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, 2025

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, 2025 was $6.7 billion, based on the closing price reported for such date on the Nasdaq Global Select Market.

As of February 19, 2026, the registrant had 192,323,359 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 2026 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, 2025, are incorporated by reference under Part III of this Annual Report on Form 10-K.

BIOMARIN PHARMACEUTICAL INC.

2025 FORM 10-K ANNUAL REPORT
TABLE OF CONTENTS

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®, ROCTAVIAN®, VIMIZIM® and VOXZOGO® are our registered trademarks. 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

- Our success depends on our ability to manage our growth and execute our corporate strategy.
- 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.
- We have in the past and may in the future pursue acquisitions of other companies or businesses, which could divert our management's attention, fail to achieve the anticipated benefits and/or expose us to other risks or difficulties.
- 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.
- 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.
- 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.
- 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.
- Changes in methods of treatment of disease or failure of our products to gain acceptance by patients or the medical community could negatively impact demand for our products and adversely affect revenues.

Amicus Acquisition Risks

- The pending Amicus Acquisition may not be completed on the currently contemplated timeline or terms, or at all.
- We may not realize the anticipated benefits from the pending Amicus Acquisition.

- The pendency of the Amicus Acquisition could adversely affect our and/or Amicus' businesses and operations.
- We expect to incur material expenses related to the Amicus Acquisition.
- We may not realize the anticipated cost savings from the Amicus Acquisition.

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 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.
- We have incurred in the past and may in the future incur 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.

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 operations and generate a significant percentage of our sales 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

BioMarin Pharmaceutical Inc. (BioMarin, we, us or our) is a leading, global rare disease biotechnology company focused on delivering medicines for people living with genetically defined conditions. Our San Rafael, California-based company, founded in 1997, has a proven track record of innovation, with eight commercial therapies and a strong clinical and preclinical pipeline. Using a distinctive approach to drug discovery and development, we seek to unleash the full potential of genetic science by pursuing category-defining medicines that have a profound impact on patients.

Recent Developments

In 2025, we achieved $3.2 billion in total revenues, including a significant contribution from our ongoing expansion of VOXZOGO, and we continued to grow our commercial business and advance our product candidate pipeline. We believe that the combination of our internal research programs, partnerships and acquisitions of external assets will allow us to continue to develop and commercialize innovative therapies for patients with serious and life-threatening rare diseases and medical conditions. We periodically conduct strategic portfolio assessments of research and development programs to determine which we believe have the strongest combination of scientific merit, opportunity for commercial success and potential value creation for stockholders. Based on such strategic portfolio assessments, certain programs that do not meet our threshold for further development and commercialization could be discontinued.

In December 2025, we entered into a definitive agreement to acquire Amicus Therapeutics, Inc. (Amicus), a publicly traded, global, biotechnology company for $14.50 per share in an all-cash transaction for a total consideration of approximately $4.8 billion. The pending acquisition is expected to strengthen our commercial portfolio by adding two new therapies for the treatment of Fabry disease and late-onset Pompe disease. The transaction is expected to close in the second quarter of 2026, subject to regulatory clearances, approval by the stockholders of Amicus and other customary closing conditions. We intend to finance the transaction through a combination of cash on hand and approximately $3.7 billion of non-convertible debt financing.

In December 2025, we entered into a debt financing commitment letter and related fee letter with certain lenders, pursuant to which the lenders have committed to provide us with debt financing up to approximately $3.7 billion (the Bridge Commitment) in the form of a 364-day senior secured bridge loan facility (Bridge Facility) for the pending acquisition of Amicus. No amounts had been drawn or were outstanding under the Bridge Commitment as of December 31, 2025. In February 2026, we issued $850.0 million in aggregate principal amount of 5.5% senior unsecured notes due 2034 (the 2034 Notes), and the proceeds from the issuance were deposited into an escrow account that will be used to finance the pending acquisition of Amicus. In connection with the issuance of the 2034 Notes, the Bridge Commitment was reduced from approximately $3.7 billion to $2.8 billion. In place of the Bridge Facility, we also expect to enter into a senior secured term loan facility for approximately $2.8 billion in aggregate principal and a new $600.0 million senior secured revolving credit facility in 2026 that will be executed prior to or concurrently with the closing of the pending Amicus acquisition. See Item 7. "Management's Discussion and Analysis of Financial Condition and Results of Operations–Financial Condition, Liquidity and Capital Resources" below for additional information.

In October 2025, we announced our plan to pursue options to divest ROCTAVIAN, including exploring out-licensing opportunities. Subsequently in December 2025, we committed to a plan to voluntarily withdraw ROCTAVIAN from the market due to lower than previously anticipated commercial opportunities. In connection with this strategic decision, we recorded approximately $240.0 million of restructuring charges in 2025 comprised of an inventory write-off, impairment of long-lived assets, severance and other costs. See Note 19 to our accompanying Consolidated Financial Statements for additional details.

In July 2025, we completed the acquisition of Inozyme Pharma, Inc. (Inozyme), a publicly traded clinical-stage biopharmaceutical company dedicated to developing innovative therapeutics. The acquisition is intended to strengthen our enzyme therapies portfolio by adding a late-stage enzyme replacement therapy, BMN 401 (formerly INZ-701), for the treatment of ectonucleotide pyrophosphatase/phosphodiesterase 1 (ENPP1) deficiency. We accounted for this transaction as an asset acquisition since the lead asset, BMN 401, represents substantially all of the fair value of the gross assets acquired. See Note 20 to our accompanying Consolidated Financial Statements for additional information related to Inozyme acquisition.

Commercial Products

Commercial Products	Indication	2025 Net Product Revenues (in millions of U.S. Dollars)
VOXZOGO (vosoritide)	Achondroplasia	$ 926.9
Enzyme Therapies:		
VIMIZIM (elosulfase alpha)	Mucopolysaccharidosis (MPS) IVA	$ 792.1
NAGLAZYME (galsulfase)	MPS VI	$ 485.4
PALYNZIQ (pegvaliase-pqpz)	Phenylketonuria (PKU)	$ 433.3
ALDURAZYME (laronidase)	MPS I	$ 208.5
BRINEURA (cerliponase alfa)	Neuronal ceroid lipofuscinosis type 2 (CLN2)	$ 186.4
KUVAN (sapropterin dihydrochloride)	PKU	$ 99.6
ROCTAVIAN (valoctocogene roxaparvovec)[(1)]	Severe Hemophilia A	$ 35.6

(1) In 2026, we announced that we will no longer market ROCTAVIAN. See Note 19 to our accompanying Consolidated Financial Statements for additional details.

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

We continue to research VOXZOGO's safety and effectiveness in children with achondroplasia while also advancing development across our CANOPY clinical program with VOXZOGO for the treatment of conditions beyond achondroplasia, including hypochondroplasia, idiopathic short stature, Noonan syndrome, Turner syndrome, and SHOX deficiency. Please see "Research and Development Programs – VOXZOGO" in this Annual Report on Form 10-K for additional information.

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.

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. 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.

In 2025, we reported positive data from the PALYNZIQ Phase 3 PEGASUS study in 12- to 17-year-olds demonstrating statistically significant reductions in blood Phe lowering compared to diet alone for adolescents with PKU.

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.

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.

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. and in the EU for children of all ages and in other 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.

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. and the EU. Several generic versions of KUVAN have also been approved either centrally by the European Commission (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.

ROCTAVIAN was conditionally approved by the EC in August 2022 and approved by the FDA in the U.S. in June 2023. Please see “Government Regulation – Adaptive Pathways” in this Annual Report on Form 10-K for additional information on conditional marketing authorizations.

In October 2025, we announced our plan to pursue options to divest ROCTAVIAN, including exploring out-licensing opportunities. Subsequently in December 2025, we committed to a plan to voluntarily withdraw ROCTAVIAN from the market due to lower than previously anticipated commercial opportunities. In connection with this strategic decision, we recorded approximately $240 million of restructuring charges in 2025 comprised of an inventory write-off, impairment of long-lived assets, severance and other costs. See Note 19 to our accompanying Consolidated Financial Statements for additional details.

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. A summary of our key clinical stage programs is provided below.

VOXZOGO

In 2025, we continued to advance development across our CANOPY clinical program with VOXZOGO for the treatment of hypochondroplasia, idiopathic short stature, Noonan syndrome, Turner syndrome, and SHOX deficiency, including a pivotal Phase 3 study in hypochondroplasia and Phase 2 studies in idiopathic short stature, Noonan syndrome, Turner syndrome, and SHOX deficiency.

BMN 333

BMN 333 is a longer-acting CNP in development for the treatment of multiple growth disorders, including achondroplasia and hypochondroplasia. We initiated the first-in-human study of BMN 333 in January 2025. In 2025, we accelerated development of BMN 333 with Phase 1 pharmacokinetic data exceeding targeted free CNP exposure levels.

BMN 351

BMN 351 is our next-generation oligonucleotide in development for the treatment of Duchenne Muscular Dystrophy (DMD). We completed enrollment into the first and second dose cohorts in late 2024. In 2025, we progressed BMN 351 with initial

Phase 1/2 data demonstrating 5.0% mean absolute dystrophin expression (without double-correction for histologic adjustment for muscle content) at week 25 in the 9 mg/kg cohort.

BMN 401

In 2025, we acquired BMN 401 (formerly INZ-701) through our acquisition of Inozyme. BMN 401 is a soluble, recombinant, or genetically engineered, ENPP1 fusion protein that is designed to increase inorganic pyrophosphate (PPi) and adenosine, to enable the potential treatment of multiple diseases caused by deficiencies in these molecules. By targeting the PPi-Adenosine Pathway, BMN 401 aims to correct pathologic mineralization and intimal proliferation, addressing the significant morbidity and mortality in these devastating diseases.

Manufacturing

We manufacture the active pharmaceutical ingredients (API) for ALDURAZYME, NAGLAZYME, PALYNZIQ and VOXZOGO in our production facilities located in Novato, California. 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, which received EU approval in 2024. Additional 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 PALYNZIQ and KUVAN API. Additionally, most of our drug product manufacturing (which includes vials, syringes, tablets, and powder) is performed externally by contract manufacturers. We expect the volume mix to 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

Our commercial organization is primarily structured around two business units: Skeletal Conditions (VOXZOGO) and Enzyme Therapies. This organization, which includes a sales force, supports 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 2026, 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, or for the

sales and marketing of products that we acquire through strategic transactions, 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 our products. 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 2025, 37% 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 PALYNZIQ 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.

VOXZOGO

VOXZOGO, for the treatment of achondroplasia, could have competition from clinical stage products under development by Abbisko Therapeutics Co Ltd., Ascendis Pharma A/S, QED Therapeutics, Inc. (a subsidiary of BridgeBio Pharma, Inc.), Ribomic Inc., Tyra Biosciences Inc., and preclinical product candidates from other companies, including Black Diamond Therapeutics, Inc., C-Biomex Co., Ltd., Changchun GeneScience Pharmaceuticals Co., Ltd., Immunoforge, Co. Ltd., Peptron Inc., Prolynx Inc., and SiSaf Ltd.

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

PALYNZIQ and KUVAN face competition from an oral small molecule marketed by PTC Therapeutics,Inc., which is approved in the U.S. and EU for the treatment of PKU. There is also potential competition from clinical stage product candidates developed by Agios Pharmaceuticals Inc., Jnana Therapeutics Inc. (a subsidiary of Otsuka Pharmaceutical Co., Ltd.), Maze Therapeutics, NGGT Inc., Relief Therapeutics, SOM Innovation Biotech, S.A., and Scohia Pharma Inc., and earlier stage product candidates, including, but not limited to, product candidates from Alltrna, Inc., Iniuva GmbH, and Aurora Therapeutics.

Generic versions of KUVAN are available in several countries around the world, including multiple generic versions in the U.S. and the EU. Several generic versions of KUVAN have also 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.

Research and Development Programs

VOXZOGO

VOXZOGO, for the treatment of hypochondroplasia, could have competition from clinical stage products under development by Ascendis Pharma A/S and QED Therapeutics, Inc. (a subsidiary of BridgeBio Pharma, Inc.), and a preclinical product candidate from Tyra Biosciences Inc.

VOXZOGO, for the treatment of idiopathic short stature could have competition from marketed branded and generic human growth hormones, clinical stage products (marketed for other indications) under development by Ascendis Pharma A/S, and Novo Nordisk A/S, and additional clinical stage products by Anhui Anke Biotechnology (Group) Co., Ltd., Xiamen Amoytop Biotech Co., Ltd. and Changchun GeneScience Pharmaceuticals Co., Ltd. as well as a preclinical stage product from Bolder BioTechnology, Inc.

VOXZOGO, for the treatment of Noonan syndrome, Turner syndrome, and SHOX deficiency could have competition from marketed branded and generic human growth hormones, marketed small molecule tyrosine kinase inhibitors, clinical stage products (marketed for other indications) under development by Ascendis Pharma A/S and Novo Nordisk A/S, an additional clinical stage product from Changchun GeneScience Pharmaceuticals Co., Ltd., and a preclinical product candidate from Cavalry Biosciences.

BMN 333

BMN 333, for the treatment of achondroplasia could have competition from clinical stage products under development by Abbisko Therapeutics Co Ltd., Ascendis Pharma A/S, QED Therapeutics, Inc. (a subsidiary of BridgeBio Pharma, Inc.), Ribomic Inc., Tyra Biosciences Inc., and preclinical product candidates from other companies, including Black Diamond Therapeutics, Inc., C-Biomex Co., Ltd., Changchun GeneScience Pharmaceuticals Co., Ltd., Immunoforge, Co. Ltd., Peptron Inc., Prolynx Inc., and SiSaf Ltd.

BMN 333, for the treatment of hypochondroplasia, could have competition from clinical stage products under development by Ascendis Pharma A/S and QED Therapeutics, Inc. (a subsidiary of BridgeBio Pharma, Inc.), and a preclinical product candidate from Tyra Biosciences Inc.

BMN 351

BMN 351, for the treatment of DMD, could have competition from marketed oligonucleotide and gene therapy products by Sarepta Therapeutics, Inc., steroids by Catalyst Pharmaceuticals, Inc., PTC Therapeutics, Inc., and Santhera Pharmaceuticals Holdings AG, and an epigenetic therapy by Italfarmaco S.p.A. In addition, BMN 351 could have potential competition from clinical product candidates for exon 51 skipping amenable DMD by Dyne Therapeutics, Inc. and gene therapy product candidates from Regenxbio, Inc. and Solid Biosciences, Inc.

BMN 401

There are currently no approved, non-generic drugs on the market in the U.S. or the EU for the treatment of ectonucleotide pyrophosphatase/phosphodiesterase 1 (ENPP1) deficiency. Although there may be companies generally pursuing

development of treatments for ectopic calcification or metabolic bone disorders, we are not aware of any novel product candidates currently in active clinical development for ENPP1 deficiency.

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; licensing and acquiring new patents and patent applications; and pursuing, litigation, administrative challenges or other types of proceedings to protect our patents and intellectual property rights. For example, we are engaged in various legal actions, including actions against Ascendis Pharma A/S in multiple jurisdictions and forums to protect our patents relating to VOXZOGO. 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, NAGLAZYME and KUVAN 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.	10,279,015	Formulation; kit	May 5, 2036
	EU	EP3294345	Formulation	May 5, 2036
PALYNZIQ	U.S.	7,534,595	Composition; method of treating	May 24, 2032[(1)]
		10,221,408	Purification	February 3, 2031
		9,557,340	Antibody detection assay	July 30, 2029
		11,505,790	Regimen	February 3, 2031
		11,918,633	Method of treating adolescent subjects	May 18, 2042
	EU	2152868	Composition; pharmaceutical composition	May 23, 2028 / May 23, 2033[(2)]
		2531209; 3025728	Formulation; purification	February 3, 2031
VIMIZIM	U.S.	8,128,925	Compositions; methods of treatment	January 16, 2029
		8,765,437	Purification; formulation; methods of treatment	January 10, 2032
	EU	2245145	Composition; use for treating	April 30, 2029[(3)]
		2595650	Purification; composition; use for treating; formulation	July 22, 2031
		3219795	Method of producing	January 16, 2029
VOXZOGO	U.S.	8,198,242	Compositions, Methods of Treatment	June 11, 2030[(4)]
		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[(5)]
		3328416	Formulation, Use	August 1, 2036

(1) For PALYNZIQ, the date of expiry includes the granted patent term extension (PTE).

(2) For PALYNZIQ, we applied for SPCs for EP 2152868, and to date have been granted 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, Germany, Greece, Hungary, Ireland, Iceland, Italy, Latvia, Lithuania, Luxembourg, Malta, Netherlands, Norway, Poland, Portugal, Spain, Slovakia, Slovenia, Sweden, and United Kingdom.

(3) For VIMIZIM, we applied for SPCs for this patent, and to date have been granted SPCs 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. PTE has also been granted in Chile to February 14, 2030.

(4) For VOXZOGO, we filed for a PTE for this patent and received Notice of Final Determination granting extension of patent term by 5 years to June 11, 2035.

(5) For VOXZOGO, we applied for SPCs for EP 2432489, and to date have been granted SPCs in Croatia, Czech Republic, Denmark, Estonia, France, Greece, Hungary, Italy, Luxembourg, Norway, Portugal, Sweden, and the United Kingdom, extending the patent expiration to May 20, 2035. PTE has been granted in Australia and Japan 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	2031[3]	2029	2029
PALYNZIQ	Expired	2030	2029
VIMIZIM	Expired	2026	Expired
VOXZOGO	2030[4]	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.

(3) BRINEURA's U.S. orphan drug exclusivity relating to the treatment of CLN2 for (i) symptomatic patients of three years of age and older expired in 2024 and (ii) patients of less than three years of age and asymptomatic patients of three years of age and older expires in 2031.

(4) VOXZOGO's U.S. orphan drug exclusivity relating to the treatment of achondroplasia for (i) children of five years of age and older expires in 2028 and (ii) children of less than five years of age expires in 2030.

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 European Medicines Agency (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 effective 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 takes 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 continued to apply in parallel to the CTR for a transitional period. From January 31, 2025 all trials must 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 efficacy 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 efficacy.
- Phase 2 - usually involves trials in a limited patient population, to determine the efficacy 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 efficacy 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 company 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 company 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. However, 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 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 FDA's accelerated approval regulations, the FDA may approve a drug, including a fast track 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 verify and describe the clinical benefit of the drug. Failure to conduct a required post-approval study with due diligence, including with respect to the conditions specified by the FDA, 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 FDA is authorized to require a post-approval study to be underway prior to approval or within a specified time period following approval and is required to specify conditions of any required post-approval study, which may include milestones such as a target date of study completion. Sponsors are required 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. The FDA may 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, as applicable, 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 with due diligence 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, PMA, or MAA supplement for a new indication typically requires clinical data similar to that in the original application, and similar procedures and actions apply in reviewing NDA/BLA, PMA, or MAA supplements as in reviewing NDAs/BLAs, PMAs, 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, device, 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 previously FDA-licensed product called a reference product. There must be no differences in 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 and animal studies. The FDA may also require comparative clinical study sufficient to demonstrate safety, purity, and potency in one or more conditions of use for which the reference product is licensed. 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 other 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. While the related rules are expected to change in the near future as described below, these products currently 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 can be lost in the EU, for example, 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, under the current rules, the relevant regulatory authority may not accept or approve any other applications to market a similar medicinal product/drug for the same therapeutic indication, except in limited circumstances, for seven years in the U.S. and ten years in the EU (extendable to twelve years under certain circumstances) 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. In the U.S., orphan drug exclusive marketing rights obtained upon approval of an orphan-designated drug 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 of the orphan product. 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 is established 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. On December 11, 2025, the European Parliament and the European Council reached a political agreement on a proposal for the revision of the EU pharmaceutical regulatory framework. Among other things, the revision will amend the duration of the data and market exclusivity, the marketing authorization procedure, the possible post-authorization conditions, the reasons for a refusal of a marketing authorization, as well as obligations and waivers in connection with Pediatric Investigation Plans (PIPs). In particular, the regulatory data protection period (during which generic/biosimilar companies cannot access product data) would amount to eight years, with one additional year of market protection (during which generic or biosimilar products cannot be sold), following a marketing authorization. Pharmaceutical companies will be eligible for additional periods of market protection under certain conditions, with a cap of eleven years on the combined regulatory protection period. Orphan medicinal products addressing a disease with no current available medicinal treatment (“breakthrough orphan medicinal products”) will benefit from up to eleven years of market exclusivity. Moreover, with a view to ensure earlier market entry of generic and biosimilar medicinal products, the new rules clarify that the so-called “Bolar” exemption (which allows generic/biosimilar manufacturers to conduct certain activities during the patent protection period of the original product) allows the conduct of necessary studies, trials and other activities for the purposes of obtaining marketing authorizations, conducting health technology assessments, obtaining pricing and reimbursement approvals, or submitting procurement tender applications.

