

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, 2020

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

(State or other jurisdiction of  
incorporation or organization)

68-0397820

(I.R.S. Employer  
Identification No.)

770 Lindaro Street San Rafael  
(Address of principal executive offices)

California

94901  
(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. Yes  No

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, 2020 was \$13.9 billion, based on the closing price reported for such date on the Nasdaq Global Select Market.

As of February 12, 2021, the registrant had 181,829,680 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 2021 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, 2020, are incorporated by reference under Part III of this Annual Report on Form 10-K.

**BIOMARIN PHARMACEUTICAL INC.  
2020 FORM 10-K ANNUAL REPORT  
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Unless the context suggests otherwise, references in this Annual Report on Form 10-K to “BioMarin,” the “Company,” “we,” “us,” and “our” refer to BioMarin Pharmaceutical Inc. and, where appropriate, its wholly owned subsidiaries.

BioMarin®, Brineura®, Kuvan®, Naglazyme®, Palynziq® and Vimizim® 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*

- The COVID-19 pandemic could materially adversely affect our business, results of operations, and financial condition.
- Because the target patient populations for our products are small, we must achieve significant market share and maintain high per-patient prices for our products to achieve profitability.
- 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.
- 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 revenue could be adversely affected.
- Changes in methods of treatment of disease could reduce demand for our products and adversely affect revenues.
- If we fail to develop new products and product candidates or compete successfully with respect to acquisitions, joint ventures, licenses or other collaboration opportunities, our ability to continue to expand our product pipeline and our growth and development would be impaired.
- The sale of generic versions of Kuvan by generic manufacturers has adversely affected and may continue to adversely affect our revenue and results of operations.

- If we do not achieve our projected development goals in the timeframes we announce and expect, the commercialization of our product candidates may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

#### *Regulatory Risks*

- If we fail to obtain regulatory approval to commercially market and sell our product candidates, or if approval of our product candidates is delayed, we will be unable to generate revenue 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 Food and Drug Administration (FDA), 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 revenue 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 revenue and results of operations.
- Government healthcare reform could increase our costs and adversely affect our revenue and results of operations.

#### *Risks Related to Valoctocogene Roxaparvovec*

- Our valoctocogene roxaparvovec program is based on a gene therapy approach, which, as a novel technology, presents additional development and treatment risks in relation to our other, more traditional drug development programs.
- As compared to our other, more traditional products, our gene therapy product candidate valoctocogene roxaparvovec, if approved, may present additional problems with respect to the pricing, coverage, and reimbursement and acceptance of the product candidate.

#### *Financial and Financing Risks*

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

#### *Manufacturing Risks*

- If we fail to comply with manufacturing regulations, our financial results and financial condition will be adversely affected.
- If we are unable to successfully develop and maintain manufacturing processes for our product candidates to produce sufficient quantities at acceptable costs, we may be unable to support a clinical trial or be forced to terminate a program, or if we are unable to produce sufficient quantities of our products at acceptable costs,

we may be unable to meet commercial demand, lose potential revenue, have reduced margins or be forced to terminate a program.

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

*Risks Related to International Operations*

- We conduct a significant amount of our sales and operations outside of the United States (U.S.), which subjects us to additional business risks that could adversely affect our revenue 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 revenue in these countries.

*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 global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare diseases and medical conditions. We select product candidates for diseases and conditions that represent a significant unmet medical need, have well-understood biology and provide an opportunity to be first-to-market or offer a significant benefit over existing products.

Our portfolio consists of several commercial products and multiple clinical and preclinical product candidates for the treatment of various diseases. We continue to invest in our clinical and preclinical product pipeline by committing significant resources to research and development programs and business development opportunities within our areas of scientific, manufacturing and technical expertise.

A summary of our commercial products is provided below:

Commercial Products	Indication	United States Orphan Drug Exclusivity Expiration <sup>(1)</sup>	United States Biologic Exclusivity Expiration <sup>(2)</sup>	European Union Orphan Drug Exclusivity Expiration <sup>(1)</sup>
Aldurazyme (laronidase)	MPS I <sup>(3)</sup>	Expired	Expired	Expired
Brineura (cerliponase alfa)	CLN2 <sup>(4)</sup>	2024	2029	2027
Kuvan (sapropterin dihydrochloride)	PKU <sup>(5)</sup>	Expired	Not Applicable	Expired
Naglazyme (galsulfase)	MPS VI <sup>(6)</sup>	Expired	Expired	Expired
Palyzinq (pegvaliase-pqpz) <sup>(7)</sup>	PKU	2025	2030	2029
Vimizim (elosulfase alpha)	MPS IVA <sup>(8)</sup>	2021	2026	2024

