess the ability to realize its deferred tax benefits. If it is more likely than not that the Company would not realize the deferred tax benefits, a valuation allowance may need to be established against all or a portion of the deferred tax assets, which will result in a charge to tax expense.

In the third quarter of 2023, the Company determined that it is more likely than not that the deferred tax assets related to a future royalty stream will be realized. In making this determination, the Company analyzed both the consistent historical royalty earnings and the forecast of future royalty earnings and reached the conclusion that it was appropriate to release the valuation allowance reserve. The release is offset by an increase due to the Company's expectation that state R&D credits generated will not be utilized.

As of December 31, 2023, the Company had the following net operating loss and tax credit carryforwards, which if not utilized, will expire as follows:

Type	Amount	Year
Federal net operating loss carryforwards	$ 3,296	2030-2033
Federal R&D and orphan drug credit carryforwards	$ 555,074	2024-2043
State net operating loss carryforwards	$ 208,592	2024-2043
Dutch net operating loss carryforwards	$ 31,361	Indefinite

Not included in the table above are $169.6 million of state research credit carryovers that will carry forward indefinitely.

The Company's net operating losses and credits could be subject to annual limitations due to ownership change limitations provided by Internal Revenue Code Section 382 and similar state provisions. An annual limitation could result in the expiration of net operating losses and tax credit carryforward before utilization. There are limitations on the tax attributes of acquired entities however, the Company does not believe the limitations will have a material impact on the utilization of the net operating losses or tax credits.

(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

The financial statement recognition of the benefit for a tax position is dependent upon the benefit being more likely than not to be sustainable upon audit by the applicable taxing authority. If this threshold is met, the tax benefit is then measured and recognized at the largest amount that is greater than 50% likely of being realized upon ultimate settlement.

A reconciliation of the beginning and ending amount of unrecognized tax benefits for the years ended December 31, 2023 and 2022, is as follows:

	December 31,	
	2023	2022
Balance at beginning of period	$ 232,856	$ 205,095
Additions based on tax positions related to the current year	41,473	26,762
Additions for tax positions of prior years	3,127	1,017
Lapse of statute of limitations	—	(18)
Balance at end of period	$ 277,456	$ 232,856

Included in the balance of unrecognized tax benefits as of December 31, 2023 were potential benefits of $266.5 million that, if recognized, would affect the effective tax rate. The Company's policy for classifying interest and penalties associated with unrecognized income tax benefits is to include such items in the income tax expense. The total amount of accrued interest and penalties was not significant as of December 31, 2023. The Company believes it will not have any material decreases in its previously unrecognized tax benefits within the next twelve months.

The Company files income tax returns in the U.S., Ireland and various foreign jurisdictions. The U.S. and foreign jurisdictions have statute of limitations ranging from three to five years. However, carryforward tax attributes that were generated in 2020 and earlier may still be adjusted upon examination by tax authorities.

U.S. income and foreign withholding taxes have not been recognized on the excess of the amount for financial reporting over the tax basis of investments in foreign subsidiaries that are essentially permanent in duration. This excess totaled approximately $15.1 million as of December 31, 2023, which will be indefinitely reinvested; deferred income taxes have not been provided on such foreign earnings.

(16) EARNINGS (LOSS) PER COMMON SHARE

Potentially issuable shares of common stock include shares issuable upon the exercise of outstanding employee stock option awards, common stock issuable under the Company’s ESPP, unvested RSUs and contingent issuances of common stock related to the Company's convertible debt.

The following table sets forth the computation of basic and diluted earnings (loss) per common share (common shares in thousands):

	Twelve Months Ended December 31,		
	2023	2022	2021
Numerator:			
Net income (loss)	$ 167,645	$ 141,561	$ (64,080)
Denominator:			
Weighted-average common shares outstanding, basic	187,834	185,266	182,852
Effect of dilutive securities:			
Common stock issuable under the Company's equity incentive plans	3,761	3,697	—
Weighted-average common shares outstanding, diluted	191,595	188,963	182,852
Earnings (loss) per common share, basic	$ 0.89	$ 0.76	$ (0.35)
Earnings (loss) per common share, diluted	$ 0.87	$ 0.75	$ (0.35)

In addition to the equity instruments included in the table above, the table below presents potential shares of common stock that were excluded from the computation of diluted earnings (loss) per common share as they were anti-dilutive (in thousands):

	Twelve Months Ended December 31,		
	2023	2022	2021
Common stock issuable under the Company's equity incentive plans	8,072	8,148	12,450
Common stock issuable under the Company's convertible debt [(1)]	8,335	8,335	8,335
Total number of potentially issuable shares	16,407	16,483	20,785

(1) If converted, the Company would issue 4.0 million shares under the 2024 Notes and 4.4 million shares under the 2027 Notes. For additional discussion of our convertible debt, see Note 10 to these Consolidated Financial Statements.