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. 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. 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 and/or notifications and information about price increases that exceed a specified threshold. 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 U.S. persons, including U.S. entities and their employees, officers, and intermediaries from engaging in certain activities, directly or indirectly, to obtain or retain business or to influence a person working in an official capacity. It is illegal to pay, provide offer to pay or authorize the provision of anything of value to any non-U.S. government official, government staff member, employee or officer of a state-owned or controlled entity, 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 in the public and private sectors. 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.

In the U.S., 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 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 necessarily mean 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 minimum 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 minimum rebate amount is generally 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 and non-innovator products may also be subject to an additional inflation rebate that is based on the amount, if any, by which the product's AMP for a given quarter exceeds the AMP for a previous baseline quarter. For many years, the total rebate amount for a unit of a drug was capped at 100% of the AMP; however, effective January 1, 2024, this cap was eliminated. Elimination of this cap has, in some cases, required pharmaceutical manufacturers to pay more in rebates than they have received on the sale of products.

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. CMS may also impose penalties against a manufacturer that is notified by CMS about a "misclassification", defined as an incorrectly reported drug attribute (for example, an erroneous classification of non-innovator rather than innovator), and fails to correct the error within 30 days. The penalties include monetary penalties and/or suspension of the drug from Medicaid coverage until the error is corrected. CMS may also suspend a manufacturer's Medicaid rebate agreement if a manufacturer fails to submit the required pricing reports or fails to correct a misclassification within 90 calendar days after receiving notice from CMS.

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 hospital outpatient departments 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, and in November 2024, CMS finalized regulations pertaining to the Medicare Part B inflation rebates. 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 individuals at least 65 years of age and certain people with disabilities. Enrollees in Part D plans, after they meet a deductible, pay a co-insurance of 25% until they reach the out-of-pocket limit, after which they have no co-insurance. The out-of-pocket limit is $2,100 in 2026 and is to be adjusted for inflation thereafter. Each manufacturer of drugs approved under NDAs or BLAs, in order for these drugs to be reimbursed by Medicare Part D, is required to enter into a Medicare Part D manufacturer discount agreement with HHS and provide a discount on those drugs dispensed to Medicare Part D enrollees. The discount is 10% of Part D enrollees' prescription costs for brand drugs above the deductible and below the out-of-pocket limit, and 20% once the out-of-pocket limit has been reached. 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, and in November 2024, CMS finalized regulations pertaining to Medicare Part D inflation rebates.

The IRA also requires 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. The negotiated price may not exceed a statutory ceiling price. 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. For 2026, the first year in which negotiated prices become effective, CMS selected 10 high-cost Medicare Part D products in 2023, negotiations began in 2024, and the negotiated maximum fair price for each product has been announced. In addition, CMS has selected and announced the negotiated maximum fair price for 15 additional Medicare Part D drugs, which will become effective in 2027. For 2028, CMS has selected an additional 15 drugs, comprised of drugs covered under Medicare Part D and, for the first time, drugs payable under Medicare Part B. For 2029 and subsequent years, 20 Part B or Part D drugs will be selected. 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. However, as a result of a statutory amendment enacted in July 2025, beginning with the 2028 negotiated price applicability year, a drug may be designated for more than one rare disease or condition and still be excluded from price negotiation, as long as the only approved indications are for such rare diseases or conditions.

In addition, the current U.S. Presidential Administration is pursuing policies to reduce regulations and expenditures across government including at HHS, which include the FDA and CMS, and related agencies. For example, on May 12, 2025, President Trump issued an executive order that, among other things, required HHS, within 30 days, to establish and communicate to drug manufacturers most favored nation (MFN) price targets designed to bring drug prices for American patients in line with those in comparably developed nations. If significant progress towards MFN pricing is not achieved, the executive order requires HHS to propose a rulemaking to implement MFN pricing. On December 23, 2025, CMS issued proposed regulations to establish, under the

Center for Medicare and Medicaid Innovation, two mandatory MFN demonstration models under Medicare Parts B and D, respectively.

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), which is administered by the Department of Veterans Affairs. 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 the manufacturer's covered drugs to be eligible for purchase by these agencies and also in order for federal funding to be available for reimbursement of the manufacturer's drugs under Medicaid and Medicare Part B. 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 government fiscal 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, and civil penalties under the Federal False Claims Act if such failures are knowing.

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 current 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 BPCA 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. Currently, 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. Furthermore, it is expected that these rules will be amended as a result of a reform of the EU pharmaceutical legislative package. On December 11, 2025, the European Parliament and the European Council reached a political agreement on the proposed revision of several European legislative instruments related to medicinal products, including pediatric products. Among other things, the revision will likely amend the PIP waivers and the possible PIP obligations.

Privacy and Security Legislation

The legislative and regulatory environments regarding privacy and data protection are continually evolving and developing, in response to increasing global attention. In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal data privacy laws, consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), and other similar laws (e.g., wiretapping laws). Numerous U.S. states have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording residents with certain rights concerning their personal data. As applicable, such rights may include the right to access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments. These state laws allow for statutory fines for noncompliance. For example, the California Consumer Privacy Act of 2018 (CCPA) applies to personal data of consumers, business representatives, and employees who are California residents, and requires businesses to provide specific disclosures in privacy notices and honor requests of such individuals to exercise certain privacy rights. The CCPA provides for fines and allows private litigants affected by certain data breaches to recover significant statutory damages.

Other jurisdictions have enacted or proposed similar legislation and/or regulations. Additionally, 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 their personal data.

Outside of the United States, the EU’s General Data Protection Regulation (EU GDPR) and UK's General Data Protection Regulation (UK GDPR and collectively, GDPR), which require, among other things, 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. 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, 2025, we had 3,221 employees worldwide, of whom 1,518 were in operations, 692 were in research and development, 495 were in sales and marketing and 516 were in administration. Of the 3,221 employees as of December 31, 2025, 2,026 employees were in the U.S. and Canada, and 1,195 employees were outside of North America, including 967 in Europe and the Middle East, 151 in Latin America and 77 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.

Compensation, Benefits and Well-being

We offer competitive compensation and benefits in order to attract, recognize 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 in recognition of performance, equity in recognition of performance and potential, healthcare benefits, among other benefits.

Workplace Culture

We believe that scientific breakthroughs happen when different perspectives come together to solve complex problems. Our dedication to this work ensures that we are harnessing the full range of our talent to drive innovation at BioMarin for patients around the world. We know that the power of different viewpoints and experiences will drive a culture of inclusion, which creates innovation and growth. BioMarin is committed to leveraging the collective genius of its global workforce and is dedicated to recruiting from a broad range of backgrounds and experiences to ensure we find and hire best talent.

Professional Growth and Development

We help our employees develop the skills and capabilities to support BioMarin's growth and innovation through a combination of development experiences on the job and formal programs. 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.

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

Our success depends on our ability to manage our growth and execute our corporate strategy.

We continue to pursue the corporate strategy we announced in 2024, which is focused on innovation, growth, and value commitment, which includes, among other things, the acceleration or discontinuation of certain programs, the continued expansion of our pipeline, updates to our commercial organizational model, and cost transformation. If we are unable to successfully execute our strategy, our business, financial condition and results of operations may be materially and adversely affected.

As part of the strategy, we are continuing to advance VOXZOGO for the treatment of conditions beyond achondroplasia, including hypochondroplasia, idiopathic short stature, Noonan syndrome, Turner syndrome, and SHOX deficiency. VOXZOGO addresses 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. We are also expanding our pipeline through external innovation. In December 2025, we announced our proposed acquisition of Amicus Therapeutics, Inc. (Amicus) to expand and diversify our rare disease product portfolio (the Amicus Acquisition). In addition, in July 2025 we acquired Inozyme Pharma, Inc. (Inozyme) to strengthen our Enzyme Therapies portfolio, adding a late-stage enzyme replacement therapy, BMN 401 (formerly INZ-701). In order to continue the development of our 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. 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.

In addition, there is no guarantee that our corporate strategy will generate its expected benefits and the costs associated with implementing such strategy may be greater than anticipated. The execution of such strategy may also adversely affect our internal programs and initiatives as well as our ability to recruit and retain skilled and motivated personnel. If we are unable to execute on our corporate strategy or realize its expected benefits, then our business, operating results and financial condition may be materially and adversely affected.

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 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 have in the past attempted and may in the future 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.

We have in the past and may in the future pursue acquisitions of other companies or businesses, which could divert our management's attention, fail to achieve the anticipated benefits and/or expose us to other risks or difficulties.

As part of our new corporate strategy, we have acquired, entered into agreements to acquire, and may continue to acquire, companies or businesses that we believe could complement, expand or enhance our product offerings. For example, in December 2025, we announced our proposed acquisition of Amicus to expand and diversify our rare disease product portfolio. In addition, in July 2025, we completed the acquisition of Inozyme to strengthen our Enzyme Therapies portfolio with BMN 401, which is currently being assessed for the treatment of ectonucleotide pyrophosphatase/phosphodiesterase 1 (ENPP1) deficiency. Our acquisition strategy may divert the attention of management and cause us to incur various expenses in identifying, investigating and pursuing suitable acquisitions, whether or not such acquisitions are consummated.

In addition, once an acquisition is closed, integrating our business practices and operations with the acquired business' so that we can fully realize the anticipated benefits of the acquisition could require us to devote significant management attention and resources. The success of current and future acquisitions will depend, in part, on our ability to realize the anticipated benefits from successfully combining our and the acquired businesses' operations. We may face risks or experience difficulties successfully integrating acquired businesses, such as Inozyme or Amicus, with our operations. Such difficulties could result in the failure to achieve revenue that we anticipate, the loss of key employees that may be difficult to replace in the very competitive pharmaceutical field, the failure to harmonize both companies' corporate cultures, the disruption of each company's ongoing businesses or inconsistencies in standards, controls, procedures and policies that adversely affect our ability to maintain relationships with suppliers, collaboration partners, clinical trial investigators or managers of our clinical trials. See "—Risks Related to the Amicus Acquisition" in this section for additional risks related to the Amicus Acquisition.

Acquisitions could also result in dilutive issuances of equity securities, the incurrence of debt, contingent liabilities, amortization expenses, impairment of goodwill and/or purchased long-lived assets, and restructuring charges, any of which could adversely affect our operating results and financial condition. For example, we have incurred, and plan to incur, significant indebtedness in connection with the Amicus Acquisition. In addition, acquired product candidates, such as BMN 401, may not result in regulatory approval, may not perform as expected, may not be successful, may require significantly greater resources and investments than originally anticipated or may not produce the revenues, earnings or business synergies that we anticipated. As a result, the anticipated benefits of an acquisition may not be realized fully within the expected timeframe or at all or may take longer to realize or cost more than expected, which could materially and adversely affect our business, financial condition, results of operations and growth prospects.

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. 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.

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 VOXZOGO, other companies are developing, and may in the future develop, products for treatment for achondroplasia that, if approved, could potentially compete with VOXZOGO even during the period of orphan drug exclusivity, for example by using an alternative formulation or a different delivery technology. As we commercialize our products, we have faced and may continue to face intense competition from other pharmaceutical companies, some of which may have more extensive resources and/or established relationships in the communities we seek to treat. 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.

We also face competition from generic versions of our products. For example, generic versions of KUVAN are available in several countries around the world, including in the U.S. and the European Union (EU), which has adversely affected and will continue to adversely affect our revenues from KUVAN. Competitors launching generic versions of our products independently establish the price of such products and determine the types of discounts or rebates they will offer parties that purchase or pay for the product. Generic competition often results in decreases in the net prices at which branded products can be sold. After any introduction of a generic product, a significant percentage of the prescriptions written for our branded products will likely be filled with the generic product. Certain U.S. state laws allow for, and in some instances in the absence of specific instructions from the prescribing physician mandate, the dispensing of generic products rather than branded products when a generic version is available. We expect that the approval and launch of generic versions of our products and the approval and launch of branded products that compete with our products will continue to have a negative impact and could have a material adverse effect on our sales of our products and on our business, financial condition, results of operations and growth prospects.

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. We expect that most families of patients will not be capable of paying for our treatments themselves. For most of our products, we expect patients to need treatment for extended periods, and for some products throughout the lifetimes of the patients. 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 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.

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. 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.

Changes in methods of treatment of disease or failure of our products to gain acceptance by patients or the medical community could negatively impact demand for our products and adversely affect revenues.

Even if our product candidates are approved, if doctors were to 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.

For example, we faced significant uncertainty as to whether gene therapy would gain the acceptance of the public or the medical community. In October 2025, we announced our plan to pursue options to divest ROCTAVIAN, including exploring out-licensing opportunities. Subsequently in December 2025, we committed to a plan to voluntarily withdraw ROCTAVIAN from the market due to lower than previously anticipated commercial opportunities. In connection with this strategic decision, we recorded approximately $240.0 million of restructuring charges in 2025 comprised of an inventory write-off, impairment of long-lived assets, severance and other cost.

In addition, if we do not accurately forecast demand or manufacture products at levels in alignment with actual demand due to the failure of our products to gain acceptance by the patients or the medical community or other factors, then we may experience product shortages, pay a fee to contract manufacturers with whom we have non-cancellable capacity reservation agreements, or build excess inventory that may need to be written off, all of which could adversely affect our operating results.

We have in the past entered 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 entered 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.

Risks Related to the Amicus Acquisition

The pending Amicus Acquisition may not be completed on the currently contemplated timeline or terms, or at all.

The consummation of the Amicus Acquisition is subject to the satisfaction or waiver of certain conditions. Satisfaction of a number of the conditions is not within our control, and it is possible that such conditions may prevent or delay or otherwise materially adversely affect our ability to complete the Amicus Acquisition. These conditions include, but are not limited to, approval of the Amicus Acquisition by Amicus' stockholders and the expiration or termination of the relevant waiting period (as it may be extended) under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 and the rules and regulations promulgated thereunder. Neither we nor Amicus can provide assurance that the conditions to completing the Amicus Acquisition will be satisfied or waived, and accordingly, that the Amicus Acquisition will be completed on the timeline that the parties anticipate or at all. If any condition to the Acquisition is not satisfied, it could delay or prevent the Amicus Acquisition from occurring, which could negatively impact us and our growth prospects.

We may not realize the anticipated benefits from the pending Amicus Acquisition.

The Amicus Acquisition involves the combination of two companies that currently operate as independent companies. While we and Amicus will continue to operate independently until the Amicus Acquisition is consummated, the success of the Amicus Acquisition will depend, in part, on our ability to realize the anticipated benefits from successfully combining our and Amicus' businesses after closing. We plan on devoting substantial management attention and resources to integrating our and Amicus' businesses so that we can fully realize the anticipated benefits of the Amicus Acquisition. This integration process may be disruptive to our and Amicus' businesses, and, if implemented ineffectively, could restrict realization of the expected benefits of the Amicus Acquisition. In addition, the acquired Amicus business, including Galafold and Pombiliti + Opfolda, may not be successful,

may require greater resources and investments than originally anticipated or may result in the assumption of unknown or contingent liabilities, which could have an adverse effect on us or our results of operations.

Potential difficulties we may encounter following closing include the following:

- the inability to successfully combine our and Amicus' businesses in a manner that permits us to realize the anticipated benefits of the Amicus Acquisition in the timeframe currently anticipated or at all;
- the failure to integrate internal systems, programs and internal controls, or applying different accounting policies, assumptions or judgments to Amicus' operational results than Amicus applied in the past;
- the inability to successfully obtain regulatory approval in new markets for, and continue to commercialize, Galafold or Pombiliti + Opfolda on the currently anticipated timeline or at all;
- The inability to effectively and efficiently integrate information technology and other systems;
- issues not discovered as part of the transactional due diligence process or unanticipated liabilities or contingencies of Amicus, including employment or severance-related obligations under applicable law or other benefits arrangements, claims by or amounts owed to vendors or other commercial disputes, cyber incidents and information technology failures or delays, matters related to data privacy, data localization and the handling of personally identifiable information, intellectual property-related claims, including Hatch-Waxman litigation, and other unknown or contingent liabilities;
- preserving the important licensing, marketing, and other commercial relationships of Amicus;
- the complexities associated with managing the combined company;
- the failure to retain key employees of either of the two companies who may be difficult to replace;
- the disruption of each company's ongoing businesses or inconsistencies in services, standards, controls, procedures and policies;
- potential unknown liabilities and unforeseen increased expenses, delays or regulatory conditions associated with the Amicus Acquisition; and
- performance shortfalls at one or both of the two companies as a result of the diversion of management's attention caused by completing the Amicus Acquisition and integrating our and Amicus' operations.

Any of these risks could adversely affect our ability to maintain relationships with collaboration partners, vendors, employees and other commercial relationships or adversely affect our or Amicus' future operational results. As a result, the anticipated benefits of the Amicus Acquisition may not be realized or at all or may take longer to realize or cost more than expected, which could adversely affect our business, financial condition, including our ability to generate sufficient cash to service our indebtedness, including our 1.25% senior subordinated convertible notes due in 2027 (the 2027 Notes), our 5.5% senior unsecured notes due in 2034 (the 2034 Notes and together with the 2027 Notes, the Notes) and the new senior secured credit facilities expected to be entered into in connection with the Amicus Acquisition, results of operations and growth prospects, and we may be forced to take other actions to satisfy our obligations under our indebtedness, which may not be successful. In addition, changes in laws and regulations could adversely impact our business, financial condition, results of operations and growth prospects after the Amicus Acquisition.

The pendency of the Amicus Acquisition could adversely affect our and/or Amicus' businesses and operations.

In connection with the pending Amicus Acquisition, some collaboration partners, vendors or other parties with commercial relationships with either of us or Amicus may delay or defer decisions, which could adversely affect the revenues, earnings, cash flows and expenses of us or Amicus, regardless of whether the Amicus Acquisition is completed. In addition, due to operating covenants in the Agreement and Plan of Merger we entered into with Amicus, Amicus may be unable (without our prior written consent), during the pendency of the Amicus Acquisition, to pursue strategic transactions, undertake significant capital projects or otherwise pursue other actions outside the ordinary course, even if such actions would prove beneficial.

We expect to incur material expenses related to the Amicus Acquisition.

We expect to incur material expenses in connection with the Amicus Acquisition and the subsequent integration of the business, operations, practices, policies and procedures of Amicus. These additional expenses could have an adverse effect on us or our results of operations. While we have assumed that a certain level of transaction and integration expenses would be incurred,

there are a number of factors beyond our control that could affect the total amount or the timing of integration expenses. Many of the expenses that will be incurred, by their nature, are difficult to estimate accurately at the present time.

We may not realize the anticipated cost savings from the Amicus Acquisition.

The benefits that we expect to achieve as a result of the Amicus Acquisition will depend, in part, on our ability to realize anticipated cost savings, including resulting from expected operational synergies and global infrastructure efficiencies. After the Amicus Acquisition, we believe that we will be able to, among other matters, save on our costs by being able to streamline administrative processes and harmonize global distribution of the two companies.