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

(2) See “Government Regulation—Healthcare Reform” in this Annual Report on Form 10-K for further discussion

(3) For the treatment of Mucopolysaccharidosis I (MPS I)

(4) For the treatment of late infantile neuronal ceroid lipofuscinosis type 2 (CLN2)

(5) For the treatment of phenylketonuria (PKU)

(6) For the treatment of Mucopolysaccharidosis VI (MPS VI)

(7) For adult patients with PKU

(8) For the treatment of Mucopolysaccharidosis IV Type A (MPS IVA)

A summary of our ongoing major development programs is provided below:

Major Product Candidates in Development	Target Indication	U.S. Orphan Designation	EU Orphan Designation	Stage
Valoctocogene roxaparvovec	Severe Hemophilia A	Yes	Yes	Clinical Phase 3
Vosoritide	Achondroplasia	Yes	Yes	Clinical Phase 3
BMN 307	PKU	Yes	Yes	Clinical Phase 1/2

See “Patents and Proprietary Rights” in this Annual Report on Form 10-K for additional information on our market protection.

Recent Developments

*Regulatory Review of Vosoritide for the Treatment of Achondroplasia*

On February 25, 2021, we announced that the FDA granted priority review designation for our New Drug Application (NDA) for vosoritide for the treatment of children with achondroplasia. On November 2, 2020, we announced that the U.S. Food

and Drug Administration (FDA) accepted our vosoritide NDA and the FDA Prescription Drug User Fee Act (PDUFA) target action date is August 20, 2021. See “Major Product Candidates in Development — Vosoritide” in this Annual Report on Form 10-K for more information regarding vosoritide.

***Product Candidate BMN 307 for the Treatment of PKU***

On February 25, 2021, we announced that we plan to dose escalate in the PHEarless Phase 1/2 study of BMN 307, a gene therapy for the treatment of PKU, based on encouraging Phe lowering and safety signals observed in study participants who were treated with the lowest dose.

***Product Candidate BMN 255 for the Treatment of a Subset of Chronic Renal Disease***

On January 11, 2021, we announced that we filed an Investigational New Drug application (IND) for BMN 255, a small molecule for the treatment of a subset of chronic renal disease.

***Updated Phase 3 Data for Product Candidate Valoctocogene Roxaparovec for the Treatment of Severe Hemophilia A***

On January 10, 2021, we announced topline results from our ongoing global Phase 3 study of valoctocogene roxaparovec for the treatment of adults with severe hemophilia A. All 134 study participants received a single dose of valoctocogene roxaparovec and completed a year or more of follow-up. Data from the study in the pre-specified primary analysis for Annualized Bleeding Rate (ABR) showed that a single dose of valoctocogene roxaparovec significantly reduced ABR by 84% compared with prior treatment with prophylactic Factor VIII infusions from 4.8 (median 2.8) bleeding episodes per year at baseline to 0.8 (median 0.0). These results were from a pre-specified group of 112 participants in a non-interventional prospective baseline observational study with a median follow-up of 60.1 weeks after dosing with valoctocogene roxaparovec. Of the 112 rollover study participants, 80% were bleed-free starting at week five after treatment. Valoctocogene roxaparovec also significantly reduced the mean annualized Factor VIII usage in the rollover population by 99%, from 135.9 (median 128.6) to 2.0 (median 0.0) infusions per year. At the end of the first year post-infusion with valoctocogene roxaparovec, 132 participants in the modified intent-to-treat (mITT) population had a mean endogenous Factor VIII expression level of 42.9 (SD 45.5, median 23.9) IU/dL, as measured by the chromogenic substrate assay, supporting the clinical benefits observed with significant reduction of bleeding episodes and Factor VIII infusion rate. Factor VIII expression declined at a slower rate compared to the Phase 1/2 study, and remained in a range to provide hemostatic efficacy. In the 17 participants who comprise a subset of the mITT population and had been dosed at least two years prior to the data cut date, Factor VIII expression declined from a mean of 42.2 (SD 50.9, median 23.9) IU/dL at the end of year one to a mean of 24.4 (SD 29.2, median 14.7) IU/dL at the end of year two with continued hemostatic efficacy demonstrated by a mean ABR of 0.9 (median 0.0) bleeding episodes per year. See “Major Product Candidates in Development — Valoctocogene Roxaparovec” in this Annual Report on Form 10-K for more information regarding valoctocogene roxaparovec.