(17) LICENSE AND COLLABORATION AGREEMENTS

In October 2019, the Company entered into a worldwide, exclusive licensing agreement with a third party for tralesinidase alfa (formerly referred to as BMN 250), an investigational enzyme replacement therapy to treat Sanfilippo Syndrome Type B. In consideration, the Company received an upfront payment of $3.0 million, a minority 15% equity ownership interest in the licensee, and is entitled to receive royalties on net sales of tralesinidase alfa and milestone payments if certain development, regulatory and sales milestones are met by the licensee. Subsequently, the third party licensee raised additional funding and issued the Company incremental shares to maintain its 15% minority interest. As of December 31, 2022, the balance of the equity investment included in Other Assets on the Company's Consolidated Balance Sheets was $12.6 million, which was fully impaired in 2023 based on new developments that lead the Company to conclude that factors existed indicating this non-marketable strategic investment was no longer realizable. The loss on the equity investment due to impairment was recorded to Other Income (Expense), Net on the Company's Consolidated Statements of Operations.

In July 2017, the Company executed a license agreement with Sarepta Therapeutics (Sarepta) that provides Sarepta with global exclusive rights to the Company's Duchenne muscular dystrophy (DMD) patent estate for EXONDYS 51 and all future exon-skipping products. Under the license agreement, Sarepta pays the Company royalties and may pay the Company certain milestone payments for exons 51, 45, 53 and possibly other exon-skipping products. In 2021, the Company and Sarepta amended the license agreement to, among other things, make the license co-exclusive at a future date and reduce future royalty rates.

On October 1, 2015, the Company entered into an agreement with Ares Trading S.A. (Merck Serono) under which the Company acquired all global rights to KUVAN and PALYNZIQ from Merck Serono, with the exception of KUVAN in Japan. Previously, the Company had exclusive rights to KUVAN in the U.S. and Canada and PALYNZIQ in the U.S. and Japan. Pursuant to the amended and restated KUVAN Agreement, if future sales milestones were met, the Company was obligated to pay Merck Serono up to a maximum of €60.0 million, all of which were met and paid as of December 31, 2023. Pursuant to the Pegvaliase

Agreement, the Company also paid Merck Serono €125.0 million in cash when the PALYNZIQ development milestones were achieved.

In October 2012, the Company licensed to Catalyst Pharmaceutical Partners, Inc. (Catalyst) the North American rights to develop and market FIRDAPSE, the Company's former commercial product for the treatment of Lambert-Eaton myasthenic syndrome. In exchange for the North American rights to FIRDAPSE, commencing in the first quarter of 2019 the Company receives royalties of 7% to 10% on net product sales of FIRDAPSE in North America.

The Company is engaged in R&D collaborations with various other entities. These provide for sponsorship of R&D by the Company and may also provide for exclusive royalty-bearing intellectual property licenses or rights of first negotiation regarding licenses to intellectual property development under the collaborations. Typically, these agreements can be terminated for cause by either party upon written notice.

In 2020, the Company entered into a research and collaboration agreement with a third party and received a convertible note, which was recorded in Other Assets on the Company's Consolidated Balance Sheets. In 2023, the Company recorded a $11.9 million impairment loss on the convertible note as it was deemed unrecoverable based on new information. The impairment loss was recorded to Other Income (Expense), Net on the Company's Consolidated Statements of Operations.

(18) COMMITMENTS AND CONTINGENCIES

Contingencies

From time to time the Company is involved in legal actions arising in the normal course of its business. The process of resolving matters through litigation or other means is inherently uncertain and it is possible that an unfavorable resolution of these matters could adversely affect the Company, its results of operations, financial condition or cash flows. The Company’s general practice is to expense legal fees as services are rendered in connection with legal matters, and to accrue for liabilities when losses are probable and reasonably estimable based on existing information. The Company accrues for the best estimate of a loss within a range; however, if no estimate in the range is better than any other, then the minimum amount in the range is accrued. Liabilities are evaluated and refined each reporting period as additional information is known. Any receivables for insurance recoveries for these liability claims are recorded as assets when it is probable that a recovery will be realized.