Our success in realizing these cost savings, and the timing of this realization, depends on many factors. Even if we are able to consummate the Amicus Acquisition successfully, this may not result in the full realization of the cost savings that we currently expect, either within the expected timeframe, or at all. In addition, we cannot assure you that the costs to achieve these cost savings will not be higher than we anticipated. Therefore, we cannot assure you that any anticipated cost savings will be achieved or that our estimates and assumptions will prove to be accurate. If our cost savings are less than our estimates or our costs savings initiatives adversely affect our business or cost more or take longer to implement than we project, or if our assumptions prove to be inaccurate, our results could be lower than we anticipate.

Our and Amicus' actual financial positions and results of operations may differ materially from the publicly filed unaudited pro forma condensed combined financial information.

The unaudited pro forma condensed combined financial information contained in our Current Report on Form 8-K filed on January 26, 2026 is presented for illustrative purposes only and may differ materially from what our actual financial position or results of operations would have been had the Amicus Acquisition been completed on the dates indicated. The unaudited pro forma condensed combined financial information has been derived from our and Amicus' audited and unaudited historical financial statements and certain adjustments and assumptions have been made regarding the combined company after giving effect to the Amicus Acquisition and related transactions. The assets and liabilities of Amicus were measured at fair value based on various preliminary estimates using assumptions that we believed were reasonable utilizing information currently available. The process for estimating the fair value of acquired assets and assumed liabilities requires the use of judgment in determining the appropriate assumptions and estimates. These estimates may be revised as additional information becomes available and as additional analyses are performed. Differences between preliminary estimates in the unaudited pro forma condensed combined financial information and the final acquisition accounting will occur and could have a material impact on the unaudited pro forma condensed combined financial information and the ultimate combined company's financial position and future results of operations.

In addition, the assumptions used in preparing the unaudited pro forma condensed combined financial information may not prove to be accurate, and other factors may affect our financial condition or results of operations following the completion of the Amicus Acquisition.

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 approval from the U.S. Food and Drug Administration (FDA) for each product candidate that we intend to commercialize, and in the EU, we must obtain approval from the European Commission (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, the EU pharmaceutical reform will generally result in a decrease in data and market exclusivity 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. Our 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 the relevant EU technical documentation assessment certificate, 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.

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 marketed products have received regulatory approval to be commercially marketed and sold in the U.S., the EU, and certain other countries. 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. Failure to continue to show favorable risk-benefit balance for BRINEURA could result in the withdrawal of the marketing approval.

Moreover, promotional communications with respect to 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, which is also subject to restrictions. Public prosecutors, industry associations, healthcare professionals and other authorities and members of the public, including competitors, 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 published 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 pricing, 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 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 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. For example, the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the PPACA), expanded 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.

Several healthcare reform initiatives culminated in the enactment of the Inflation Reduction Act (IRA) in August 2022, which, among other things, eliminated, beginning in 2025, the coverage gap under Medicare Part D by significantly lowering the enrollee maximum out-of-pocket costs and requiring manufacturers to subsidize, through a newly established manufacturer discount program, 10% of Part D enrollees' prescription costs for brand drugs below the out-of-pocket limit, and 20% once the out-of-pocket limit has been reached. The IRA also requires the 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. The negotiated price may not exceed a statutory ceiling price. Only high-expenditure single-source drugs that have been approved for at least seven years (11 years for biologics) can be selected by CMS for negotiation, with the negotiated price taking effect two years after the selection year. For 2026, the first year in which negotiated prices become effective, CMS selected 10 high-cost Medicare Part D products in 2023, negotiations began in 2024, and the negotiated maximum fair price for each product has been announced. In addition, CMS has selected and announced the negotiated maximum fair price for 15 additional Medicare Part D drugs which will become effective in 2027. For 2028, CMS has selected an additional 15 drugs, comprised of drugs covered under Medicare Part D and, for the first time, drugs payable under Medicare Part B. For 2029 and subsequent years, 20 Part B or Part D drugs will be selected. The IRA also imposes rebates on Medicare Part B and Part D drugs whose prices have increased at a rate greater than the rate of inflation, and in 2024, CMS finalized regulations for the Medicare Part B and Part

D inflation rebates. 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 began 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.

In addition, the current U.S. Presidential Administration is pursuing policies to reduce regulations and expenditures across government including at HHS, which include the FDA and CMS, and related agencies. For example, on May 12, 2025, President Trump issued an executive order that, among other things, required HHS, within 30 days, to establish and communicate to drug manufacturers most favored nation (MFN) price targets designed to bring drug prices for American patients in line with those in comparably developed nations. If significant progress towards MFN pricing is not achieved, the executive order requires HHS to propose a rulemaking to implement MFN pricing. On December 23, 2025, CMS issued proposed regulations to establish, under the Center for Medicare and Medicaid Innovation, two mandatory MFN demonstration models under Medicare Parts B and D, respectively. If these rules or other MFN pricing rules are finalized, they are likely to reduce prices of at least some drugs in the United States, if they are also sold in comparator countries. Even if we do not market drugs in such countries, we will be indirectly affected if our drugs competed with drugs whose prices were reduced as a result of MFN pricing initiatives.

At the U.S. state level, legislatures have also increasingly enacted laws and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, price and price increase 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 may 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 entered into force in January 2025 and has resulted 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. Under the current rules 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 EC and EU Member States cannot accept another marketing authorization (MA) application or accept an application to extend existing authorizations for similar medicinal products for the same indication and no 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. Furthermore, it is expected that these periods will be shortened as a result of a reform of the EU pharmaceutical legislative package. On December 11, 2025, the European Parliament and the European Council reached a political agreement on the proposed revision of several European legislative instruments related to medicinal products, including orphan products. Among other things, the revision will amend the duration of the regulatory exclusivity. In particular, the regulatory data protection period (during which other companies cannot access product data) would amount to eight years, with one additional year of market protection (during which generic or biosimilar products cannot be sold), following an MA. Pharmaceutical companies would be eligible for additional periods of market protection under certain conditions, with a cap of eleven years on the combined regulatory protection period. Orphan medicinal products addressing a disease with no current available medicinal treatment (“breakthrough orphan medicinal products”) would benefit from up to eleven years of market exclusivity. These rules, if formally approved by the European Parliament and the European Council to become law, could adversely affect our products.

In addition, 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, for example, "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, 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, 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. 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. The FDA could also interpret the term “condition” narrowly, which could allow for indirect competition during the period of exclusivity. 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 clinically superior by means of greater safety or effectiveness by making a major contribution to patient care. Further, 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 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 Biologics License Application (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 the EU, a biosimilar is typically defined as a biological medicine similar to another already approved biological medicine. Developers of biosimilars are required to demonstrate by the best possible means that their biological medicine is highly similar to the reference medicine in physicochemical and biological terms, notwithstanding natural variability inherent to all biological medicines; and that any observed differences are duly justified with regard to their potential impact on safety and efficacy.

Our products approved under BLAs in the U.S., Marketing Authorization Applications (MAAs) in the EU, or comparable regulatory approval applications in other countries, as well as our product candidates that may be approved in the future, could be reference products for biosimilar marketing applications. The FDA has been changing the requirements for demonstrating biosimilarity, which may make it easier for biosimilars referencing our product to be licensed. In the U.S., a follow-on biologic may be deemed interchangeable with, and automatically substitutable for, a reference product if its sponsor can 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. In the U.S., standards for interchangeability also are changing to make it easier for biosimilars to demonstrate interchangeability. Even though the BPCIA establishes a period of 12 years of data exclusivity for reference products, 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 currently 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.

The approval of biosimilar products referencing any of our products could have a material adverse impact on sales of our products and on our business, financial condition, results of operations and growth prospects due to increased competition and pricing pressures.

Disruptions at the FDA, the EMA, other comparable regulatory authorities and other government agencies, including a reduction in some agencies' workforces and/or inadequate funding, 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.

Disruptions at regulatory authorities and government agencies due to changes in funding levels, government shutdowns, reorganization, reduction in force or statutory, regulatory and policy changes can affect their ability to hire and retain key personnel and carry out their normal functions that support our business. For example, the current U.S. administration recently implemented or proposed policies, including substantial reductions to the FDA's workforce, that may affect the FDA's review process and hinder the ability of the FDA to timely review and approve regulatory submissions for our product candidates. 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 the recent shut down that began on October 1, 2025 and lasted until November 12, 2025. 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 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, 2025, we had cash, cash equivalents and investments totaling $2.1 billion and debt obligations of $600.0 million (undiscounted), which consisted of our 2027 Notes. In February 2026, we issued $850.0 million in aggregate principal amount of the 2034 Notes. The 2027 Notes, if not converted, will be required to be repaid in cash at maturity in May 2027.

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 2027 Notes (if not converted) and the 2034 Notes.

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, protect, 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;
- our future plans for strategic investments and/or acquisitions;
- 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 in the past and may in the future incur 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, 2025, we had $600.0 million (undiscounted) principal amount of indebtedness, all of which was outstanding under the 2027 Notes. In August 2024, we entered into an unsecured credit agreement (the 2024 Credit Agreement) with Citibank, N.A., as the administrative agent, and the other lenders party thereto, providing for $600.0 million in revolving loan commitments (the 2024 Credit Facility). The 2024 Credit Facility matures in August 2029. Additionally, in February 2026, we issued the 2034 Notes. As of December 31, 2025, no amounts were outstanding under the 2024 Credit Facility. 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.

In addition, the 2024 Credit Facility and the indenture governing our 2034 Notes contain, and any future indebtedness that we may incur (including the new senior secured credit facilities expected to be entered into in connection with the Amicus Acquisition) may contain, financial and/or other restrictive covenants that limit our ability to operate our business, raise capital or make payments under our other indebtedness. If we fail to comply with these covenants or to make payments under our indebtedness when due, then we would be in default under that indebtedness, which could, in turn, result in that and our other indebtedness becoming immediately payable in full. If we default under the 2024 Credit Agreement or the indentures governing our Notes, the outstanding borrowings thereunder could become immediately due and payable, the 2024 Credit Facility lenders could refuse to permit additional borrowings under the facility, or it could lead to defaults under agreements governing our current or future indebtedness, including the 2024 Credit Agreement and the indentures governing the Notes, as applicable.

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 of the 2027 Notes, which, if not converted, will be required to be repaid in cash at maturity in May 2027, and the 2034 Notes, which are required to be repaid in cash at maturity in February 2034. 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. In addition, we also may borrow up to $600.0 million in revolving loans under the 2024 Credit Facility, which would be required to be repaid in cash at maturity in August 2029.

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, 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 candidates 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. Additionally, we have in the past experienced and may in the future experience strong demand that outpaces our projections and supply constraints. If our manufacturing capabilities are unable to meet demand, we may lose potential revenues with respect to our products or experience adverse impacts on our development programs.

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.

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 nonclinical or 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;
- geopolitical instability resulting from war, terrorism and other violence; 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 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 operations and generate a significant percentage of our sales 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;
- diminished protection of intellectual property in some countries outside of the U.S.;
- impact of new or increased tariffs, other trade protection measures (such as import or export licensing requirements), and escalating trade tensions;

- difficulty in staffing and managing international operations;
- differing labor regulations and business practices;
- parallel trade in our products, such as importation or our products, whether legally or illegally, from countries where our products are sold at lower prices into countries where the products are sold at higher prices;
- 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 but have, or are in the process, of being officially approved for national reimbursement in countries where patient numbers are sufficient for it to apply. 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 Japanese Yen, the Canadian Dollar, the Argentine Peso, the Colombian Peso, the Mexican Peso, the British Pound and several other currencies, changes in those currencies relative to the U.S. Dollar (USD) have in the past and may in the

future impact our revenues and expenses. If the USD were to weaken against another currency (as has been the case against the Euro in 2025), 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 individuals, entities, and their intermediaries from directly or indirectly making, offering, providing, promising, or authorizing provision of 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.

Global trade issues and changes in and uncertainties with respect to trade policies and export regulations, including import and export license requirements, trade sanctions, tariffs and international trade disputes, could adversely impact our business and operations, and reduce the competitiveness of our products and services relative to local and global competitors.

We operate in a global economy, and our business depends on a global supply chain for the development, manufacturing, and distribution of our pharmaceutical products, and for the advancement of our preclinical and clinical development programs. There is inherent risk, based on the complex relationships among the United States and the countries in which we conduct our business, that political, diplomatic, and national security factors can lead to global trade restrictions and changes in trade policies and export regulations that may adversely affect our business and operations. The current international trade and regulatory environment is subject to significant ongoing uncertainty.

The ongoing trade tensions between the United States and other jurisdictions have resulted in multiple rounds of tariffs and anticipated tariffs affecting pharmaceuticals and pharmaceutical ingredients, including finished drug products, manufacturing equipment, and related supplies. Such tariffs may significantly increase our costs for certain products. The Bureau of Industry and Security, U.S. Department of Commerce, has initiated an investigation to determine whether pharmaceutical ingredients, including finished drug product, manufactured outside the United States pose a national security risk and should be subject to additional tariffs. Unlike consumer goods, pharmaceuticals face unique regulatory constraints that make rapid supply chain adjustments particularly difficult and costly. Since we conduct manufacturing and packaging operations in Ireland for certain of our products and also engage contract manufacturers around the world, the import of our products into the United States is subject to tariffs. Notwithstanding the U.S. Supreme Court's recent decision invalidating tariffs imposed under the International Emergency Economic Powers Act, the magnitude and impact of tariffs are uncertain and are subject to a variety of factors, including the effective date and duration of additional tariffs, changes in the amount, scope and nature of tariffs in the future, including as a result of litigation or other challenges, any retaliatory tariffs that other countries may impose in response to tariffs levied by the United States and any mitigating actions that may become available. Trade restrictions and export regulations, or increases in tariffs and additional taxes, including any retaliatory measures, could negatively impact demand, increase our supply chain complexity and our manufacturing costs, decrease margins, reduce the competitiveness of our products, or restrict our ability to sell products, provide services or purchase necessary equipment and supplies, any or all of which could have a material and adverse effect on our business, results of operations, or financial condition. In addition, the dynamic and unpredictable tariff and trade landscape creates substantial uncertainty and significant planning challenges for our operations and complicates our long-term investment decisions regarding manufacturing facilities, supply chain optimization, and research and development locations.

Current or future tariffs could result in increased research and development expenses, including with respect to increased costs associated with APIs, raw materials, laboratory equipment and research materials and components. Trade restrictions affecting the import of materials necessary for clinical trials could result in delays to our development timelines. Increased development costs and extended development timelines could place us at a competitive disadvantage compared to companies operating in regions with more favorable trade relationships and could reduce investor confidence and negatively impact our business, results of operations, financial condition and growth prospects.

The complexity of announced or future tariffs may also increase the risk that we or our customers or suppliers may be subject to civil or criminal enforcement actions in the United States or foreign jurisdictions related to compliance with trade regulations. Foreign governments may also adopt non-tariff measures, such as procurement preferences or informal disincentives to engage with, purchase from or invest in U.S. entities, which may limit our ability to compete internationally and attract non-U.S. investment, employees, customers and suppliers. Foreign governments may also take other retaliatory actions against U.S. entities, such as decreased intellectual property protection, increased enforcement actions, or delays in regulatory approvals, which may result in heightened international legal and operational risks. In the event foreign governments impose tariffs or other retaliatory measures against U.S. entities, our ability to pass increased costs to customers in such foreign jurisdictions may be

limited by the structure of pharmaceutical pricing and reimbursement systems and how any such tariffs were implemented in such countries. In addition, the United States and other governments have imposed and may continue to impose additional sanctions, such as trade restrictions or trade barriers, which could restrict us from doing business directly or indirectly in or with certain countries or parties and may impose additional costs and complexity to our business.

Trade disputes, tariffs, restrictions and other political tensions between the United States and other countries may also exacerbate unfavorable macroeconomic conditions including inflationary pressures, foreign exchange volatility, financial market instability, and economic recessions or downturns. The ultimate impact of current or future tariffs and trade restrictions remains uncertain and could materially and adversely affect our business, financial condition, and prospects. While we actively monitor these risks, any prolonged economic downturn, escalation in trade tensions, or deterioration in international perception of U.S.-based companies could materially and adversely affect our business, ability to access the capital markets or other financing sources, results of operations, financial condition and prospects. In addition, tariffs and other trade developments have and may continue to heighten the risks related to the other risk factors described elsewhere in this report.

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. 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.

In addition, we have pursued, and may in the future pursue, litigation, administrative challenges or other types of proceedings to protect our patents and intellectual property rights. Such proceedings are often protracted and expensive, and have an unpredictable outcomes, which can include the initiation of defensive proceedings against us in retaliation.

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. Additionally, if our employees, consultants or contractors use generative artificial intelligence (AI) technologies to develop our proprietary technology and compounds, it may impact our ability to obtain or successfully defend certain intellectual property rights. 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 or other third parties have in the past and may in the future interfere with our patent process in a variety of ways. Competitors or other third parties may claim that they have an ownership interest in our patents, 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 or other third parties may also claim that we are infringing on their patents and therefore we cannot practice our technology. Competitors or other third parties 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, any such party 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 and other third parties continually seek intellectual property protection for their technology. Several of our products 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 other third parties or that we will not infringe intellectual property rights of competitors or other third parties 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 others 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 has fluctuated, 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;
- strategic transactions, including acquisition of products, businesses, or other assets;
- generic competition from current and 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;
- the impact of new or increased tariffs and escalating trade tensions;
- 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;
- industry, financial analyst, or investor reaction to public announcements by us or our competitors; 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. 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 2027 Notes will dilute the ownership interest of existing stockholders, including holders who had previously converted their 2027 Notes, or may otherwise depress the price of our common stock.

The conversion of some or all of the 2027 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 2027 Notes may encourage short selling by market participants because the

conversion of the 2027 Notes could be used to satisfy short positions, or anticipated conversion of the 2027 Notes into shares of our common stock could depress the price of our common stock.

The fundamental change repurchase feature of the 2027 Notes and the change of control repurchase feature of the 2034 Notes may delay or prevent an otherwise beneficial attempt to take us over.

The terms of the 2027 Notes require us to offer to repurchase the 2027 Notes in the event of a fundamental change (as defined in the indenture governing the 2027 Notes) and the terms of the 2034 Notes require us to offer to repurchase the 2034 Notes in the event of a change of control (as defined in the indenture governing the 2034 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, as well as our ability to integrate employees who join us as the result of an acquisition or business combination. 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. Changes to our management team, organizational structure, and corporate strategy could cause retention and morale concerns among current employees, and may create 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. 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. We have in the past and may in the future announce reductions in our global workforce, which could lead to employee attrition beyond our intended reductions in force and adverse effects on employee morale, diversion of management attention, and adverse effects to our reputation as an employer, which could in turn make it more difficult for us to hire employees in the future. 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.

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, legislation referred to as the One Big Beautiful Bill (OBBB) Act enacted in 2025, along with prior U.S. federal tax reform legislation, enacted many significant changes to the U.S. taxation of business entities, including, among other changes, the imposition of minimum taxes and excise taxes under certain circumstances, changes to the taxation of income derived from international operations, changes in the deduction and amortization of research and development expenditures, and limitations on the deductibility of business interest. For tax years beginning after December 31, 2024, the OBBB Act restores the tax deductibility of domestic research and development expenses in the year incurred, which expenses had been required under prior legislation to be capitalized and subsequently amortized over five years. The OBBB Act did not change the tax treatment of expenses incurred in research and development activities conducted outside the United States, which expenses continue to be required to be capitalized and amortized over 15 years. We are evaluating the potential impacts this and other changes under the OBBB Act may have on our business. Future guidance from the Internal Revenue Service and other tax authorities with respect to any legislation may affect us, and certain aspects of such legislation could be repealed or modified in subsequent legislation or sunset in future years. 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 the implementation of proposals, commonly referred to as "BEPS 2.0", which have made (and are expected to continue to make) important changes to the international tax system. These proposals include, among other measures, the imposition of 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. A number of countries in which we conduct business have enacted, or are in the process of enacting, core elements of Pillar Two rules (with further provisions expected to be enacted in the future). The OECD has issued (and is expected to continue to issue further) administrative guidance providing transition and safe harbor rules in relation to the implementation of the Pillar Two proposal. For example, on January 5, 2026, the OECD published details of a proposed "side-by-side" arrangement providing for, among other things, additional safe harbors for multinational groups headquartered in certain qualifying jurisdictions, which

includes the U.S. Based on the applicable thresholds, we are within the scope of the Pillar Two rules, but do not currently expect such rules to have a material adverse impact on our effective tax rate. 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. We continue to monitor developments and are evaluating the potential impacts of the Pillar Two rules, including on our effective tax rates, and considering our eligibility to qualify for the relevant safe harbor rules (including under the proposed "side-by-side" arrangement).