***Two-Year Phase 3 Data for Product Candidate Vosoritide for the Treatment of Achondroplasia***

On December 21, 2020, we announced results from our pivotal global Phase 3 randomized, double-blind, placebo-controlled study of vosoritide in approximately 121 children with achondroplasia ages 5-14 for two years. The data demonstrated that children in the open-label long-term extension of the Phase 3 study maintained an increase in Annual Growth Velocity (AGV) through the second year of continuous treatment. An analysis comparing 52 children who were randomized and treated with vosoritide for two years to 38 children from the run-in study who were randomized to receive placebo with an untreated observation period of two years showed improvement in one-year height change in the treated group relative to the untreated group. The one-year height change improvement in the second year of treatment, 1.79 cm, was similar to the one-year height change improvement in the first year of treatment, 1.73 cm. The cumulative height gain over the two-year period for treated children was 3.52 cm more than untreated children. See “Major Product Candidates in Development — Vosoritide” in this Annual Report on Form 10-K for more information regarding vosoritide.

**Summary of Commercial Products and Development Programs****Commercial Products**

Net Product Revenues related to our commercial products consisted of the following (in millions):

Commercial Products	Years Ended December 31,		
	2020	2019	2018
Vimizim	\$ 544.4	\$ 544.3	\$ 482.0
Kuvan	\$ 457.7	\$ 463.4	\$ 433.6
Naglazyme	\$ 391.3	\$ 374.3	\$ 345.9
Palyzniq	\$ 171.0	\$ 86.9	\$ 12.2
Aldurazyme	\$ 130.1	\$ 97.8	\$ 135.1
Brineura	\$ 110.2	\$ 72.0	\$ 39.9

**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 glycosaminoglycans 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. We have identified over 2,000 patients worldwide suffering from MPS IVA and estimate that the total number of patients suffering from MPS IV A worldwide could be as many as 3,000.

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

**Kuvan**

Kuvan is a proprietary synthetic oral form of 6R-BH4, a naturally occurring enzyme co-factor for phenylalanine hydroxylase (PAH), indicated for patients with PKU. Kuvan is the first drug for the treatment of PKU, which is an inherited metabolic disease that affects at least 50,000 diagnosed patients under the age of 40 in the developed world. We believe that approximately 30% to 50% of those with PKU could benefit from treatment with Kuvan. PKU is caused by a deficiency of activity of an enzyme, PAH, which is required for the metabolism of Phe. Phe is an essential amino acid found in all protein-containing foods. Without sufficient quantity or activity of PAH, Phe accumulates to abnormally high levels in the blood, resulting in a variety of serious neurological complications, including severe mental retardation and brain damage, mental illness, seizures and other cognitive problems. As a result of newborn screening efforts implemented in the 1960s and early 1970s, virtually all PKU patients under the age of 40 in developed countries have been diagnosed at birth. Currently, 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.

Kuvan tablets were granted marketing approval for the treatment of PKU in the U.S. in December 2007 and in the EU in December 2008. In December 2013, the FDA approved the use of Kuvan powder for oral solution that is provided in a dose sachet packet allowing faster dissolution of powder in solution compared to the current tablet form. We commenced the commercial launch of this new form of Kuvan in February 2014. We market Kuvan 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.

Two companies previously filed paragraph IV certifications and submitted abbreviated new drug applications (ANDAs) to produce sapropterin dihydrochloride tablets and powder and we subsequently entered into settlement agreements regarding Kuvan with both companies. Generic versions of Kuvan first became available in the U.S. in the fourth quarter of 2020. Please see "Risk Factors" included in Part I, Item 1A of this Annual Report on Form 10-K for more information regarding the settlement agreements and for a discussion of the risks posed by generic versions of Kuvan. Please see "Government Regulation – The Hatch-Waxman Act" in this Annual Report on Form 10-K for additional information regarding ANDAs.