The Company was involved in a purported shareholder class action lawsuit filed against the Company and certain officers and directors alleging violations under Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 as amended (the Exchange Act) for making materially false or misleading statements regarding the clinical trials and Biologics License Application (BLA) for ROCTAVIAN (formerly known as valoctocogene roxaparvovec) by purportedly failing to disclose that differences between the Company’s Phase 1/2 and Phase 3 clinical studies limited the ability of the Phase 1/2 study to support ROCTAVIAN’s durability of effect and, as a result, that it was foreseeable that the FDA would not approve the BLA without additional data. On March 21, 2023, the Court entered an order staying all proceedings and vacating all deadlines because the parties agreed to settle the case through a binding term sheet. The Court preliminarily approved the settlement on June 8, 2023. On November 14, 2023, the court granted final approval of the settlement and entered final judgment. The Company maintains directors and officers liability insurance that covers exposure related to this class action lawsuit. As of December 31, 2022, the Company had recorded an estimated long-term loss contingency of $13.0 million on the Company’s Consolidated Balance Sheets. The same amount was recorded for expected insurance recoveries. During 2023, the amount recorded on the Company's Consolidated Balance Sheets increased to $39.0 million based on the final settlement and was reclassified to short term. As of December 31, 2023, the $39.0 million settlement liability and related receivable have been released following the final judgment. There was a net zero impact on the Company's Consolidated Statements of Cash Flows and Consolidated Statement of Operations in the years presented.

The Company recently received a subpoena from the U.S. Department of Justice (DOJ) requesting that the Company produce certain documents regarding sponsored testing programs relating to VIMIZIM and NAGLAZYME. The Company has produced the requested documents in response to the subpoena and is cooperating fully. The Company is unable to make any assurances regarding the outcome of the investigation by the DOJ, or the impact, if any, that such investigation may have on the Company’s business, Consolidated Balance Sheets, Statements of Operations or Statements of Cash Flows.

Contingent Payments

As of December 31, 2023, the Company was subject to contingent payments, primarily comprised of development, regulatory and commercial milestones. Those considered reasonably possible totaled $763.3 million, of this amount the Company may pay up to $30.1 million in 2024 if certain contingencies are met. $591.5 million of the total balance related to early-stage development programs licensed from two third parties.

Other Commitments

The Company uses experts and laboratories at universities and other institutions to perform certain R&D activities. These amounts are included as R&D expense as services are provided. In the normal course of business, the Company enters into various firm purchase commitments primarily to procure active pharmaceutical ingredients, certain inventory-related items and certain third-party R&D services, production services and facility construction services. The Company also has commitments related to enterprise resource planning system implementation costs. As of December 31, 2023, such commitments were estimated at $354.1 million, of which $325.9 million is expected to be paid in 2024 as underlying goods and services are received. The Company has also licensed technology from third parties, for which it is required to pay royalties upon future sales, subject to certain annual minimums.

Exhibit 21.1

Subsidiaries of BioMarin Pharmaceutical Inc. as of December 31, 2023

Name	Direct Parent	Ownership	Jurisdiction of Incorporation
BioMarin Commercial Ltd	BioMarin Pharmaceutical Inc.	100%	Ireland
BioMarin International Ltd	BioMarin Commercial Ltd.	100%	Ireland

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the registration statements (No. 333-136963, 333-168552, 333-188620, 333-206094, 333-218695, 333-234231, 333-262824 and 333-275273) on Form S-8 of our reports dated February 26, 2024, with respect to the consolidated financial statements of BioMarin Pharmaceutical Inc. and the effectiveness of internal control over financial reporting.