Our ability to use net operating losses to offset future taxable income may be subject to certain limitations.

Under the provisions of the Internal Revenue Code of 1986, as amended (the Internal Revenue Code), changes in our ownership may limit the amount of pre-change net operating loss carryforwards (NOLs) that can be utilized annually in the future to offset taxable income. Section 382 of the Internal Revenue Code imposes limitations on a company's ability to use its NOLs to offset its taxable income if one or more stockholders or groups of stockholders that each own at least five percent of the company's stock increase their aggregate ownership (by value) by more than 50 percentage points over their lowest ownership percentages within a rolling three-year period. Similar rules may apply under state and foreign tax laws. Thus, changes in our ownership may limit our ability to use our NOLs. Subsequent statutory or regulatory changes in respect of the utilization of NOLs for federal, state or foreign purposes, such as suspensions on the use of NOLs or limitations on the deductibility of NOLs carried forward, or other unforeseen reasons, may result in our existing NOLs expiring or otherwise being unavailable to offset future income tax liabilities. For these reasons, we may not be able to utilize a material portion of our NOLs.

If we are found in violation of healthcare 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. As first disclosed in our Annual Report on Form 10-K for the year ended December 31, 2023, we 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), together with the California Privacy Rights Act (CPRA), requires covered businesses that process personal data of California residents to disclose their data collection, use and sharing practices. Further, the CCPA provides California residents with data privacy rights (including the ability to opt out of the sale of personal data), imposes new operational requirements for covered businesses, and 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 CPRA, among other things, gives California residents the ability to limit use of certain sensitive personal data, establishes restrictions on the retention of personal data, expands the types of data breaches subject to the CCPA's private right of action, and establishes a new California Privacy Protection Agency to implement and enforce the new law.

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 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 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.

We, and the third parties with whom we work, are subject to stringent and evolving U.S. and foreign laws, regulations and rules, contractual obligations, industry standards, policies and other obligations related to data privacy and security. Actual or perceived failure to comply with such obligations by us or the third parties with whom we work could lead to regulatory investigations or actions, litigation, fines and penalties, disruptions of our business operations, reputational harm, loss of revenue or profits, and other adverse business consequences.

In the ordinary course of business, we collect, receive, store, process, generate, use, transfer, disclose, make accessible, protect, secure, dispose of, transmit, and share (collectively, process) personal data and other sensitive information, including proprietary and confidential business data, trade secrets, intellectual property, data we collect about trial participants in connection with clinical trials, sensitive third-party data, business plans, transactions, financial information and medical information collected by our patient access management team (collectively, sensitive data). Our data processing activities subject us to certain data privacy and security obligations, such as various laws, regulations, guidance, industry standards, external and internal privacy and security policies, contractual requirements, and other obligations relating to data privacy and security.

In the United States, federal, state, and local governments have enacted numerous data privacy and security laws, including data breach notification laws, personal data privacy laws, consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act), and other similar laws (e.g., wiretapping laws). For example, HIPAA, as amended by HITECH, imposes specific requirements relating to the privacy, security, and transmission of individually identifiable health information. Additionally, numerous

U.S. states have enacted comprehensive privacy laws that impose certain obligations on covered businesses, including providing specific disclosures in privacy notices and affording residents with certain rights concerning their personal data. As applicable, such rights may include the right to access, correct, or delete certain personal data, and to opt-out of certain data processing activities, such as targeted advertising, profiling, and automated decision-making. The exercise of these rights may impact our business and ability to provide our products and services. Certain states also impose stricter requirements for processing certain personal data, including sensitive information, such as conducting data privacy impact assessments. These state laws allow for statutory fines for noncompliance. For example, the CCPA applies to personal data of consumers, business representatives, and employees who are California residents and requires businesses to provide specific disclosures in privacy notices and honor requests of California residents to exercise certain privacy rights. The CCPA provides for fines for intentional violations and allows private litigants affected by certain data breaches to recover significant statutory damages. Although some U.S. comprehensive privacy laws exempt some data processed in the context of clinical trials, these laws may increase compliance costs and potential liability with respect to other personal data we may maintain about California residents. Similar laws are being considered in several other states, as well as at the federal and local levels, and we expect more jurisdictions to pass similar laws in the future.

Outside the United States, an increasing number of laws, regulations, and industry standards may govern data privacy and security. For example, the European Union's General Data Protection Regulation (EU GDPR), United Kingdom's GDPR (UK GDPR) (collectively, the GDPR), Brazil's General Data Protection Law (Lei Geral de Proteção de Dados Pessoais, or LGPD) (Law No. 13,709/2018), and China's Personal Information Protection Law (PIPL) impose strict requirements for processing personal data. For example, under the GDPR, companies may face temporary or definitive bans on data processing and other corrective actions; fines of up to 20 million Euros under the EU GDPR / 17.5 million pounds sterling under the UK GDPR or 4% of annual global revenue, whichever is greater; or private litigation related to processing of personal data brought by classes of data subjects or consumer protection organizations authorized at law to represent their interests.

In addition, we may be unable to transfer personal data from Europe and other jurisdictions to the United States or other countries due to data localization requirements or limitations on cross-border data flows. Europe and other jurisdictions have enacted laws requiring data to be localized or limiting the transfer of personal data to other countries. In particular, the European Economic Area (EEA) and the UK have significantly restricted the transfer of personal data to the United States and other countries whose privacy laws it believes are inadequate. Other jurisdictions have adopted may adopt similarly stringent data localization and cross-border data transfer laws. Although there are currently various mechanisms that may be used to transfer personal data from the EEA and UK to the United States in compliance with law, such as the EEA standard contractual clauses, the UK's International Data Transfer Agreement / Addendum, and the EU-U.S. Data Privacy Framework and the UK extension thereto (which allows for transfers to relevant U.S.-based organizations who self-certify compliance and participate in the Framework), these mechanisms are subject to legal challenges, and there is no assurance that we can satisfy or rely on these measures to lawfully transfer personal data to the United States. If there is no lawful manner for us to transfer personal data from the EEA, the UK, or other jurisdictions to the United States, or if the requirements for a legally-compliant transfer are too onerous, we could face significant adverse consequences, including by limiting our ability to conduct clinical trial activities in Europe and elsewhere, the interruption or degradation of our operations, the need to relocate part of or all of our business or data processing activities to other jurisdictions (such as Europe) at significant expense, increased exposure to regulatory actions, substantial fines and penalties, the inability to transfer data and work with partners, vendors and other third parties, and injunctions against our processing or transferring of personal data necessary to operate our business. Some European regulators have ordered certain companies to suspend or permanently cease certain transfers of personal data to recipients outside Europe for allegedly violating the GDPR's cross-border data transfer limitations. Additionally, companies that transfer personal data to recipients outside of the EEA and/or UK to other jurisdictions, particularly to the United States, are subject to increased scrutiny from regulators individual litigants and activist groups.

Additionally, the U.S. Department of Justice issued a rule entitled the Preventing Access to U.S. Sensitive Personal Data and Government-Related Data by Countries of Concern or Covered Persons, which places additional restriction on certain data transactions involving countries of concern (e.g., China, Russia, Iran) and covered persons that may impact certain business activities such as vendor engagements, sale or sharing of data, employment of certain individuals, and investor agreements. Violations of the rule could lead to significant civil and criminal fines and penalties. The rule applies regardless of whether data is anonymized, key-coded, pseudonymized, de-identified or encrypted, which presents particular challenges for companies like ours and may impact our ability to transfer data in connection with certain transactions or agreements.

We are subject to new laws governing the privacy of consumer health data, including reproductive, sexual orientation, and gender identity privacy rights that provide consumers certain rights with respect to their health data and create a private right of action to allow individuals to sue for violations.

Our employees and personnel, as well as third parties with whom we work, use, or may in the future use, generative AI technologies to perform their work, and the disclosure and use of personal data in generative AI technologies is subject to various privacy laws and other privacy obligations. Governments have passed and are likely to pass additional laws regulating generative AI. Our use of this technology could result in additional compliance costs, regulatory investigations and actions, and lawsuits. If these factors limit or otherwise impair our effective use of generative AI, it could make our business less efficient and result in competitive disadvantages.

In addition to data privacy and security laws, we are contractually subject to industry standards adopted by industry groups and may become subject to additional such obligations in the future. We are also bound by other contractual obligations related to data privacy and security, and our efforts to comply with such obligations may not be successful. We publish privacy policies, marketing materials, and other statements, such as statements related to compliance with certain certifications or self-regulatory principles, regarding data privacy and security. Regulators across the world are increasingly scrutinizing these statements, and if these policies, materials or statements are found to be deficient, lacking in transparency, deceptive, unfair, misleading, or misrepresentative of our practices, we may be subject to investigation, enforcement actions by regulators, or other adverse consequences.

Obligations related to data privacy and security (and consumers' data privacy expectations) are quickly changing, becoming increasingly stringent, and creating uncertainty. Additionally, these obligations may be subject to differing applications and interpretations, which may be inconsistent or conflict among jurisdictions. Preparing for and complying with these obligations requires us to devote significant resources and may necessitate changes to our services, information technologies, systems, and practices and to those of any third parties that process personal data on our behalf.

We may at times fail (or be perceived to have failed) in our efforts to comply with our data privacy and security obligations. Moreover, despite our efforts, our personnel or third parties with whom we work may fail to comply with such obligations, which could negatively impact our business operations. If we or the third parties with whom we work fail, or are perceived to have failed, to address or comply with applicable data privacy and security obligations, we could face significant consequences, including but not limited to: government enforcement actions (e.g., investigations, fines, penalties, audits, inspections, and similar); litigation (including class-action claims) and mass arbitration demands; additional reporting requirements and/or oversight; bans or restrictions on processing personal data; and orders to destroy or not use personal data. In particular, plaintiffs have become increasingly more active in bringing privacy-related claims against companies, including class claims. Some of these claims allow for the recovery of statutory damages on a per violation basis, and, if viable, carry the potential for monumental statutory damages, depending on the volume of data and the number of violations. Any of these events could have a material adverse effect on our reputation, business, or financial condition, including but not limited to loss of customers; inability to process personal data or to operate in certain jurisdictions; interruptions or stoppages in our business operations (including clinical trials); limited ability to develop or commercialize our products; expenditure of time and resources to defend any claim or inquiry; adverse publicity; or substantial changes to our business model or 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 adopted and came into force in December 2024. These rules apply to products placed on the market or put into service as of December 9, 2026, and aims to make it easier for certain victims to claim damages for certain defective products, for example by alleviating their burden of proof.

We, and the third parties with whom we work, 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.

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 or interruptions associated with the implementation of new or upgraded technology systems or with maintenance or adequate support of existing systems could disrupt or reduce, and has in the past disrupted or reduced, the efficiency of our operations and expose us to greater risk of security breaches. Cybersecurity incidents resulting in the failure of our ERP 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. In particular, we have implemented certain modules of a new global ERP system and will continue to implement other components of the new system, which has replaced, and will continue to replace, legacy operating and financial systems. The preparation and implementation of a new ERP system has required, and will continue to require, significant investment of capital and human resources. Our results of operations could be adversely affected if we continue to 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.

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 on to operate our business, may be vulnerable to loss, damage, denial-of-service, unauthorized access or misappropriation. 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 unauthorized activity such as malware, hacking, business email compromise, phishing and other social engineering attacks (including deep fakes, which may be increasingly more difficult to identify as fake), ransomware or other cyberattacks. In particular, severe ransomware attacks are becoming increasingly prevalent and can lead to significant interruptions in our operations, ability to provide our products or services, loss of sensitive data and income, reputational harm, and diversion of funds. Extortion payments may alleviate the negative impact of a ransomware attack, but we may be unwilling or unable to make such payments due to, for example, applicable laws or regulations prohibiting such payments. Remote work poses increased risks to our information technology systems and data, as our employees utilize network connections, computers and devices outside our premises or network, including working at home, while in transit and in public locations.

Future or past business transactions (such as acquisitions or integrations) could expose us to additional cybersecurity risks and vulnerabilities, as our systems could be negatively affected by vulnerabilities present in acquired or integrated entities' systems and technologies. Furthermore, we may discover security issues that were not found during due diligence of such acquired or integrated entities, and it may be difficult to integrate companies into our information technology environment and security program.

While we have implemented measures to protect our information systems and data stored in those systems and those of the third parties with whom we work, our efforts may not be successful. It may also be difficult and/or costly to detect, investigate, mitigate, contain, and remediate a security incident. Our efforts to do so may not be successful. Actions taken by us or the third parties with whom we work to detect, investigate, mitigate, contain, and remediate a security incident could result in outages, data losses, and disruptions of our business.

We rely on third parties to operate critical business systems to process sensitive information in a variety of contexts, including, without limitation, cloud-based infrastructure, data center facilities, encryption and authentication technology, employee email, clinical trial functions, manufacturing partners, and other functions. Our ability to monitor these third parties' information security practices is limited, and these third parties may not have adequate information security measures in place. If the third parties with whom we work experience a security incident or other interruption, we could experience adverse consequences. While we may be entitled to damages if the third parties with whom we work fail to satisfy their privacy or security-related obligations to us, any award may be insufficient to cover our damages, or we may be unable to recover such award. Threat actors may also gain access to other networks and systems after a compromise of our networks and systems. For example, threat actors may use an initial compromise of one part of our environment to gain access to other parts of our environment, or leverage a compromise of our networks or systems to gain access to the networks or systems of third parties with whom we work, such as through phishing or supply chain attacks.

We take steps designed to detect, mitigate, and remediate vulnerabilities in our information systems (such as our hardware and/or software, including that of third parties with whom we work). We have not and may not in the future, however, detect and remediate all such vulnerabilities including on a timely basis. Further, we have and may in the future experience delays in developing and deploying remedial measures and patches designed to address identified vulnerabilities. Vulnerabilities could be exploited and result in a security incident.

Any of the previously identified or similar threats have in the past and may in the future cause a security incident or other interruption that have in the past and may in the future result in unauthorized, unlawful, or accidental acquisition, modification, destruction, loss, alteration, encryption, disclosure of, or access to our sensitive information or our information technology systems, or those of the third parties with whom we work. We have experienced and may continue to experience security 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.

In addition to experiencing a security incident, third parties may gather, collect, or infer sensitive information about us from public sources, data brokers, or other means that reveals competitively sensitive details about our organization and could be used to undermine our competitive advantage or market position.

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. For example, in December 2023, we entered into a Cooperation Agreement with Elliott Investment Management L.P., Elliott Associates, L.P. and Elliott International, L.P., which expired in December 2024 pursuant to the terms of the agreement. 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. 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.

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, wildfire, or other catastrophic disaster could cause damage to our facilities 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 VIMIZIM. This facility is 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, tariffs and escalating trade tensions, 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. 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.

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, and the third parties with whom we work, 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 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 our cybersecurity risk management processes, including oversight and mitigation of risks from cybersecurity threats. The Board and the Audit Committee receive periodic reports, summaries, and presentations from our senior management concerning our significant cybersecurity threats and risk and the processes the Company has implemented to address them.

We have an Executive Cybersecurity Committee (ECC), which is comprised of our Chief Financial Officer (CFO), Chief Digital and Information Officer, Chief Legal Officer and Chief Accounting Officer, 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 Chief Digital and Information Officer, 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 Chief Digital and Information Officer. 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 and 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 Digital and Information Officer, who reports to the CEO. Our Chief Digital and Information Officer joined us in January 2026, and has more than 20 years of experience in information technology and artificial intelligence in the biopharmaceutical industry.

Item 2. Properties

The following table contains information about our significant owned and leased properties as of December 31, 2025:

Location	Approximate Square Feet	Use	Lease Expiration Date
San Rafael facility, San Rafael, California	407,200	Corporate headquarters, laboratory and office	Owned property
Several facilities in Novato, California	292,300	Clinical and commercial manufacturing, laboratory and office	Owned property
Several leased facilities in Novato, California	149,500	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

None.

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.

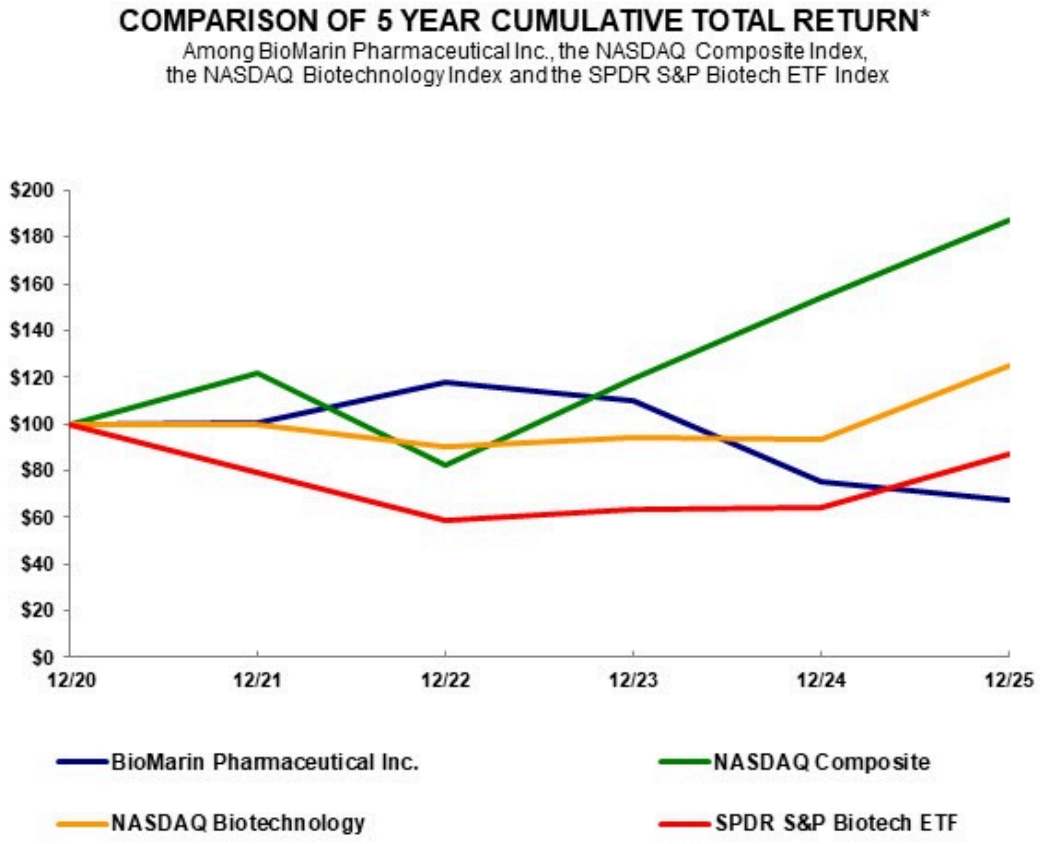
Holders

As of February 19, 2026, there were 28 holders of record of 192,323,359 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, 2020 in stock or index, including reinvestment of dividends

	2020	2021	2022	2023	2024	2025
BioMarin Pharmaceutical Inc.	$ 100.00	$ 100.75	$ 118.02	$ 109.96	$ 74.96	$ 67
Nasdaq Composite Index	$ 100.00	$ 122.18	$ 82.43	$ 119.22	$ 154.48	$ 187
Nasdaq Biotechnology	$ 100.00	$ 100.02	$ 89.90	$ 94.03	$ 93.49	$ 124
SPDR S&P Biotech ETF	$ 100.00	$ 79.55	$ 58.97	$ 63.45	$ 64.09	$ 87

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. The 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

We are a leading, global rare disease biotechnology company focused on delivering medicines for people living with genetically defined conditions. Our San Rafael, California-based company, founded in 1997, has a proven track record of innovation with eight commercial therapies and a strong clinical and preclinical pipeline. Using a distinctive approach to drug discovery and development, we seek to unleash the full potential of genetic science by pursuing category-defining medicines that have a profound impact on patients. A summary of our commercial products, as of December 31, 2025, is provided below:

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

2025 Financial Highlights

Key components of our results of operations include the following:

	Twelve Months Ended December 31,		
	2025	2024	2023
Total revenues	$ 3,221.3	$ 2,853.9	$ 2,419.2
Cost of sales	$ 717.4	$ 580.2	$ 532.1
Research and development (R&D)	$ 921.9	$ 747.2	$ 746.8
Selling, general and administrative (SG&A)	$ 1,153.0	$ 1,009.0	$ 892.4
Provision for income taxes	$ 133.6	$ 114.9	$ 20.9
Net income	$ 348.9	$ 426.9	$ 167.6

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, geopolitical instability, impact of new or increased tariffs and escalating trade tensions, regulatory uncertainty, 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

In 2025, we achieved $3.2 billion in total revenues, including a significant contribution from our ongoing expansion of VOXZOGO, and we continued to grow our commercial business and advance our product candidate pipeline. We believe that the combination of our internal research programs and partnerships and acquisitions of external assets will allow us to continue to develop and commercialize innovative therapies for people with serious and life-threatening rare diseases and medical conditions. We periodically conduct strategic portfolio assessment of research and development programs to determine which we believe have the strongest combination of scientific merit, opportunity for commercial success and potential value creation for stockholders. Based on such strategic portfolio assessments, certain programs that do not meet its threshold for further development and commercialization could be discontinued.