### *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 certain complex carbohydrates known as glycosaminoglycans (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 ammonia lyase enzyme, which is delivered through subcutaneous injection to reduce blood Phe concentrations. On May 24, 2018, the FDA approved the use of Palynziq in adult patients with PKU who have uncontrolled blood Phe concentrations greater than 600 micromol/L (10mg/dL) on existing management. In October 2020, we announced that the FDA approved our supplemental Biologics License Application (BLA) to increase the maximum allowable dose of Palynziq Injection for treatment of adults with PKU to 60 mg daily. Previously, the maximum dose was 40 mg daily. Palynziq is our second approved treatment for PKU. Palynziq is only available in the U.S. through the Palynziq Risk Evaluation and Mitigation Strategy (REMS) program, which is required by the FDA to mitigate the risk of anaphylaxis while using the product. Notable requirements of our REMS program include the following:

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

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

On May 6, 2019, we announced that the European Commission (EC) granted marketing authorization for Palynziq in the EU, at doses of up to 60 mg once daily, to reduce Phe concentrations in patients with PKU aged 16 and older who have inadequate blood Phe control (blood Phe levels greater than 600 micromol/L) despite prior management with available treatment options.

### *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 glycosaminoglycans (GAGs). Aldurazyme is approved for marketing in the U.S., the EU and other international markets for patients with MPS I. MPS I is a progressive and debilitating life-threatening genetic disease, for which no other drug treatment currently exists, 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 Genzyme (Genzyme), now a wholly owned subsidiary of Sanofi. Under our collaboration agreement with Genzyme, we are responsible for manufacturing Aldurazyme and supplying it to Genzyme. We receive payments ranging from 39.5% to 50% on worldwide net Aldurazyme sales by Genzyme depending on sales volume. Genzyme and we 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 us and Genzyme 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 Genzyme and us.

## Brineura

Brineura is a recombinant human tripeptidyl peptidase 1 (TPP1) and is approved for the treatment of patients with CLN2, a form of Batten disease, in the U.S., the EU and other international markets. 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. We estimate that up to 1,200 to 1,600 cases exist worldwide. On April 27, 2017, Brineura was approved in the U.S. to slow the progression of loss of ambulation in symptomatic pediatric patients three years of age and older with CLN2. Brineura is the first treatment approved to slow the progression of loss of ambulation in children with CLN2 disease.

On June 1, 2017, we announced that the EC granted marketing authorization for Brineura in the EU to treat children with CLN2. Brineura is the first treatment approved in the EU for the treatment of CLN2. On April 21, 2017, the Committee for Medicinal Products for Human Use (CHMP), the scientific committee of the European Medicines Agency (EMA) adopted a positive opinion for our Marketing Authorisation Application (MAA) for Brineura following an accelerated review procedure, reserved for medicinal products expected to be a major public health interest. Brineura was one of the first therapies to go through this process.

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 – Combination Products" in this Annual Report on Form 10-K for additional information on combination products.

## Major Product Candidates in Development

### *Valoctocogene Roxaparvovec*

Valoctocogene roxaparvovec is an adeno associated virus (AAV5) vector drug development candidate designed to restore factor VIII plasma concentrations in patients with severe hemophilia A. Hemophilia A, also called factor VIII deficiency or classic hemophilia, is a genetic disorder caused by missing or defective factor VIII, a clotting protein. According to the World Federation of Hemophilia rankings of severity of hemophilia A, the normal range of factor VIII activity levels is between 50% and 150%, expressed as a percentage of normal factor activity in blood, the mild hemophilia A range of factor VIII activity levels is between 5% and 40%, the moderate hemophilia A range of factor VIII activity levels is between 1% and 5%, and the severe hemophilia range of factor VIII activity levels is less than 1%. People living with hemophilia A are not able to form blood clots efficiently and are at risk for excessive bleeding from modest injuries, potentially endangering their lives. People with severe hemophilia often bleed spontaneously into their muscles or joints.

On May 21, 2020, we announced an update to our previously reported results of an open-label Phase 1/2 study of valoctocogene roxaparvovec for the treatment of adults with severe hemophilia A. The four-year update for the 6e13 vg/kg and three-year update for the 4e13 vg/kg cohorts demonstrated that all subjects in both cohorts had remained off prophylactic Factor VIII treatment since receiving their single dose of valoctocogene roxaparvovec. In addition, cumulative mean annualized bleed rates (ABR) remain less than one in both cohorts and below pre-treatment baseline levels.