/s/ KPMG LLP
San Francisco, California
February 26, 2024

Exhibit 31.1

CERTIFICATION

I, Alexander Hardy, certify that:

1. I have reviewed this Annual Report on Form 10-K of BioMarin Pharmaceutical Inc.;

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

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

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

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

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

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

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

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

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

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

Date: February 26, 2024

/S/ ALEXANDER HARDY

Alexander Hardy
Chief Executive Officer

Exhibit 31.2

CERTIFICATION

I, Brian R. Mueller certify that:

1. I have reviewed this Annual Report on Form 10-K of BioMarin Pharmaceutical Inc.;

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

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

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

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

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

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

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

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

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

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

Date: February 26, 2024

/S/ BRIAN R. MUELLER

Brian R. Mueller
Executive Vice President, Finance &
Chief Financial Officer

Exhibit 32.1

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

We, Alexander Hardy and Brian R. Mueller, hereby certify, pursuant to 18 U.S.C. §1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002, that BioMarin Pharmaceutical Inc.'s Annual Report on Form 10-K for the period ended December 31, 2023, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and the information contained in such Annual Report on Form 10-K fairly presents, in all material respects, the financial condition and results of operations of BioMarin Pharmaceutical Inc.

/s/ ALEXANDER HARDY

Alexander Hardy
Chief Executive Officer

Date: February 26, 2024

/s/ BRIAN R. MUELLER

Brian R. Mueller
Executive Vice President, Finance &
Chief Financial Officer

Date: February 26, 2024

This certification accompanies the Annual Report on Form 10-K to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of BioMarin Pharmaceutical Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Annual Report on Form 10-K), irrespective of any general incorporation language contained in such filing.

BIOMARIN PHARMACEUTICAL INC.

Dodd-Frank Incentive Compensation Recoupment Policy

(adopted October 4, 2023)

1. Introduction

The Board of Directors (the "***Board***") of BioMarin Pharmaceutical Inc., a Delaware corporation (the "***Company***"), has determined that it is in the best interests of the Company and its stockholders to adopt this Dodd-Frank Incentive Compensation Recoupment Policy (this "***Policy***") providing for the Company's recoupment of Recoverable Incentive Compensation that is received by Covered Officers of the Company under certain circumstances. Certain capitalized terms used in this Policy have the meanings given to such terms in Section 3 below.

This Policy is designed to comply with, and shall be interpreted to be consistent with, Section 10D of the Exchange Act, Rule 10D-1 promulgated thereunder ("***Rule 10D-1***") and Nasdaq Listing Rule 5608 (the "***Listing Standards***").

2. Effective Date

This Policy shall apply to all Incentive Compensation that is received by a Covered Officer on or after October 2, 2023 (the "***Effective Date***"). Incentive Compensation is deemed "***received***" in the Company's fiscal period in which the Financial Reporting Measure specified in the Incentive Compensation award is attained, even if the payment or grant of such Incentive Compensation occurs after the end of that period.

3. Definitions

"***Accounting Restatement***" means an accounting restatement that the Company is required to prepare due to the material noncompliance of the Company with any financial reporting requirement under the securities laws, including any required accounting restatement to correct an error in previously issued financial statements that is material to the previously issued financial statements, or that would result in a material misstatement if the error were corrected in the current period or left uncorrected in the current period.

"***Accounting Restatement Date***" means the earlier to occur of (a) the date that the Board, a committee of the Board authorized to take such action, or the officer or officers of the Company authorized to take such action if Board action is not required, concludes, or reasonably should have concluded, that the Company is required to prepare an Accounting Restatement, or (b) the date that a court, regulator or other legally authorized body directs the Company to prepare an Accounting Restatement.

"***Administrator***" means the Compensation Committee or, in the absence of such committee, the Board.

"***Code***" means the U.S. Internal Revenue Code of 1986, as amended, and the regulations promulgated thereunder.

"***Compensation Committee***" means the Compensation Committee of the Board.

"***Covered Officer***" means each current and former Executive Officer.

"***Exchange***" means the Nasdaq Stock Market.

"***Exchange Act***" means the U.S. Securities Exchange Act of 1934, as amended.

"***Executive Officer***" means the Company's president, principal financial officer, principal accounting officer (or if there is no such accounting officer, the controller), any vice-president of the Company in charge of a principal business unit, division, or function (such as sales, administration, or finance), any other officer who performs a policy-making function, or any other person who performs similar policy-making functions for the Company. Executive officers of the Company's parent(s) or subsidiaries are deemed executive officers of the Company if they perform such policy-making functions for the Company. Policy-making function is not intended to include policy-making functions that are not significant. Identification of an executive officer for purposes of this Policy would include at a minimum executive officers identified pursuant to Item 401(b) of Regulation S-K promulgated under the Exchange Act.