In December 2025, we entered into a definitive agreement to acquire Amicus Therapeutics, Inc. (Amicus), a publicly traded, global, biotechnology company for $14.50 per share in an all-cash transaction for a total consideration of approximately $4.8 billion. The pending acquisition is expected to strengthen our commercial portfolio by adding two new therapies for the treatment of Fabry disease and late-onset Pompe disease. The transaction is expected to close in the second quarter of 2026, subject to regulatory clearances, approval by the stockholders of Amicus and other customary closing conditions. We intend to finance the transaction through a combination of cash on hand and approximately $3.7 billion of non-convertible debt financing.

In December 2025, we entered into a debt financing commitment letter (the Commitment Letter) and related fee letter with certain lenders, pursuant to which the lenders have committed to provide us with debt financing up to approximately $3.7 billion (the Bridge Commitment) in the form of a 364-day senior secured bridge loan facility (Bridge Facility) for the pending acquisition of Amicus. No amounts had been drawn or were outstanding under the Bridge Commitment as of December 31, 2025. In February 2026, we issued $850.0 million in aggregate principal amount of 5.5% senior unsecured notes due 2034 (the 2034 Notes), and the proceeds from the issuance were deposited into an escrow account that will be used to finance the pending acquisition of Amicus. In connection with the issuance of the 2034 Notes, the Bridge Commitment was reduced from approximately $3.7 billion to $2.8 billion. In place of the Bridge Facility, we also expect to enter into a senior secured term loan facility for approximately $2.8 billion in aggregate principal and a new $600.0 million senior secured revolving credit facility in 2026 that will be executed prior to or concurrently with the closing of the pending Amicus acquisition. See "Financial Condition, Liquidity and Capital Resources" below for additional information.

In October 2025, we announced our plan to pursue options to divest ROCTAVIAN, including exploring out-licensing opportunities. Subsequently in December 2025, we committed to a plan to voluntarily withdraw ROCTAVIAN from the market due to lower than previously anticipated commercial opportunities. In connection with this strategic decision, we recorded approximately $240.0 million of restructuring charges in 2025 comprised of an inventory write-off, impairment of long-lived assets, severance and other costs. See Note 19 to our accompanying Consolidated Financial Statements for additional details.

In July 2025, we completed the acquisition of Inozyme Pharma, Inc. (Inozyme), a publicly traded clinical-stage biopharmaceutical company dedicated to developing innovative therapeutics. The acquisition is intended to strengthen our enzyme therapies portfolio by adding a late-stage enzyme replacement therapy, BMN 401 (formerly INZ-701), for the treatment of ectonucleotide pyrophosphatase/phosphodiesterase 1 (ENPP1) deficiency. We accounted for this transaction as an asset acquisition since the lead asset, BMN 401, represents substantially all of the fair value of the gross assets acquired. See Note 20 to our accompanying Consolidated Financial Statements for additional information related to Inozyme acquisition.

(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,				
	2025	2024	2023	2025 vs. 2024	2024 vs. 2023
VOXZOGO	$ 926.9	$ 735.1	$ 469.9	$ 191.8	$ 265.2
Enzyme Therapies:					
VIMIZIM	792.1	739.8	701.0	52.3	38.8
NAGLAZYME	485.4	479.6	420.3	5.8	59.3
PALYNZIQ	433.3	355.0	303.9	78.3	51.1
ALDURAZYME	208.5	183.9	131.2	24.6	52.7
BRINEURA	186.4	169.1	161.9	17.3	7.2
KUVAN	99.6	120.9	180.8	(21.3)	(59.9)
ROCTAVIAN	35.6	26.0	3.5	9.6	22.5
Total net product revenues	$ 3,167.8	$ 2,809.4	$ 2,372.5	$ 358.4	$ 436.9

Net Product Revenues include revenues generated from our commercial products. In the U.S., our 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 program, and ALDURAZYME is marketed worldwide by Sanofi. Outside the U.S., our commercial products are sold to authorized distributors or directly to government purchasers or hospitals, which act as the end users.

The increase in Net Product Revenues in 2025 as compared to 2024 was primarily attributed to the following:

- VOXZOGO: higher sales volume from new patients initiating therapy across all regions;
- PALYNZIQ: higher sales volume from new patients initiating therapy, primarily in the U.S.;
- VIMIZIM: higher sales volume due to timing of orders in countries that place large government orders, primarily from countries in the Middle East and Latin America;
- ALDURAZYME: higher sales volume due to timing of order fulfillment to Sanofi as we recognize ALDURAZYME revenues when the product is released and control is transferred to Sanofi; and
- BRINEURA: higher sales volume from new patients initiating therapy, primarily in the U.S. and Latin America.

These increases were partially offset by the following:

- KUVAN: lower product revenues attributed to increasing generic competition as a result of the loss of market exclusivity.

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 in the past and may continue to create significant period to period variation in our revenues.

With respect to VOXZOGO, see also the risk factor "Our success depends on our ability to manage our growth and execute our corporate strategy." in "Risk Factors" included in Part I, Item 1A of this Annual Report for additional information on risk factors that could impact our business and operations.

We face exposure to movements in foreign currency exchange rates, and use foreign currency exchange forward contracts to hedge a percentage of our foreign currency exposure, primarily the Euro. Certain currencies are not included in our hedging program, such as the Argentine Peso. With respect to the risks posed by fluctuations of both hedged and unhedged currencies against the USD, see the risk factor "Our international operations pose currency risks, which may adversely affect our

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

operating results and net income" in "Risk Factors" included in Part I, Item 1A of this Annual Report for additional information. The following table shows our Net Product Revenues denominated in USD and foreign currencies:

	Twelve Months Ended December 31,				
	2025	2024	2023	2025 vs. 2024	2024 vs. 2023
Sales denominated in USD	$ 1,604.2	$ 1,366.0	$ 1,137.8	$ 238.2	$ 228.2
Sales denominated in foreign currencies	1,563.6	1,443.4	1,234.7	120.2	208.7
Total net product revenues	$ 3,167.8	$ 2,809.4	$ 2,372.5	$ 358.4	$ 436.9

	Twelve Months Ended December 31,				
	2025	2024	2023	2025 vs. 2024	2024 vs. 2023
Unfavorable impact of foreign currency exchange rates on product sales denominated in currencies other than USD	$ (40.7)	$ (107.8)	$ (100.0)	$ 67.1	$ (7.8)

The unfavorable impact of foreign currency exchange rates on USD reported results in 2025 was primarily driven by weakening of the Argentine Peso, Brazilian Real and Mexican Peso, partially offset by strengthening of the Euro. The unfavorable impact of foreign currency exchange rates on USD reported results in 2024 was primarily driven by weakening of the Argentine Peso and the Japanese Yen.

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 results 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.

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 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, idle plant costs and charges for inventory write downs.

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

	Twelve Months Ended December 31,				
	2025	2024	2023	2025 vs. 2024	2024 vs. 2023
Total revenues	$ 3,221.3	$ 2,853.9	$ 2,419.2	$ 367.4	$ 434.7
Cost of sales	$ 717.4	$ 580.2	$ 532.1	$ 137.2	$ 48.1
Gross margin	77.7 %	79.7 %	78.0 %	(2.0)%	1.7 %

Cost of Sales increased in 2025 compared to 2024 primarily due to $119.2 million write-off of ROCTAVIAN inventory as a result of our strategic decision in the fourth quarter of 2025 to voluntarily withdraw ROCTAVIAN from the market. Gross margin decreased in 2025 compared to 2024 primarily due to ROCTAVIAN inventory write-off, partially offset by increased sales volume of higher-margin products within our Enzyme Therapies portfolio.

Research and Development

R&D expense includes costs associated with the research and development of product candidates and post-marketing research commitments related to our commercial products. R&D expense primarily includes preclinical and clinical studies, personnel and raw materials costs associated with manufacturing clinical product, quality control and assurance, other R&D activities, R&D facilities and regulatory costs.

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

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 probable 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.

R&D expense consisted of the following:

	Twelve Months Ended December 31,				
	2025	2024	2023	2025 vs. 2024	2024 vs. 2023
Research and early pipeline	$ 383.7	$ 434.0	$ 393.1	$ (50.3)	$ 40.9
Later-stage clinical programs	308.3	27.6	62.6	280.7	(35.0)
Marketed products	229.9	285.6	291.1	(55.7)	(5.5)
Total R&D expense	$ 921.9	$ 747.2	$ 746.8	$ 174.7	$ 0.4

R&D expense increased in 2025 compared to 2024 primarily due to higher spend on Later-stage clinical programs as a result of the $221.0 million In-Process Research and Development (IPR&D) charge following the Inozyme acquisition and continued progression of VOXZOGO for hypochondroplasia. These increases were partially offset by lower spend on Research and early pipeline due to discontinued programs and lower spend on Marketed products mainly related to ROCTAVIAN.

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.

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

SG&A expenses consisted of the following:

	Twelve Months Ended December 31,				
	2025	2024	2023	2025 vs. 2024	2024 vs. 2023
S&M expense	$ 530.2	$ 476.7	$ 488.4	$ 53.5	$ (11.7)
G&A expense	622.8	532.3	404.0	90.5	128.3
Total SG&A expense	$ 1,153.0	$ 1,009.0	$ 892.4	$ 144.0	$ 116.6

S&M expenses by product were as follows:

	Twelve Months Ended December 31,				
	2025	2024	2023	2025 vs. 2024	2024 vs. 2023
Enzyme Therapies	$ 247.1	$ 219.0	$ 225.3	$ 28.1	$ (6.3)
VOXZOGO	184.2	134.1	108.9	50.1	25.2
ROCTAVIAN	30.7	76.7	104.5	(46.0)	(27.8)
Other	68.2	46.9	49.7	21.3	(2.8)
Total S&M expense	$ 530.2	$ 476.7	$ 488.4	$ 53.5	$ (11.7)

The increase in S&M expense for 2025 compared to 2024 was primarily due to increased spending related to global expansion of VOXZOGO for achondroplasia and pre-launch activities on VOXZOGO for hypochondroplasia, and higher spend on demand generating activities for Enzyme Therapies. These increases in S&M spend were partially offset by reduced activities related to ROCTAVIAN as we focused on our commercial efforts in the U.S., Germany and Italy in 2025.

The increase in G&A expense for 2025 compared to 2024 was primarily due to $118.5 million of restructuring charges related to impairment of long-lived assets as a result of our strategic decision in the fourth quarter of 2025 to voluntarily withdraw ROCTAVIAN from the market, and partially due to incremental administrative costs related to ongoing support of business initiatives during the year. These increases were partially offset by bad debt expense recorded in the fourth quarter of 2024 that did not recur in 2025.

Intangible Asset Amortization and Gain on Sale of Nonfinancial Assets

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

	Twelve Months Ended December 31,				
	2025	2024	2023	2025 vs. 2024	2024 vs. 2023
Amortization of intangible assets	$ 19.4	$ 43.3	$ 62.2	$ (23.9)	$ (18.9)
Gain on sale of nonfinancial assets	$ —	$ 10.0	$ —	$ (10.0)	$ 10.0

Amortization of intangible assets: the decrease in amortization expense for 2025 as compared to 2024 was due to the increase in the estimated useful life of an intangible asset as a result of the extension of a patent during the second half of 2024 and an intangible asset becoming fully amortized during the fourth quarter of 2024.

Gain on Sale of Nonfinancial Assets: in the first quarter of 2024, we recognized a gain of $10.0 million due to a third party's achievement of a regulatory approval milestone related to previously sold intangible assets.

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.

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

	Twelve Months Ended December 31,				
	2025	2024	2023	2025 vs. 2024	2024 vs. 2023
Interest income	$ 74.9	$ 74.9	$ 58.3	$ —	$ 16.6

Interest Income during 2025 compared to 2024 was relatively flat. We expect Interest Income to decrease over the next 12 months due to lower cash and investment balances as the pending Amicus acquisition will be financed through a combination of cash on hand and non-convertible debt financing.

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,				
	2025	2024	2023	2025 vs. 2024	2024 vs. 2023
Interest expense	$ 10.9	$ 12.7	$ 17.3	$ (1.8)	$ (4.6)

Interest Expense decreased in 2025 as compared to 2024 primarily due to settlement of 2024 Notes that matured in August 2024. We expect Interest Expense to increase over the next 12 months due to financing related to the pending Amicus acquisition, including the 2034 Notes issued on February 12, 2026. See Note 20 to our accompanying Consolidated Financial Statements for additional information regarding financing related to the pending acquisition of Amicus.

Other Income (Expense), Net

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

	Twelve Months Ended December 31,				
	2025	2024	2023	2025 vs. 2024	2024 vs. 2023
Other income (expense), net	$ 9.0	$ (4.7)	$ (38.2)	$ 13.7	$ 33.5

The increase in Other Income (Expense), Net, in 2025 compared to 2024 was primarily due to proceeds from insurance related to damaged goods.

Provision for Income Taxes

Provision for Income Taxes for the periods presented was as follows:

	Twelve Months Ended December 31,				
	2025	2024	2023	2025 vs. 2024	2024 vs. 2023
Provision for income taxes	$ 133.6	$ 114.9	$ 20.9	$ 18.7	$ 94.0

Our Provision for Income Taxes in 2025 and 2024 consisted of state, federal and foreign current tax expense which was offset by foreign tax credits, and deferred tax benefits from federal orphan drug credits and federal R&D credits. In July 2025, the One Big Beautiful Bill (OBBB Act) was signed into law in the U.S. This legislation includes a broad range of U.S. tax reforms provisions which become effective through 2027. Those effective in 2025 are reflected in our 2025 results. The Provision for Income Taxes in 2025 increased compared to 2024, primarily due to non-deductible acquired IPR&D related to the Inozyme acquisition, tax expense related to the expiration of unexercised options, partially offset by reduction in tax expense as a result of the OBBB Act.

Certain countries in which we have operations, including Ireland, have adopted Pillar Two framework, recently released from the Organisation for Economic Co-operation and Development (OECD), including a minimum tax rate of 15%. The U.S. has not enacted legislation to adopt the Pillar Two framework. The adoption of the Pillar Two framework did not have a material impact

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

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 2024 Compared to 2023

For a discussion of our results of operations pertaining to 2024 as compared to 2023 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, 2024 (filed with the Securities and Exchange Commission (SEC) on February 24, 2025).

Financial Condition, Liquidity and Capital Resources

Our cash, cash equivalents, and investments as of December 31, 2025 and 2024 were as follows:

	2025	2024	2025 vs. 2024
Cash and cash equivalents	$ 1,311.7	$ 942.8	$ 368.9
Short-term investments	248.9	194.9	54.0
Long-term investments	492.2	521.2	(29.0)
Total cash, cash equivalents and investments	$ 2,052.8	$ 1,658.9	$ 393.9

We believe cash generated from sales of our commercial products, in addition to our cash, cash equivalents and short-term investments, including proceeds from the 2034 Notes and external financings, will be sufficient to satisfy our liquidity requirements for at least the next 12 months, including our agreement to acquire Amicus in an all-cash transaction. 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 long-term investment balances. We will need to raise additional funds by issuing equity, debt or convertible securities, taking loans or entering into collaborative or other agreements if we are unable to satisfy our liquidity requirements. For example, we may require additional financing to fund the repayment of our outstanding indebtedness, 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. In addition, depending on prevailing market conditions, our liquidity requirements, contractual restrictions, and other factors, we may also from time to time seek to retire or purchase our outstanding debt through cash purchases and/or exchanges for equity securities, in open market purchases, privately negotiated transactions or otherwise.

We are mindful that conditions in the current macroeconomic environment, such as inflation, changes in interest and foreign currency exchange rates, natural disasters, geopolitical instability, impact of new or increased tariffs and escalating trade tensions, regulatory uncertainty, 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, 2025 and 2024 were as follows:

	2025	2024	2025 vs. 2024
Net cash provided by operating activities	$ 828.0	$ 572.8	$ 255.2
Net cash provided by (used in) investing activities	$ (414.2)	$ 136.5	$ (550.7)
Net cash used in financing activities	$ (42.4)	$ (526.4)	$ 484.0

The increase in net cash provided by operating activities in 2025 compared to 2024 was attributed to increase in net income adjusted for non-cash items mainly related to inventory write-off and asset impairments for ROCTAVIAN and IPR&D charges from Inozyme acquisition, and partially due to the timing of payments to vendors and cash receipts from our customers.

The increase in net cash used in investing activities in 2025 compared to 2024 was primarily attributable to $285.2 million net cash paid for the acquisition of Inozyme, lower net maturities of available-for-sale securities, and higher purchases of property, plant and equipment.

The decrease in net cash used in financing activities in 2025 compared to 2024 was primarily due to $495.0 million settlement of the 2024 Notes that matured in August 2024. The decrease was also attributable to lower proceeds from exercises of equity awards in 2025.

Financing and Credit Facilities

In December 2025, in connection with the pending acquisition of Amicus, we entered into the Commitment Letter and related fee letter with certain lenders, pursuant to which the lenders have committed to the Bridge Commitment, to provide us with debt financing in an aggregate principal amount of up to approximately $3.7 billion in the form of the 364-day senior secured Bridge Facility, subject to customary conditions and entry into definitive financing and ancillary documentation as set forth therein. No amounts had been drawn or were outstanding under the Bridge Commitment as of December 31, 2025.

In February 2026, we issued $850.0 million in aggregate principal amount of the 2034 Notes, and the proceeds from the issuance were deposited into an escrow account that will be used to finance the pending acquisition of Amicus. In connection with the issuance of the 2034 Notes, the Bridge Commitment was reduced from approximately $3.7 billion to $2.8 billion. In the event that the acquisition is not completed on or prior to December 19, 2026, or upon the occurrence of certain other events, we will be required to redeem all of the 2034 Notes at par and pay any accrued and unpaid interest. In place of the Bridge Facility, we also expect to enter into a senior secured term loan facility for approximately $2.8 billion in aggregate principal and a new $600.0 million senior secured revolving credit facility in 2026 (the New Revolving Facility) that will be executed prior to or concurrently with the closing of the pending Amicus acquisition. Upon entry into the senior secured term loan facility, the remaining Bridge Commitment will be reduced to zero. Under the New Revolving Facility, we may also borrow up to $150 million to pay fees and expenses related to the pending acquisition of Amicus.

Our $600.0 million (undiscounted) of total convertible debt as of December 31, 2025 consisting of our 1.25% senior subordinated convertible notes due in 2027 (the 2027 Notes), will impact our liquidity due to semi-annual cash interest payments and repayment of the principal amount in cash at maturity in May 2027 if not converted.

Our $600.0 million unsecured revolving credit facility (Revolving Facility) as of December 31, 2025 is intended to finance ongoing working capital needs and other general corporate initiatives. The Revolving Facility matures in August 2029 and contains financial covenants including a maximum total net leverage ratio and a minimum interest coverage ratio. As of December 31, 2025 there were no amounts outstanding under the Revolving Facility and we were in compliance with all covenants. The New Revolving Facility is expected to replace the existing Revolving Facility.

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, 2025, we had purchase obligations of approximately $590.8 million, of which $354.1 million is expected to be paid in 2026. 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.

As of December 31, 2025, we had lease payment obligations of $57.3 million, of which $10.3 million is payable in 2026. See Note 9 to our accompanying Consolidated Financial Statements for details on our lease liabilities.

Unrecognized Tax Benefits

As of December 31, 2025, our liability for unrecognized tax benefits was $380.9 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.

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

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 Income. 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.

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, 2025	$ 195.0	$ 487.8	$ (436.7)	$ 246.1
Year ended December 31, 2024	$ 152.1	$ 435.1	$ (392.2)	$ 195.0
Year ended December 31, 2023	$ 115.0	$ 370.7	$ (333.6)	$ 152.1

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.

Valuation of assets and liabilities in connection with acquisitions

We have acquired and continue to acquire intangible assets in connection with business combinations and asset acquisitions. These intangible assets consists primarily of IPR&D for product candidates. Discounted cash flow models are typically used to determine the fair value of acquired intangible assets for the purposes of allocating consideration paid to the net assets in an acquisition. These models require us to make certain judgments, which include:

- developing appropriate probability of success rates for unapproved product candidates considering their stages of development;
- estimating time and resources needed to complete the development and approval of the product;
- projecting time to approval;
- risks related to the viability of potential alternative treatments in future target markets;
- revenue projections;
- and discount rate.

The judgments made in determining estimated fair values assigned to assets acquired and liabilities assumed in an acquisition, as well as estimated asset lives, can materially affect our consolidated results of operations. The fair values of intangible assets, including acquired IPR&D, are determined using information available near the acquisition date based on estimates and assumptions that are deemed reasonable by management. Depending on the facts and circumstances, we may deem it necessary to engage an independent valuation expert to assist in valuing significant assets and liabilities. The fair values of identifiable intangible assets are primarily determined using the income method.

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 2025, approximately 49% of our net product sales were denominated in foreign currencies and 19% 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, 2025, we had open forward contracts with net notional amounts of $1.5 billion. A hypothetical 10% adverse movement in foreign currency exchange rates compared with the USD relative to exchange rates as of December 31, 2025 would have resulted in a reduction in the value received over the remaining life of these contracts by approximately $152.9 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, 2025, 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 $39.6 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.

As of December 31, 2025, 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, 2025, 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 $8.6 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 Income 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 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, 2025 (in millions of U.S. Dollars):

	Expected Maturity					
	2026	2027	2028	2029	2030	Total
Available-for-sale debt securities	$ 248.9	$ 257.2	$ 171.4	$ 47.5	$ 12.3	$ 741.2
Average interest rate	3.8 %	3.8 %	3.8 %	3.8 %	3.9 %	3.8 %

We have outstanding $600.0 million (undiscounted) of the 2027 Notes. The interest rate on the 2027 Notes is fixed and therefore does not expose us to risk related to rising interest rates. As of December 31, 2025, the fair value of our convertible debt was $576.3 million.

In February 2026, we issued $850.0 million aggregate principal amount of 5.5% senior unsecured notes due in 2034 (the 2034 Notes). The interest rate on the 2034 Notes is fixed and therefore does not expose us to risk related to rising interest rates. See Note 21 to our accompanying Consolidated Financial Statements for additional details.

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, 2025.

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, 2025. 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, 2025 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

Except as noted below, 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.

In January 2025, we began deploying a new global enterprise resource planning (ERP) system at certain subsidiaries, replacing existing operating and financial systems. The new ERP implementation is scheduled to occur in phases through 2026, with post-implementation activities following thereafter. In January 2026, the final phase of deployment was completed. 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 have updated our internal control over financial reporting, as necessary, to accommodate related changes in our financial management processes resulting from this implementation. As the implementation and post-implementation activities take place, we will continue to 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, 2025, none of our directors or officers (as defined in Rule 16a-1(f) under the Exchange Act) adopted or terminated any “Rule 10b5-1 trading arrangement” or “non-Rule 10b5-1 trading arrangement,” as those terms are defined in Item 408 of Regulation S-K.

Voluntary Withdrawal of ROCTAVIAN from Market

On December 17, 2025, we committed to a plan to voluntarily withdraw ROCTAVIAN from the market due to lower than previously anticipated commercial opportunities. In connection with this strategic decision, we recorded approximately $240.0

million of restructuring charges in 2025 comprised of an inventory write-off, impairment of long-lived assets, severance and other costs. See Note 19 to our accompanying Consolidated Financial Statements for additional details.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections

Not applicable.

Part III

Item 10. Directors, Executive Officers and Corporate Governance

We have adopted a written Global Code of Conduct and Business Ethics, which is applicable to all employees and directors, including our Chief Executive Officer, Chief Financial Officer, other executive officers and senior financial personnel. A copy of our Global Code of Conduct and Business Ethics is available in the Corporate Governance section of the Investors section of our website at www.biomarin.com. Information on our website is not incorporated by reference in this Annual Report on Form 10-K. If we make any substantive amendments to our Global Code of Conduct and Business Ethics or grant any waiver from a provision of our Global Code of Conduct and Business Ethics to any executive officer or director, we will promptly disclose the nature of the amendment or waiver on our website in accordance with the requirements of Item 5.05 of Form 8-K.

The remaining information required by this Item is incorporated into this section by reference to the information contained in the proxy statement for our 2026 annual meeting of stockholders.

Item 11. Executive Compensation

The information required by this Item is incorporated into this section by reference to the information contained in the proxy statement for our 2026 annual meeting of stockholders.

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

The information required by this Item is incorporated into this section by reference to the information contained in the proxy statement for our 2026 annual meeting of stockholders.

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

The information required by this Item is incorporated into this section by reference to the information contained in the proxy statement for our 2026 annual meeting of stockholders.

Item 14. Principal Accountant Fees and Services

The information required by this Item is incorporated into this section by reference to the information contained in the proxy statement for our 2026 annual meeting of stockholders.

Part IV

Item 15. Exhibits, Financial Statement Schedules

Exhibit Index

Exhibit Number	Description
2.1	Agreement and Plan of Merger, dated as of May 16, 2025, by and among Inozyme Pharma, Inc., BioMarin Pharmaceutical Inc., and Incline Merger Sub, Inc., previously filed with the SEC on May 16, 2025 as Exhibit 2.1 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
2.2	Agreement and Plan of Merger, dated as of December 19, 2025, by and among Amicus Therapeutics, Inc., BioMarin Pharmaceutical Inc., and Lynx Merger Sub 1, Inc., previously filed with the SEC on December 19, 2025 as Exhibit 2.1 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
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.
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	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.
4.3	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.
4.4	Indenture, dated as of February 12, 2026, between BioMarin Pharmaceutical Inc. and U.S. Bank Trust Company, National Association, as trustee, including the Form of Global Note representing BioMarin Pharmaceutical, Inc.'s 5.500% Senior Notes due 2034 as Exhibit A thereto, previously filed with the SEC on February 12, 2026, as Exhibit 4.1 to the Company's Current Report on Form 8-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	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.
10.8	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.
10.9	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.

10.10	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.
10.11†	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.
10.12†	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.
10.13†	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.
10.14†	BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, as amended on August 1, 2025, previously filed with the SEC on August 5, 2025 as Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q (File No.000-26727), which is incorporated herein by reference.
10.15†	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.
10.16†	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.
10.17†	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.
10.18†*	BioMarin Pharmaceutical Inc. Summary of Independent Director Compensation.
10.19†	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.
10.20†	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.
10.21†	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.
10.22†	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.
10.23†	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.
10.24†	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.
10.25†	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.
10.26	Credit Agreement, dated as of August 28, 2024, by and among BioMarin Pharmaceutical Inc., as the Borrower, Citibank, N.A., as Administrative Agent, and the Lenders party thereto, previously filed with the SEC on September 4, 2024 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
10.27	Form of Tender and Support Agreement, previously filed with the SEC on May 16, 2025 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
19.1	BioMarin Pharmaceutical Inc. Insider Trading Policy, previously filed with the SEC on February 24, 2025 as Exhibit 19.1 to the Company's Annual Report on Form 10-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, previously filed with the SEC on February 26, 2024 as Exhibit 97.1 to the Company's Annual Report on Form 10-K (File No. 000-26727).
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, 2025, 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, 2025 and December 31, 2024, (ii) Consolidated Statements of Income for the years ended December 31, 2025, 2024 and 2023, (iii) Consolidated Statements of Comprehensive Income for the years ended December 31, 2025, 2024 and 2023, (iv) Consolidated Statements of Stockholders’ Equity for the years ended December 31, 2025, 2024 and 2023, (v) Consolidated Statements of Cash Flows for the years ended December 31, 2025, 2024 and 2023, 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, 2026

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, 2026
/S/ BRIAN R. MUELLER **Brian R. Mueller**	Executive Vice President, Finance & Chief Financial Officer (Principal Financial Officer)	February 26, 2026
/S/ RASHMI RAMCHANDANI **Rashmi Ramchandani**	Vice President, Chief Accounting Officer (Principal Accounting Officer)	February 26, 2026
/S/ RICHARD A. MEIER **Richard A. Meier**	Chair of the Board of Directors	February 26, 2026
/S/ ELIZABETH MCKEE ANDERSON **Elizabeth McKee Anderson**	Director	February 26, 2026
/S/ BARBARA BODEM **Barbara Bodem**	Director	February 26, 2026
/S/ IAN T. CLARK **Ian T. Clark**	Director	February 26, 2026
/S/ ATHENA COUNTOURIOTIS, M.D. **Athena Countouriotis, M.D.**	Director	February 26, 2026
/S/ WILLARD H. DERE, M.D. **Willard H. Dere, M.D.**	Director	February 26, 2026
/S/ MARK ENYEDY **Mark Enyedy**	Director	February 26, 2026
/S/ MAYKIN HO **Maykin Ho**	Director	February 26, 2026
/S/ ROBERT J. HOMBACH **Robert J. Hombach**	Director	February 26, 2026
/S/ TIMOTHY P. WALBERT **Timothy P. Walbert**	Director	February 26, 2026

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, 2025 and 2024, the related consolidated statements of income, comprehensive income, stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2025, 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, 2025, 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, 2025 and 2024, and the results of its operations and its cash flows for each of the years in the three-year period ended December 31, 2025, 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, 2025 based on criteria established in *Internal Control – Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission.

Basis for Opinions
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 audits 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 audits 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, 2025, the Company recognized $208.5 million in ALDURAZYME net product revenue. Under this 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, 2026

BIOMARIN PHARMACEUTICAL INC.
CONSOLIDATED STATEMENTS OF INCOME
Years Ended December 31, 2025, 2024 and 2023
(In thousands of U.S. Dollars, except per share amounts)

	2025	2024	2023
REVENUES:			
Net product revenues	$ 3,167,759	$ 2,809,445	$ 2,372,538
Royalty and other revenues	53,494	44,470	46,688
Total revenues	3,221,253	2,853,915	2,419,226
OPERATING EXPENSES:			
Cost of sales	717,442	580,235	532,062
Research and development	921,930	747,184	746,773
Selling, general and administrative	1,153,017	1,009,025	892,406
Intangible asset amortization	19,386	43,257	62,211
Gain on sale of nonfinancial assets	—	(10,000)	—
Total operating expenses	2,811,775	2,369,701	2,233,452
INCOME FROM OPERATIONS	409,478	484,214	185,774
Interest income	74,904	74,883	58,339
Interest expense	(10,899)	(12,666)	(17,335)
Other income (expense), net	8,997	(4,668)	(38,215)
INCOME BEFORE INCOME TAXES	482,480	541,763	188,563
Provision for income taxes	133,579	114,904	20,918
NET INCOME	$ 348,901	$ 426,859	$ 167,645
EARNINGS PER SHARE, BASIC	$ 1.82	$ 2.25	$ 0.89
EARNINGS PER SHARE, DILUTED	$ 1.80	$ 2.21	$ 0.87
Weighted average common shares outstanding, basic	191,787	190,027	187,834
Weighted average common shares outstanding, diluted	197,394	196,708	191,595

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

BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME

Years Ended December 31, 2025, 2024 and 2023

(In thousands of U.S. Dollars)

	2025	2024	2023
NET INCOME	$ 348,901	$ 426,859	$ 167,645
OTHER COMPREHENSIVE INCOME (LOSS):			
Available-for-sale debt securities:			
Unrealized holding gain (loss) arising during the period, net of tax impact of $(587), $(289) and $(3,922), respectively	1,942	959	12,963
Cash flow hedges:			
Unrealized holding gain (loss) arising during the period, net of tax impact of $0 for all periods presented	(92,542)	104,354	(37,720)
Less: reclassifications to net income, net of tax impact of $0 for all periods presented	(15,474)	14,872	164
Net change in unrealized holding gain (loss), net of tax	(77,068)	89,482	(37,884)
OTHER COMPREHENSIVE INCOME (LOSS), NET OF TAX	(75,126)	90,441	(24,921)
COMPREHENSIVE INCOME	$ 273,775	$ 517,300	$ 142,724

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

BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED BALANCE SHEETS

December 31, 2025 and 2024

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

	December 31, 2025	December 31, 2024
ASSETS		
Current assets:		
Cash and cash equivalents	$ 1,311,679	$ 942,842
Short-term investments	248,930	194,864
Accounts receivable, net	908,214	660,535
Inventory	1,298,883	1,232,653
Other current assets	185,784	201,533
Total current assets	3,953,490	3,232,427
Noncurrent assets:		
Long-term investments	492,242	521,238
Property, plant and equipment, net	952,508	1,043,041
Intangible assets, net	213,837	255,278
Goodwill	196,199	196,199
Deferred tax assets	1,508,697	1,489,366
Other assets	277,049	251,391
Total assets	$ 7,594,022	$ 6,988,940
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 759,031	$ 606,988
Total current liabilities	759,031	606,988
Noncurrent liabilities:		
Long-term convertible debt, net	597,176	595,138
Other long-term liabilities	150,816	128,824
Total liabilities	1,507,023	1,330,950
Stockholders' equity:		
Common stock, $0.001 par value: 500,000,000 shares authorized; 192,300,101 and 190,761,349 shares issued and outstanding, respectively	192	191
Additional paid-in capital	5,956,582	5,802,068
Company common stock held by the Nonqualified Deferred Compensation Plan	(10,508)	(11,227)
Accumulated other comprehensive income (loss)	(13,473)	61,653
Retained earnings (accumulated deficit)	154,206	(194,695)
Total stockholders' equity	6,086,999	5,657,990
Total liabilities and stockholders' equity	$ 7,594,022	$ 6,988,940

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

BIOMARIN PHARMACEUTICAL INC.
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
Years Ended December 31, 2025, 2024 and 2023
(In thousands of U.S. Dollars and share amounts in thousands)

	2025	2024	2023
Shares of Common Stock, beginning balances	190,761	188,598	186,251
Issuances under equity incentive plans	1,539	2,163	2,347
Shares of Common Stock, ending balances	192,300	190,761	188,598
Total stockholders' equity, beginning balances	$ 5,657,990	$ 4,951,549	$ 4,603,156
Common stock:			
Beginning balances	191	189	186
Issuances under equity incentive plans, net of tax	1	2	3
Ending balances	192	191	189
Additional paid-in capital:			
Beginning balances	5,802,068	5,611,562	5,404,895
Issuances under equity incentive plans, net of tax	(41,555)	(28,354)	(7,162)
Stock-based compensation	196,788	217,493	212,828
Change in Common stock held by the Nonqualified Deferred Compensation plan (NQDC)	(719)	1,367	1,001
Ending balances	5,956,582	5,802,068	5,611,562
Company common stock held by the NQDC:			
Beginning balances	(11,227)	(9,860)	(8,859)
Change in Common stock held by the NQDC	719	(1,367)	(1,001)
Ending balances	(10,508)	(11,227)	(9,860)
Accumulated other comprehensive income (loss):			
Beginning balances	61,653	(28,788)	(3,867)
Other comprehensive income (loss)	(75,126)	90,441	(24,921)
Ending balances	(13,473)	61,653	(28,788)
Retained earnings (accumulated deficit)			
Beginning balances	(194,695)	(621,554)	(789,199)
Net income	348,901	426,859	167,645
Ending balances	154,206	(194,695)	(621,554)
Total stockholders' equity, ending balances	$ 6,086,999	$ 5,657,990	$ 4,951,549

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

BIOMARIN PHARMACEUTICAL INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
Years Ended December 31, 2025, 2024 and 2023
(In thousands of U.S. dollars)

	2025	2024	2023
CASH FLOWS FROM OPERATING ACTIVITIES:			
Net income	$ 348,901	$ 426,859	$ 167,645
Adjustments to reconcile net income to net cash provided by operating activities:			
Depreciation and amortization	79,557	96,426	104,386
Non-cash interest expense	2,622	3,359	4,188
Accretion of discount on investments	(4,801)	(8,345)	(9,228)
Stock-based compensation	181,409	201,571	207,099
Gain on sale of nonfinancial assets	—	(10,000)	—
Impairment of assets	125,012	19,889	38,608
ROCTAVIAN inventory write-off	119,208	—	—
Deferred income taxes	48,738	56,096	(44,981)
Unrealized foreign exchange gain	4,459	(16,753)	28,446
Acquired in-process research & development expense	220,963	—	—
Other	(4,414)	20,135	(365)
Changes in operating assets and liabilities:			
Accounts receivable, net	(228,054)	(57,909)	(190,435)
Inventory	(116,929)	(63,530)	(157,058)
Other current assets	8,891	(3,778)	(50,335)
Other assets	(38,573)	(73,700)	(31,149)
Accounts payable and accrued liabilities	66,136	(32,240)	68,853
Other long-term liabilities	14,869	14,761	23,585
Net cash provided by operating activities	827,994	572,841	159,259
CASH FLOWS FROM INVESTING ACTIVITIES:			
Purchases of property, plant and equipment	(103,038)	(85,424)	(96,691)
Maturities and sales of investments	337,801	633,018	864,863
Purchases of investments	(355,875)	(410,250)	(868,496)
Proceeds from sale of nonfinancial assets	—	10,000	—
Purchase of intangible assets	(7,937)	(11,994)	(10,920)
Acquisition, net of cash acquired	(285,193)	—	—
Other	—	1,141	—
Net cash provided by (used in) investing activities	(414,242)	136,491	(111,244)
CASH FLOWS FROM FINANCING ACTIVITIES:			
Proceeds from exercises of awards under equity incentive plans	14,460	49,277	69,353
Taxes paid related to net share settlement of equity awards	(55,965)	(77,560)	(76,319)
Repayments of convertible debt	—	(494,987)	—
Other	(889)	(3,177)	(11,761)
Net cash used in financing activities	(42,394)	(526,447)	(18,727)
Effect of exchange rate changes on cash	(2,521)	4,830	1,308
NET INCREASE IN CASH AND CASH EQUIVALENTS	368,837	187,715	30,596
Cash and cash equivalents:			
Beginning of period	$ 942,842	$ 755,127	$ 724,531
End of period	$ 1,311,679	$ 942,842	$ 755,127
SUPPLEMENTAL CASH FLOW DISCLOSURES:			
Cash paid for interest	$ 7,245	$ 10,361	$ 10,303
Cash paid for income taxes	$ 96,205	$ 57,269	$ 73,312
SUPPLEMENTAL CASH FLOW DISCLOSURES FOR NON-CASH INVESTING AND FINANCING ACTIVITIES:			
Accounts payable and accrued liabilities related to fixed assets	$ 3,780	$ 3,751	$ 10,280
Accounts payable and accrued liabilities related to intangible assets	$ 1,247	$ 6,327	$ 8,000

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

BioMarin Pharmaceutical Inc. (the Company or BioMarin) is a global biotechnology company dedicated to translating the promise of genetic discovery into medicines that make a profound impact on the life of each patient. The San Rafael, California-based company, founded in 1997, has a proven track record of innovation with eight commercial therapies and a strong clinical and preclinical pipeline. Using a distinctive approach to drug discovery and development, BioMarin pursues treatments that offer new possibilities for patients and families around the world navigating rare or difficult to treat genetic conditions.