"***Financial Reporting Measures***" means measures that are determined and presented in accordance with the accounting principles used in preparing the Company's financial statements, and any measures derived wholly or in part from such measures, including Company stock price and total stockholder return ("***TSR***"). A measure need not be presented in the Company's financial statements or included in a filing with the SEC in order to be a Financial Reporting Measure.

"***Incentive Compensation***" means any compensation that is granted, earned or vested based wholly or in part upon the attainment of a Financial Reporting Measure.

"***Lookback Period***" means the three completed fiscal years immediately preceding the Accounting Restatement Date, as well as any transition period (resulting from a change in the Company's fiscal year) within or immediately following those three completed fiscal years (except that a transition period of at least nine months shall count as a completed fiscal year). Notwithstanding the foregoing, the Lookback Period shall not include fiscal years completed prior to the Effective Date.

"***Recoverable Incentive Compensation***" means Incentive Compensation received by a Covered Officer during the Lookback Period that exceeds the amount of Incentive Compensation that would have been received had such amount been determined based on the Accounting Restatement, computed without regard to any taxes paid (*i.e.*, on a gross basis without regard to tax withholdings and other deductions). For any compensation plans or programs that take into account Incentive Compensation, the amount of Recoverable Incentive Compensation for purposes of this Policy shall include, without limitation, the amount contributed to any notional account based on Recoverable Incentive Compensation and any earnings to date on that notional amount. For any Incentive Compensation that is based on stock price or TSR, where the Recoverable Incentive Compensation is not subject to mathematical recalculation directly from the information in an Accounting Restatement, the Administrator will determine the amount of Recoverable Incentive Compensation based on a reasonable estimate of the effect of the Accounting Restatement on the stock price or TSR upon which the Incentive Compensation was received. The Company shall maintain documentation of the determination of that reasonable estimate and provide such documentation to the Exchange in accordance with the Listing Standards.

"***SEC***" means the U.S. Securities and Exchange Commission.

4. Recoupment

(a) Applicability of Policy. This Policy applies to Incentive Compensation received by a Covered Officer (i) after beginning services as an Executive Officer, (ii) who served as an Executive Officer at any time during the performance period for such Incentive Compensation, (iii) while the Company had a class of securities listed on a national securities exchange or a national securities association, and (iv) during the Lookback Period.

(b) Recoupment Generally. Pursuant to the provisions of this Policy, if there is an Accounting Restatement, the Company must reasonably promptly recoup the full amount of the Recoverable Incentive Compensation, unless the conditions of one or more subsections of Section 4(c) of this Policy are met and the Compensation Committee, or, if such committee does not consist solely of independent directors, a majority of the independent directors serving on the Board, has made a determination that recoupment would be impracticable. Recoupment is required regardless of whether the Covered Officer engaged in any misconduct and regardless of fault, and the Company's obligation to recoup Recoverable Incentive Compensation is not dependent on whether or when any restated financial statements are filed.

(c) Impracticability of Recovery. Recoupment may be determined to be impracticable if, and only if:

(i) the direct expense paid to a third party to assist in enforcing this Policy would exceed the amount of the applicable Recoverable Incentive Compensation; provided that, before concluding that it would be impracticable to recover any amount of Recoverable Incentive Compensation based on expense of enforcement, the Company shall make a reasonable attempt to recover such Recoverable Incentive Compensation, document such reasonable attempt(s) to recover, and provide that documentation to the Exchange in accordance with the Listing Standards; or

(ii) recoupment of the applicable Recoverable Incentive Compensation would likely cause an otherwise tax-qualified retirement plan, under which benefits are broadly available to employees of the Company, to fail to meet the requirements of Code Section 401(a)(13) or Code Section 411(a) and regulations thereunder.

(d) Sources of Recoupment. To the extent permitted by applicable law, the Administrator shall, in its sole discretion, determine the timing and method for recouping Recoverable Incentive Compensation hereunder, provided that such recoupment is undertaken reasonably promptly. The Administrator may, in its discretion, seek recoupment from a Covered Officer from any of the following sources or a combination thereof, whether the applicable compensation was approved, awarded, granted, payable or paid to the Covered Officer prior to, on or after the Effective Date: (i) direct repayment of Recoverable Incentive Compensation previously paid to the Covered Officer; (ii) cancelling prior cash or equity-based awards (whether vested or unvested and whether paid or unpaid); (iii) cancelling or offsetting against any planned future cash or equity-based awards; (iv) forfeiture of deferred compensation, subject to compliance with Code Section 409A; and (v) any other method authorized by applicable law or contract. Subject to compliance with any applicable law, the Administrator may effectuate recoupment under this Policy from any amount otherwise payable to the Covered Officer, including amounts payable to such individual under any otherwise applicable Company plan or program, *e.g.*, base salary, bonuses or commissions and compensation previously deferred by the Covered Officer. The Administrator need not utilize the same method of recovery for all Covered Officers or with respect to all types of Recoverable Incentive Compensation.