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 other events or transactions that occurred subsequent to the balance sheet date and prior to the filing of this Annual Report on Form 10-K except for the transactions disclosed in Note 21 to these Consolidated Financial Statements.

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 (Loss) (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

The Company values inventory at the lower of cost and net realizable value. The Company determines the cost of inventory using the standard-cost and average-cost methods, which approximates cost based 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 Income.

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, Research and Development (R&D) and SG&A, as appropriate, in the Consolidated Statements of Income. 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 Income.

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

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

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 in the Consolidated Statements of Income.

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 amortized over their estimated useful lives on a straight-line basis. Amortization expense is recorded in Intangible Asset Amortization on the Company's Consolidated Statements of Income, 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, lease 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 Income. 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 Income. Impairment charges related to all other finite-lived intangible assets that are not material are recorded to Intangible Asset Amortization in the Consolidated Statements of Income.

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.

The following table presents the Company's capitalized software costs for the periods presented:

	December 31,	
Capitalized Software Classification	**2025**	**2024**
Assets:		
Other current assets	$ 9.1	$ 6.4
Other assets	97.0	65.7
Total Capitalized Software	$ 106.1	$ 72.1

Amortization expense related to capitalized software was $7.4 million for the year ended December 31, 2025 and was not material for the years ended December 31, 2024 and 2023.

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 Income, 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 Income. 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 (R&D) 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 Income and are expensed as incurred. Advertising expenses were $50.5 million, $34.5 million and $27.8 million in 2025, 2024 and 2023, respectively.

Earnings Per Common Share

Basic earnings per share is calculated by dividing Net Income by the weighted average shares of common stock outstanding during the period. Diluted earnings 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 stock 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 Income. 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 a 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 assets. If it is more likely than not that the Company would not realize the deferred tax assets, 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 Other Expense, Net in the Consolidated Statements of Income totaled $6.1 million, $8.6 million and $27.7 million in 2025, 2024 and 2023, 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 change 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 Other income (expense), net, in the Consolidated Statements of Income.

Acquisitions

Acquisitions of businesses are accounted for using the acquisition method of accounting. The Company allocates the purchase price of acquired businesses to the tangible and intangible assets acquired and liabilities assumed based on their estimated fair values on the acquisition date. The purchase price allocation process requires management to make significant estimates and assumptions, especially at the acquisition date with respect to intangible assets and acquired IPR&D assets. Any excess of the purchase price over the estimated fair values of the net assets acquired is recognized as goodwill.

If it is determined that the net assets acquired do not meet the definition of a business combination under the acquisition method of accounting, the transaction is accounted for as an asset acquisition and no goodwill is recognized. Acquired IPR&D assets with no alternative future use under the acquisition method of accounting are charged to R&D in the Consolidated Statements of Income.

Fair Value of Financial Instruments

The Company applies fair value accounting for all financial assets and liabilities and nonfinancial 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 resulting from business acquisitions. The estimated fair value of acquired finite-lived intangible assets is measured by applying a probability-based income approach utilizing an appropriate discount rate as of the acquisition date.

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

Recent Accounting Pronouncements

New Accounting Pronouncements Issued and Adopted

Income Taxes

In December 2023, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (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 Company adopted this ASU in December 2025 on a prospective basis and it did not have a material impact on the Company's Consolidated Financial Statements. See Note 15 - *Income Taxes* to these Consolidated Financial Statements for further information.

New Accounting Pronouncements Issued and Not Yet Adopted

Income Statement Disaggregation

In November 2024, the FASB issued ASU 2024-03, Income Statement - Reporting Comprehensive Income Topic 220, *Expense Disaggregation Disclosures*. The guidance requires disclosure of additional information about specific expense categories in the notes to financial statements at interim and annual reporting periods. The disclosure requirements will be applied on a prospective basis, with the option to apply it retrospectively. The effective date for the update is for fiscal years beginning after December 15, 2026 and interim periods within fiscal years beginning after December 15, 2027. The Company is currently evaluating the effect of the update on the Company's related disclosures.

Internal-use Software

In September 2025, the FASB issued ASU 2025-06, *Intangibles - Goodwill and Other - Internal-Use Software Topic 350-40: Accounting for and Disclosure of Software Costs*. The guidance clarifies the accounting for costs related to internal-use software by removing all references to project stages and clarifying the threshold to be applied to begin capitalizing. The guidance also specifies that disclosure under Accounting Standards Codification Topic 360-10, *Property, Plant, and Equipment - Overall*,

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

apply to capitalized software costs. The Company may apply the guidance using a prospective, retrospective or modified transition approach. The effective date for the update is for fiscal years beginning after December 15, 2027 and interim periods within fiscal years beginning after December 15, 2027. The Company is currently evaluating the effect of the update on the Company's Consolidated Financial Statements and related disclosures.

Accounting pronouncements not listed above were assessed and determined to be either not applicable or did not have a material impact on the Company's consolidated financial statements.

(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, 2025 and 2024, respectively:

	December 31, 2025						
	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	$ 415,760	$ —	$ —	$ 415,760	$ 415,760	$ —	$ —
Level 2:							
Money market instruments	895,919	—	—	895,919	895,919	—	—
Corporate debt securities	472,572	3,286	(3)	475,855	—	189,566	286,289
U.S. government agency securities	206,018	1,197	(1)	207,214	—	59,115	148,099
Asset-backed securities	57,687	420	(4)	58,103	—	249	57,854
Subtotal	1,632,196	4,903	(8)	1,637,091	895,919	248,930	492,242
Total	$ 2,047,956	$ 4,903	$ (8)	$ 2,052,851	$ 1,311,679	$ 248,930	$ 492,242

	December 31, 2024						
	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	$ 329,619	$ —	$ —	$ 329,619	$ 329,619	$ —	$ —
Level 2:							
Money market instruments	613,223	—	—	613,223	613,223	—	—
Corporate debt securities	503,202	2,410	(390)	505,222	—	168,104	337,118
U.S. government agency securities	72,027	359	(33)	72,353	—	896	71,457
Asset-backed securities	138,508	363	(344)	138,527	—	25,864	112,663
Subtotal	1,326,960	3,132	(767)	1,329,325	613,223	194,864	521,238
Total	$ 1,656,579	$ 3,132	$ (767)	$ 1,658,944	$ 942,842	$ 194,864	$ 521,238

(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.

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

As of December 31, 2025, 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 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.

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,	
	2025	2024
Finite-lived intangible assets	$ 723,966	$ 721,110
Accumulated amortization	(510,129)	(465,832)
Net carrying value	$ 213,837	$ 255,278

The following table summarizes the carrying value and estimated remaining life of the Company's finite-lived intangible assets as of December 31, 2025:

	Net Balance	Average Remaining Life
Acquired intellectual property	$ 127,163	7.4 years
Technology transfer	83,397	6.0 years[(1)]
License payments	3,277	4.9 years
Total	$ 213,837	

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

As of December 31, 2025, 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
2026	$ 30,281
2027	30,281
2028	28,709
2029	27,470
2030	27,332
Thereafter	55,538
	$ 199,611

(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,	
	2025	2024
Building and improvements	$ 910,710	$ 892,484
Manufacturing and laboratory equipment	574,576	542,856
Computer hardware and software	202,588	204,768
Land	90,781	90,781
Leasehold improvements	44,035	44,368
Furniture and equipment	41,647	41,871
Land improvements	27,565	27,433
Construction-in-progress	134,911	90,271
	2,026,813	1,934,832
Accumulated depreciation	(1,074,305)	(891,791)
Total property, plant and equipment, net	$ 952,508	$ 1,043,041

Depreciation expense, net of amounts capitalized into inventory, was $48.5 million, $46.6 million and $40.3 million for the years ended December 31, 2025, 2024 and 2023, respectively.

(5) INVENTORY

Inventory consisted of the following:

	December 31,	
	2025	2024
Raw materials	$ 106,510	$ 154,341
Work-in-process	801,061	550,678
Finished goods	391,312	527,634
Total inventory	$ 1,298,883	$ 1,232,653

(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,	
	2025	2024
Accounts payable and accrued operating expenses	$ 312,768	$ 235,403
Accrued compensation expense	219,422	202,513
Accrued rebates payable	166,925	120,835
Foreign currency exchange forward contracts	31,007	13,056
Lease liability	8,685	7,574
Accrued royalties payable	7,968	7,923
Accrued income taxes	3,667	12,567
Other	8,589	7,117
Total accounts payable and accrued liabilities	$ 759,031	$ 606,988

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, 2025, 2024 and 2023, were as follows:

	Balance at Beginning of Period	Provision for Current Period Sales	Payments	Balance at End of Period
Year ended December 31, 2025:				
Accrued rebates	$ 120,835	312,122	(266,032)	$ 166,925
Reserve for cash discounts	$ 2,644	24,822	(24,266)	$ 3,200
Year ended December 31, 2024:				
Accrued rebates	$ 96,179	230,801	(206,145)	$ 120,835
Reserve for cash discounts	$ 5,390	18,771	(21,517)	$ 2,644
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

(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.

Other than the Company's fixed-rate convertible debt disclosed in Note 10 to these Consolidated Financial Statements, there were no financial assets or liabilities that were remeasured using Level 1 inputs as of December 31, 2025 and 2024. Refer to Notes 2 and 8 to these Consolidated Financial Statements for other financial assets and liabilities measured at fair value. The

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

Company had no financial assets or liabilities that are remeasured on a recurring basis using Level 3 inputs as of December 31, 2025 and 2024.

Level 2 assets and liabilities that are remeasured using significant observable inputs consisted of the following, except for derivatives, which are discussed in Note 8 – *Derivative Instruments and Hedging Strategies*:

	December 31,	
	2025	2024
Assets:		
Other current assets:		
NQDC Plan assets	$ 3,765	$ 2,928
Other assets:		
NQDC Plan assets	41,689	34,978
Restricted investments (1)	375	514
Total other assets	42,064	35,492
Total assets	$ 45,829	$ 38,420
Liabilities:		
Current liabilities:		
NQDC Plan liability	$ 3,765	$ 2,928
Other long-term liabilities:		
NQDC Plan liability	41,689	34,978
Total liabilities	$ 45,454	$ 37,906

(1) The restricted investments as of December 31, 2025 and 2024 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.

(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.

	December 31,	
Forward Contracts	2025	2024
Derivatives designated as hedging instruments:		
Sell	$ 1,573,184	$ 1,371,816
Purchase	$ 385,499	$ 289,967
Derivatives not designated as hedging instruments:		
Sell	$ 356,285	$ 344,101
Purchase	$ 36,798	$ 63,617

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

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

	December 31,	
Balance Sheet Location	**2025**	**2024**
Derivatives designated as hedging instruments:		
Asset Derivatives - Level 2 (1)		
Other current assets	$ 17,585	$ 60,192
Other assets	5,591	14,514
Subtotal	$ 23,176	$ 74,706
Liability Derivatives - Level 2 (1)		
Accounts payable and accrued liabilities	$ 30,134	$ 12,381
Other long-term liabilities	9,905	2,536
Subtotal	$ 40,039	$ 14,917
Derivatives not designated as hedging instruments:		
Asset Derivatives - Level 2 (1)		
Other current assets	$ 2,479	$ 4,934
Liability Derivatives - Level 2 (1)		
Accounts payable and accrued liabilities	$ 873	$ 675
Total Derivatives Assets	$ 25,655	$ 79,640
Total Derivatives Liabilities	$ 40,912	$ 15,592

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

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

	Years Ended December 31,		
	2025	**2024**	**2023**
Derivatives Designated as Cash Flow Hedging Instruments	**Cash Flow Hedging Gains (Losses) Reclassified into Earnings**		
Net product revenues	$ (20,611)	$ 14,708	$ (186)
Operating expenses	$ 5,137	$ 164	$ 350
Derivatives Not Designated as Hedging Instruments	**Gains (Losses) Recognized in Earnings**		
Operating expenses	$ (32,536)	$ 33,966	$ (8,808)

As of December 31, 2025, the Company expects to reclassify unrealized losses of $12.9 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, 2025	December 31, 2024
Assets:			
Operating	Other assets	$ 35,243	$ 28,680
Financing	Other assets	4,046	5,071
Total ROU assets		$ 39,289	$ 33,751
Liabilities:			
Current:			
Operating	Accounts payable and accrued liabilities	$ 8,569	$ 7,233
Financing	Accounts payable and accrued liabilities	116	341
Noncurrent:			
Operating	Other long-term liabilities	36,576	30,501
Financing	Other long-term liabilities	436	756
Total lease liabilities		$ 45,697	$ 38,831

Maturities of lease liabilities as of December 31, 2025 by fiscal year were as follows:

	Operating	Financing	Total
2026	$ 10,066	$ 235	$ 10,301
2027	9,355	222	9,577
2028	8,592	141	8,733
2029	6,332	—	6,332
2030	3,848	—	3,848
Thereafter	18,459	—	18,459
Total lease payments	56,652	598	57,250
Less: Imputed interest	(11,507)	(46)	(11,553)
Present value of lease liabilities	$ 45,145	$ 552	$ 45,697

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

Lease Cost	Classification	Years Ended December 31, 2025	2024	2023
Operating [1]	Operating expenses	$ 11,330	$ 14,154	$ 14,197
Financing:				
Amortization	Operating expenses	1,729	1,703	3,360
Interest expense	Interest expense	6	5	2,648
Total lease costs		$ 13,065	$ 15,862	$ 20,205

(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:

	December 31,	
	2025	2024
Weighted average remaining lease term (in years):		
Operating leases	7.0	6.5
Financing leases	2.8	3.1
Weighted average discount rate:		
Operating leases	6.2%	6.1%
Financing leases	6.0%	5.4%

As of December 31, 2025, no leases were expected to commence that would create significant rights and obligations for the Company.

	Years Ended December 31,		
Supplemental Cash Flow Information	**2025**	**2024**	**2023**
Cash paid for amounts included in the measurement of lease liabilities:			
Cash used in operating activities:			
Operating leases	$ 9,342	$ 13,388	$ 9,980
Financing leases	$ 47	$ 23	$ 51
Cash used in financing activities:			
Financing leases	$ 889	$ 216	$ 2,286
ROU assets obtained in exchange for lease obligations:			
Operating leases	$ 12,933	$ 2,812	$ 16,321
Financing leases	$ 661	$ 1,196	$ 68

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

(10) DEBT

Convertible Notes

As of December 31, 2025, the Company had outstanding fixed-rate convertible notes for an undiscounted aggregate principal amount of $600.0 million. The following table summarizes information regarding the Company's convertible notes:

	December 31,	
	2025	2024
1.250% senior subordinated convertible notes due in May 2027 (the 2027 Notes)	600,000	600,000
Unamortized discount net of deferred offering costs	(2,824)	(4,862)
Total convertible debt, net	$ 597,176	$ 595,138
Fair value of fixed-rate convertible debt [1]:		
2027 Notes	$ 576,267	$ 558,894

(1) 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,		
	2025	2024	2023
Coupon interest expense	$ 7,500	$ 9,564	$ 10,465
Accretion of discount on convertible notes	1,942	2,775	3,359
Amortization of debt issuance costs	107	391	594
Total interest expense on convertible debt	$ 9,549	$ 12,730	$ 14,418

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 $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.

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

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 per common share.

Revolving Credit Facility

In August 2024, the Company entered into an unsecured revolving credit facility providing for $600.0 million in revolving loan commitments. The credit facility was intended to finance ongoing working capital needs and for other general corporate purposes. The credit facility contains financial covenants including a maximum total net leverage ratio and a minimum interest coverage ratio. The credit facility matures in August 2029. As of December 31, 2025, there were no amounts outstanding under the credit facility and the Company was in compliance with all covenants.

(11) ACCUMULATED OTHER COMPREHENSIVE INCOME

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 as of 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 as of December 31, 2023	$ (29,658)	$ 870	$ (28,788)
Other comprehensive income (loss) before reclassifications	104,354	1,248	105,602
Less: gain (loss) reclassified from AOCI	14,872	—	14,872
Tax effect	—	(289)	(289)
Net current period other comprehensive income	89,482	959	90,441
AOCI balance as of December 31, 2024	$ 59,824	$ 1,829	$ 61,653
Other comprehensive income (loss) before reclassifications	(92,542)	2,529	(90,013)
Less: gain (loss) reclassified from AOCI	(15,474)	—	(15,474)
Tax effect	—	(587)	(587)
Net current period other comprehensive income (loss)	(77,068)	1,942	(75,126)
AOCI balance as of December 31, 2025	$ (17,244)	$ 3,771	$ (13,473)

(12) SEGMENT INFORMATION

The Company operates and is managed as one operating segment which derives revenue from activities related to the development and commercialization of innovative therapies for people with serious and life-threatening rare diseases and medical conditions.

The Company's commercial organization is responsible for marketing its approved products worldwide. The Company's R&D organization is responsible for research and discovery of new product candidates and supporting the development and registration efforts for potential new products. The Company's technical operations group is responsible for the development of

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

manufacturing processes, supplying clinical drug product, and the manufacturing and distribution of its commercial products. The Company is also supported by corporate staff functions.

The Company's Chief Executive Officer as the CODM manages and allocates resources to the operations of the total company by assessing the overall level of resources available and how to best allocate them to support the Company's long-term company-wide strategic goals. In making this decision, the CODM uses consolidated financial information for the purposes of evaluating performance, allocating resources, setting incentive compensation targets and planning and forecasting for future periods.

The key measure of segment profit or loss used by the CODM to allocate resources and assess the Company's performance is its Consolidated Net Income, as reported on the Consolidated Statements of Income. The CODM's analysis includes a comparison to budgeted results. Segment assets provided to the CODM are consistent with those reported on the Consolidated Balance Sheets with particular emphasis on the Company's available liquidity including cash, cash equivalents, investments, accounts receivable and inventory.

The following table includes information about segment revenue, significant segment expenses, and segment measure of profitability:

	Years Ended December 31,		
	2025	2024	2023
Total revenues	$ 3,221,253	$ 2,853,915	$ 2,419,226
Less:			
Cost of sales	717,442	580,235	532,062
R&D expenses			
Research and early pipeline	383,705	434,023	393,078
Later-stage clinical programs	308,334	27,581	62,604
Marketed products	229,891	285,580	291,091
SG&A expenses			
S&M expenses	530,156	476,739	488,442
G&A expenses	622,861	532,286	403,964
Other segment expense, net [1]	79,963	90,612	80,340
Net income	$ 348,901	$ 426,859	$ 167,645

(1) Other segment expense, net, during the years ended December 31, 2025, 2024 and 2023 included Intangible Asset Amortization, Interest Income and Expense, Other Income (Expense), Net and Provision for Income Taxes. The year ended December 31, 2024 also included Gain on Sale of Nonfinancial Assets.

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

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

	Years Ended December 31,		
	2025	2024	2023
VOXZOGO	$ 926,923	$ 735,092	$ 469,881
Enzyme Therapies:			
VIMIZIM	792,051	739,784	701,053
NAGLAZYME	485,400	479,584	420,292
PALYNZIQ	433,310	355,047	303,919
ALDURAZYME	208,508	183,887	131,248
BRINEURA	186,397	169,083	161,889
KUVAN	99,530	120,902	180,767
ROCTAVIAN	35,640	26,066	3,489
Total net product revenues	3,167,759	2,809,445	2,372,538
Royalty and other revenues	53,494	44,470	46,688
Total revenues	$ 3,221,253	$ 2,853,915	$ 2,419,226

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,		
	2025	2024	2023
United States	$ 1,104,973	$ 924,810	$ 771,314
Europe	874,331	829,031	669,331
Latin America	435,478	378,084	332,437
Rest of world	544,469	493,633	468,208
Total net product revenues marketed by the Company	2,959,251	2,625,558	2,241,290
ALDURAZYME net product revenues marketed by Sanofi	208,508	183,887	131,248
Total net product revenues	$ 3,167,759	$ 2,809,445	$ 2,372,538

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

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,		
	2025	2024	2023
Customer A	14 %	13 %	14 %
Customer B	12 %	12 %	12 %
Customer C	11 %	10 %	10 %
Total	37 %	35 %	36 %

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,	
	2025	2024
Long-lived assets by geography:		
United States	$ 639,669	$ 755,069
Ireland	331,287	308,123
Rest of world	20,841	13,600
Total long-lived assets	$ 991,797	$ 1,076,792

Concentration Information

On a consolidated basis, two customers accounted for 20% and 10% of the Company's December 31, 2025 accounts receivable balance, respectively, compared to December 31, 2024 when two customers accounted for 20% and 11% of the accounts receivable balance, respectively. As of December 31, 2025 and 2024, the accounts receivable balance for Sanofi included $148.0 million and $96.8 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, geopolitical instability, impact of new or increased tariffs and escalating trade tensions, regulatory uncertainty, 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.

(13) EQUITY COMPENSATION PLANS AND STOCK-BASED COMPENSATION

Equity Compensation Plans

Shares Available Under Equity Compensation Plans

As of December 31, 2025, an aggregate of approximately 54.9 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 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

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

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) and RSUs with market-based vesting conditions (base TSR-RSUs) 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 monthly thereafter. The contractual term of stock option awards is generally 10 years from the grant date. As of December 31, 2025, approximately 43.1 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 which was 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, 2025, the Company issued 0.3 million shares under the ESPP. As of December 31, 2025, approximately 7.0 million shares were authorized and 2.0 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 Income for all stock-based compensation arrangements was as follows:

	Years Ended December 31,		
	2025	2024	2023
Cost of sales	$ 14,165	$ 15,131	$ 17,604
Research and development	54,691	59,545	65,714
Selling, general and administrative	112,553	126,895	123,781
Total stock-based compensation expense	$ 181,409	$ 201,571	$ 207,099

Stock-based compensation of $27.2 million, $28.3 million and $21.7 million was capitalized into inventory for the years ended December 31, 2025, 2024 and 2023, 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, 2025:

	Shares	Weighted Average Grant Date Fair Value
Non-vested units as of December 31, 2024	4,536,723	$ 83.88
Granted	3,033,951	$ 68.23
Vested	(1,719,176)	$ 83.38
Forfeited	(625,092)	$ 79.27
Non-vested units as of December 31, 2025	5,226,406	$ 75.50

The weighted-average grant date fair values per share of RSUs with service-based vesting granted during the years ended December 31, 2025, 2024 and 2023, was $68.23, $82.98 and $88.96, respectively. The total intrinsic values of restricted stock that vested and released in the years ended December 31, 2025, 2024 and 2023, was $114.3 million, $152.2 million and $149.8 million, respectively.

As of December 31, 2025, total unrecognized compensation cost related to unvested RSUs with service-based vesting conditions of $279.5 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, 2025:

	Shares	Weighted Average Grant Date Fair Value
Non-vested units as of December 31, 2024	427,310	$ 84.29
Granted	279,134	$ 71.55
Vested	(228,252)	$ 83.43
Forfeited	(9,960)	$ 79.32
Non-vested units as of December 31, 2025	468,232	$ 79.60

The weighted-average grant date fair value of the PRSUs for the years ended December 31, 2025, 2024 and 2023, was $71.55, $81.27 and $89.22, respectively.

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

As of December 31, 2025, total unrecognized compensation expense related to non-vested PRSUs of $5.5 million was expected to be recognized over a weighted average period of 1.6 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 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 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, 2025:

	Shares	Weighted Average Grant Date Fair Value
Non-vested units as of December 31, 2024	443,340	$ 120.92
Granted	214,578	$ 117.60
Vested	(132,390)	$ 136.49
Forfeited	(11,650)	$ 116.96
Non-vested units as of December 31, 2025	513,878	$ 115.61

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,		
	2025	2024	2023
Grant date fair value	$117.60	102.07	$132.56
Expected volatility	22.4 – 155.5%	20.8 – 168.3%	22.4 – 152.1%
Dividend yield	0.0%	0.0%	0.0%
Expected term	2.8 - 4.8 years	2.3 - 2.8 years	2.8 years
Risk-free interest rate	4.0 - 4.1%	3.6 - 4.6%	3.8%

As of December 31, 2025, total unrecognized compensation expense of $24.2 million related to base TSR-RSUs was expected to be recognized over a weighted average period of 2.1 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, 2025. 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, 2024	5,747,448	$ 86.44		$ 5,198
Granted	791,700	$ 69.63		
Exercised	—	$ —		
Expired and forfeited	(895,698)	$ 101.60		
Options outstanding as of December 31, 2025	5,643,450	$ 81.77	4.9	$ 266,346
Options unvested as of December 31, 2025	1,309,927	$ 75.55	8.7	$ 266,346
Exercisable as of December 31, 2025	4,331,730	$ 83.65	3.8	$ —

(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 $59.43, the closing price of the Company's common stock on the Nasdaq Global Select Market on December 31, 2025.

The weighted-average grant date fair values of stock options granted in the years ended December 31, 2025, 2024 and 2023, were $30.06, $35.87 and $39.30, respectively. No options were exercised during the year ended December 31, 2025. The total intrinsic values of options exercised during the years ended December 31, 2024 and 2023, were $9.5 million and $25.9 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,		
	2025	2024	2023
Expected volatility	37.0 – 39.6%	38.0 – 39.4%	37.8 – 40.3%
Dividend yield	0.0%	0.0%	0.0%
Expected term	5.3 – 5.9 years	4.7 – 6.2 years	4.7 – 6.2 years
Risk-free interest rate	3.7 – 4.5%	3.5 – 4.5%	3.5 – 4.6%

As of December 31, 2025, total unrecognized compensation cost related to unvested stock options of $34.6 million was expected to be recognized over a weighted average period of 2.5 years.

(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,		
	2025	2024	2023
Expected volatility	26.9 – 35.7%	24.0 – 36.9%	24.0 – 48.0%
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	3.6 – 4.2%	4.1 – 5.5%	0.06 – 5.5%

As of December 31, 2025, total unrecognized compensation cost related to unvested stock purchase rights under the ESPP of $11.3 million was expected to be recognized over a weighted average period of 1.3 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 $33.4 million, $34.4 million and $32.7 million for the years ended December 31, 2025, 2024 and 2023, 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 Income Taxes was based on Income before Income Taxes as follows:

	Years Ended December 31,		
	2025	2024	2023
U.S. Source	$ (262,617)	$ 130,503	$ (453,840)
Non-U.S. Source	745,097	411,260	642,403
Income before income taxes	$ 482,480	$ 541,763	$ 188,563

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

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

	Years Ended December 31,		
	2025	2024	2023
Provision for income taxes			
Federal	$ 32,428	$ 32,344	$ 25,120
State and local	11,528	8,813	5,098
Foreign	40,885	17,651	35,681
	84,841	58,808	65,899
Provision for deferred income taxes:			
Federal	5,498	(2,117)	(70,754)
State and local	(5,148)	(5,166)	(8,030)
Foreign	48,388	63,379	33,803
	48,738	56,096	(44,981)
Provision for income taxes	$ 133,579	$ 114,904	$ 20,918

The following is a reconciliation of the statutory federal income tax expense and rate to the Company's effective tax rate for the year ended December 31, 2025:

	Year Ended December 31,	
	2025	%
Federal Tax Expense	$ 101,321	21.0%
State and local income taxes, net of federal income tax effect[1]	1,413	0.3
Foreign tax effects		
Ireland		
Statutory tax rate difference between Ireland and United States	(47,988)	(10.0)
Other	(2,013)	(0.4)
Other Foreign Jurisdictions	2,927	0.6
Effects of changes in tax laws or rates enacted in the current period		
Effect of cross-border tax laws		
Global intangible low taxed income (GILTI)	88,510	18.3
Foreign derived intangible income (FDII)	(30,052)	(6.2)
Subpart F Income	5,353	1.1
Tax credits		
Foreign Tax Credits	(31,972)	(6.6)
Orphan Drug Credits	(13,638)	(2.8)
R&D tax credits	(7,495)	(1.6)
Changes in valuation allowances	5,274	1.1
Nontaxable or nondeductible items		
Nondeductible IPR&D[2]	45,709	9.5
Stock compensation expense	16,225	3.4
162m Addback	6,764	1.4
Other	1,198	0.3
Changes in unrecognized tax benefits[3]	(8,969)	(1.9)
Other Adjustments	1,012	0.2
Effective Tax Rate	$ 133,579	27.7%

(1) State taxes in Pennsylvania, Michigan and Illinois made up a simple majority (greater than 50%) of the tax effect in this category.
(2) Non-deductible IPR&D charges in 2025 of $45.7 million primarily related to the impact of a $221.0 million one-time, non-tax deductible charge for the acquisition of Inozyme Pharma, Inc. (Inozyme).
(3) Changes in unrecognized tax benefits for all jurisdictions are aggregated within this category.

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

The following is a reconciliation of the statutory federal income tax expense to the Company's effective tax rate for the years ended December 31, 2024 and 2023:

	Years Ended December 31,	
	2024	2023
Federal statutory income tax rate	$ 113,770	$ 39,598
State and local taxes	4,756	(3,614)
Orphan Drug & General Business Credit	(35,486)	(39,535)
Stock compensation expense	7,467	2,209
Foreign Source Income Subject to US Tax	44,492	47,721
Foreign tax rate differential [(1)]	(34,905)	(69,987)
Section 162(m) limitation	9,278	9,699
Tax Reserves	32,560	27,296
Intra-entity transfer of assets	(33,432)	5,019
Valuation allowance/deferred benefit	7,175	3,723
Other	(771)	(1,211)
Effective income tax rate	$ 114,904	$ 20,918

(1) For the year ended December 31, 2024, the foreign rate differential included foreign local tax expense which was at an effective rate lower than the U.S. statutory rate offset by elimination of intercompany sales. 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.

Cash paid for income taxes, net of refunds received, by jurisdiction for the year ended December 31, 2025, was as follows.

	Year Ended December 31, 2025
Federal	$ 33,000
State	8,719
Foreign	
Ireland	45,807
United Kingdom	5,027
Other	3,652
Total cash paid for income taxes	$ 96,205

(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,	
	2025	2024
Net deferred tax assets:		
Net operating loss carryforwards	$ 105,642	$ 18,585
Tax credit carryforwards	398,769	462,925
Accrued expenses, reserves, and prepaids	148,124	119,986
Intangible assets	625,084	696,096
Capitalized R&D expenses	367,200	310,081
Stock-based compensation	35,848	42,609
Lease liabilities	6,054	7,209
Inventory	37,158	19,119
Other	(219)	1,168
Valuation allowance	(181,743)	(126,311)
Total deferred tax assets	1,541,917	1,551,467
Joint venture basis difference	(989)	(1,037)
Acquired intangibles	(803)	(915)
ROU Assets	(3,911)	(4,684)
Property, plant and equipment	(27,573)	(55,923)
Total deferred tax liabilities	(33,276)	(62,559)
Net deferred tax assets	$ 1,508,641	$ 1,488,908

The increase in net deferred tax assets is primarily related to additional net operating loss carryforwards from Inozyme acquisition, capitalization of R&D expenses, impairment charges related to the divestiture of ROCTAVIAN, partially offset by valuation allowance related to acquired Inozyme net operating loss carryforwards, utilization of current year R&D credits and intangible asset amortization.

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 2025, the Company determined that it is more likely than not that part of the deferred tax assets acquired in the Inozyme acquisition related to net operating losses and R&D credits will not be realized due to Section 382 limitations and state Separate Return Limitation Year (SRLY) rules and, therefore, a valuation allowance was recorded as part of the acquisition purchase accounting. 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, 2025, 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	$ 336,545	Indefinite
Federal net operating loss carryforwards	$ 2,632	2030-2033
Federal R&D and orphan drug credit carryforwards	$ 441,294	2028-2045
State net operating loss carryforwards	$ 466,453	2025-2045
Dutch net operating loss carryforwards	$ 26,927	Indefinite

Not included in the table above are $191.3 million of state research credit carryovers that will carry forward indefinitely.

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

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.

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, 2025 and 2024, is as follows:

	December 31,	
	2025	2024
Balance at beginning of period	$ 325,035	$ 277,456
Additions based on tax positions related to the current year	48,843	47,682
Additions (reductions) for tax positions of prior years	(2,206)	(103)
Acquired Tax Positions	9,214	—
Balance at end of period	$ 380,886	$ 325,035

Included in the balance of unrecognized tax benefits as of December 31, 2025 were potential benefits of $366.8 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, 2025. 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 2022 and earlier may still be adjusted upon examination by tax authorities. The Company's 2022 federal income tax return is currently under audit by the IRS.

The Company has not provided U.S. federal and applicable foreign withholding income taxes on the undistributed earnings of certain foreign subsidiaries as such earnings are intended to be indefinitely reinvested outside the U.S. The Company is unable to reasonably estimate the amount of the unrecognized deferred tax liability associated with these undistributed earnings.

During the year ended December 31, 2025, the Company received distributions from its Irish Subsidiary's previously taxed earnings and profits (PTEP). These distributions did not result in U.S. federal income taxes or Irish withholding taxes. Any related state income tax impact was estimated to be immaterial for the year ended December 31, 2025. Going forward, the Company intends to repatriate the Irish Subsidiary's earnings to the extent that such repatriations are not restricted by local rulings, and do not incur substantial incremental costs.

(16) EARNINGS 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 per common share (common shares in thousands):

	Years Ended December 31,		
	2025	2024	2023
Numerator:			
Net income, basic	$ 348,901	$ 426,859	$ 167,645
Add: Interest expense, net of tax, on the Company's convertible debt	7,334	7,327	—
Net income, diluted	$ 356,235	$ 434,186	$ 167,645
Denominator:			
Weighted-average common shares outstanding, basic	191,787	190,027	187,834
Effect of dilutive securities:			
Common stock issuable under the Company's equity incentive plans	1,242	2,316	3,761
Common stock issuable under the Company's convertible debt [1]	4,365	4,365	—
Weighted-average common shares outstanding, diluted	197,394	196,708	191,595
Earnings per common share, basic	$ 1.82	$ 2.25	$ 0.89
Earnings per common share, diluted	$ 1.80	$ 2.21	$ 0.87

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 per common share as they were anti-dilutive (in thousands):

	Years Ended December 31,		
	2025	2024	2023
Common stock issuable under the Company's equity incentive plans	11,307	9,438	8,072
Common stock issuable under the Company's convertible debt [1]	—	—	8,335
Total number of potentially issuable shares	11,307	9,438	16,407

(1) If converted, the Company would issue 4.4 million shares under the 2027 Notes and, for the year ended December 31, 2023, would have issued 4.0 million shares under the Company's 2024 Notes, which matured and were settled in August 2024.

(17) LICENSE AND COLLABORATION AGREEMENTS

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 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.

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

(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.

As first disclosed in its Annual Report on Form 10-K for the year ended December 31, 2023, the Company 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 and financial statements.

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. As of December 31, 2025, such commitments were estimated at $590.8 million, of which $354.1 million is expected to be paid in 2026 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.

(19) RESTRUCTURING

During the fourth quarter of 2025, the Company committed to a plan to voluntarily withdraw ROCTAVIAN from the market due to lower than previously anticipated commercial opportunities. In connection with this strategic decision, the Company recorded approximately $240.0 million of restructuring charges in 2025 primarily related to inventory and long-lived assets which will no longer provide an economic benefit to the Company. The impaired ROCTAVIAN long-lived assets included dedicated facilities, specialized equipment and intangible assets.

The inventory write-off was included in Cost of Sales, and the remaining restructuring charges were included in SG&A in the Company's Consolidated Statement of Income. Restructuring charges consisted of the following:

	Year Ended December 31,
	2025
Inventory write-off	$ 119,208
Long-lived asset impairments	118,522
Severance and other related costs	3,523
	$ 241,253

The restructuring related liabilities were not material as of December 31, 2025, and were recorded in Accounts Payable and Accrued Liabilities on the in the Company's Consolidated Balance Sheet.

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

(20) ACQUISITIONS

Asset Acquisition

On July 1, 2025, the Company completed its acquisition of Inozyme, a publicly traded clinical-stage biopharmaceutical company dedicated to developing innovative therapeutics, for a total consideration of approximately $285.0 million, net of cash acquired. Upon closing, Inozyme became a wholly-owned subsidiary of the Company.

The Company accounted for this transaction as an asset acquisition since substantially all of the fair value of the gross assets acquired was concentrated in a single identifiable IPR&D asset, BMN 401 (formerly INZ-701). The Company recognized the acquired assets and assumed liabilities based on the consideration paid, inclusive of transaction costs, on a relative fair value basis. As the acquired IPR&D does not have an alternative future use, the Company recorded approximately $221.0 million in acquired IPR&D expense related to the INZ-701 IPR&D asset during the third quarter of 2025. The IPR&D charge is included in R&D on the Company's Consolidated Statements of Income.

The following summarizes the total consideration transferred and allocation to the assets acquired, liabilities assumed and acquired IPR&D:

Cash consideration for outstanding shares	$	260,424
Cash consideration for equity awards		9,950
Consideration paid to Inozyme		270,374
Payment of Inozyme debt		49,095
Employee-related incentive payments		2,714
Transaction costs		6,950
Total consideration	$	329,133
Cash and cash equivalents	$	43,939
Other assets		10,779
Deferred tax assets		68,697
Other liabilities		(15,245)
Total identifiable assets acquired, net		108,170
Acquired IPR&D		220,963
Total assets and liabilities	$	329,133

Pending Acquisition

In December 2025, the Company entered into a definitive agreement to acquire Amicus Therapeutics, Inc. (Amicus), a publicly traded, global, biotechnology company for $14.50 per share in an all-cash transaction for a total consideration of approximately $4.8 billion. The pending acquisition is expected to strengthen the Company's commercial portfolio by adding two new therapies for the treatment of Fabry disease and late-onset Pompe disease. The transaction is expected to close in the second quarter of 2026, subject to regulatory clearances, approval by the stockholders of Amicus and other customary closing conditions. The accounting treatment as a business combination or asset acquisition will be determined in the period the transaction closes.

The Company intends to finance the transaction through a combination of cash on hand and non-convertible debt financing. In December 2025, the Company entered into a debt financing commitment letter and related fee letter with certain lenders, pursuant to which the lenders have committed to provide the Company with debt financing up to approximately $3.7 billion (the Bridge Commitment) in the form of 364-day senior secured bridge loan facility (Bridge Facility) for the pending acquisition of Amicus. In place of the Bridge Facility, the Company also expects to enter into a senior secured term loan facility and a new senior secured revolving credit facility in 2026 that will be executed prior to or concurrently with the closing of the pending Amicus acquisition. No amounts have been drawn or were outstanding under the Bridge Commitment as of December 31, 2025. The Company incurred approximately $22.8 million in commitment fees related to the Bridge Commitment that were deferred and included in Other Current Assets on the Consolidated Balance Sheet as of December 31, 2025.

(21) SUBSEQUENT EVENTS

In February 2026, the Company issued $850.0 million in aggregate principal amount of 5.5% senior unsecured notes due 2034 (the 2034 Notes), and the proceeds from the issuance were deposited into an escrow account that will be used to finance the pending acquisition of Amicus. In the event that the acquisition in not completed on or prior to December 19, 2026, or upon the occurrence of certain other events, the Company will be required to redeem all of the Notes at par and pay any accrued and unpaid interest. Subsequent to issuance of the 2034 Notes, the Bridge Facility was reduced from $3.7 billion to $2.8 billion.