(e) No Indemnification of Covered Officers. Notwithstanding any indemnification agreement, applicable insurance policy or any other agreement or provision of the Company's certificate of incorporation or bylaws to the contrary, no Covered Officer shall be entitled to indemnification or advancement of expenses in connection with any enforcement of this Policy by the Company, including paying or reimbursing such Covered Officer for insurance premiums to cover potential obligations to the Company under this Policy.

(f) Indemnification of Administrator. Any members of the Administrator, and any other members of the Board who assist in the administration of this Policy, shall not be personally liable for any action, determination or interpretation made with respect to this Policy and shall be indemnified by the Company to the fullest extent under applicable law and Company policy with respect to any such action, determination or interpretation. The foregoing sentence shall not limit any other rights to indemnification of the members of the Board under applicable law or Company policy.

(g) No "Good Reason" for Covered Officers. Any action by the Company to recoup or any recoupment of Recoverable Incentive Compensation under this Policy from a Covered Officer shall not be deemed (i) "good reason" for resignation or to serve as a basis for a claim of constructive termination under any benefits or compensation arrangement applicable to such Covered Officer, or (ii) to constitute a breach of a contract or other arrangement to which such Covered Officer is party.

5. Administration

Except as specifically set forth herein, this Policy shall be administered by the Administrator. The Administrator shall have full and final authority to make any and all determinations required under this Policy. Any determination by the Administrator with respect to this Policy shall be final, conclusive and binding on all interested parties and need not be uniform with respect to each individual covered by this Policy. In carrying out the administration of this Policy, the Administrator is authorized and directed to consult with the full Board or such other committees of the Board as may be necessary or appropriate as to matters within the scope of such other committee's responsibility and authority. Subject to applicable law, the Administrator may authorize and empower any officer or employee of the Company to take any and all actions that the Administrator, in its sole discretion, deems necessary or appropriate to carry out the purpose and intent of this Policy (other than with respect to any recovery under this Policy involving such officer or employee).

6. Severability

If any provision of this Policy or the application of any such provision to a Covered Officer shall be adjudicated to be invalid, illegal or unenforceable in any respect, such invalidity, illegality or unenforceability shall not affect any other provisions of this Policy, and the invalid, illegal or unenforceable provisions shall be deemed amended to the minimum extent necessary to render any such provision or application enforceable.

7. No Impairment of Other Remedies

Nothing contained in this Policy, and no recoupment or recovery as contemplated herein, shall limit any claims, damages or other legal remedies the Company or any of its affiliates may have against a Covered Officer arising out of or resulting from any actions or omissions by the Covered Officer. This Policy does not preclude the Company from taking any other action to enforce a Covered Officer's obligations to the Company, including, without limitation, termination of employment and/or institution of civil proceedings. This Policy is in addition to the requirements of Section 304 of the Sarbanes-Oxley Act of 2002 ("***SOX 304***") that are applicable to the Company's Chief Executive Officer and Chief Financial Officer and to any other compensation recoupment policy and/or similar provisions in any employment, equity plan, equity award, or other individual agreement, to which the Company is a party or which the Company has adopted or may adopt and maintain from time to time; provided, however, that compensation recouped pursuant to this Policy shall not be duplicative of compensation recouped pursuant to SOX 304 or any such compensation recoupment policy and/or similar provisions in any such employment, equity plan, equity award, or other individual agreement except as may be required by law.

8. Amendment; Termination

The Administrator may amend, terminate or replace this Policy or any portion of this Policy at any time and from time to time in its sole discretion. The Administrator shall amend this Policy as it deems necessary to comply with applicable law or any Listing Standard.

9. Successors

This Policy shall be binding and enforceable against all Covered Officers and, to the extent required by Rule 10D-1 and/or the applicable Listing Standards, their beneficiaries, heirs, executors, administrators or other legal representatives.

10. Required Filings

The Company shall make any disclosures and filings with respect to this Policy that are required by law, including as required by the SEC.

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