On January 10, 2021, we announced topline results from our ongoing global Phase 3 study of valoctocogene roxaparvovec for the treatment of adults with severe hemophilia A. All 134 study participants received a single dose of valoctocogene roxaparvovec and completed a year or more of follow-up. Data from the study in the pre-specified primary analysis for ABR showed that a single dose of valoctocogene roxaparvovec significantly reduced ABR by 84% compared with prior treatment with prophylactic Factor VIII infusions from 4.8 (median 2.8) bleeding episodes per year at baseline to 0.8 (median 0.0). These results were from a pre-specified group of 112 participants in a non-interventional prospective baseline observational study with a median follow-up of 60.1 weeks after dosing with valoctocogene roxaparvovec. Of the 112 rollover study participants, 80% were bleed-free starting at week five after treatment. Valoctocogene roxaparvovec also significantly reduced the mean annualized Factor VIII usage in the rollover population by 99%, from 135.9 (median 128.6) to 2.0 (median 0.0) infusions per year. At the end of the first year post-infusion with valoctocogene roxaparvovec, 132 participants in the modified intent-to-treat (mITT) population had a mean endogenous Factor VIII expression level of 42.9 (SD 45.5, median 23.9) IU/dL, as measured by the chromogenic substrate assay, supporting the clinical benefits observed with significant reduction of bleeding episodes and Factor VIII infusion rate. Factor VIII expression declined at a slower rate compared to the Phase 1/2 study, and remained in a range to provide hemostatic efficacy. In the 17 participants who comprise a subset of the mITT population and had been dosed at least two years prior to the data cut date, Factor VIII expression declined from a mean of 42.2 (SD 50.9, median 23.9) IU/dL at the end of year one to a mean of 24.4 (SD 29.2, median 14.7) IU/dL at the end of year two with continued hemostatic efficacy demonstrated by a mean ABR of 0.9 (median 0.0) bleeding episodes per year.

In addition to the ongoing Phase 1/2 and Phase 3 studies of valoctocogene roxaparvovec described above, we have multiple other clinical studies of valoctocogene roxaparvovec underway. We recently began enrolling participants in a Phase 3b, single arm, open-label study to evaluate the efficacy and safety of valoctocogene roxaparvovec at a dose of 6e13 vg/kg with prophylactic corticosteroids in people with severe hemophilia A. We are conducting a Phase 1/2 study with the 6e13kg/vg dose of valoctocogene roxaparvovec in approximately 10 participants with pre-existing AAV5 antibodies, as well as another Phase 1/2 Study with the 6e13 vg/kg dose of valoctocogene roxaparvovec in people with severe hemophilia A with active or prior FVIII inhibitors.

Overall, valoctocogene roxaparovec continues to have a favorable safety profile and has been generally well tolerated by participants across all doses in the Phase 1/2 and Phase 3 studies. None of the study participants developed inhibitors to Factor VIII or thromboembolic events. One participant in the Phase 3 study was lost to follow-up. The most common adverse events associated with valoctocogene roxaparovec across studies occurred early and included transient infusion-associated reactions and transient, asymptomatic, and mild to moderate rises in the levels of certain proteins and enzymes measured in liver function tests. Common, steroid-related side effects can occur with temporary use of corticosteroid (or alternative immunosuppressants) to manage elevated levels of certain proteins and enzymes in the liver. These side effects have generally been mild to moderate, manageable and reversible.

On February 20, 2020, we announced that the FDA accepted for priority review our BLA for valoctocogene roxaparovec for the treatment of adults with severe hemophilia A. On August 18, 2020, the FDA issued a Complete Response Letter (CRL) to our BLA for valoctocogene roxaparovec for the treatment of adults with severe hemophilia A. In the CRL, the FDA introduced a new request for two-year follow-up safety and efficacy data on all study participants from our ongoing Phase 3 study of valoctocogene roxaparovec. The Phase 3 study was fully enrolled in November 2019, and the last patient in the study will complete two years of follow-up in November 2021. We plan to meet with the FDA to review the two-year follow-up data request and share the one-year top-line Phase 3 data described above.

On December 23, 2019, we announced that the EMA validated our MAA for valoctocogene roxaparovec for the treatment of adults with severe hemophilia A, which had been in review under accelerated assessment since January 2020. The EMA recently requested the 52-week results from the full Phase 3 study cohort of 134 subjects to inform their benefit-risk assessment. To facilitate this submission within the EMA regulatory framework, we recently withdrew our MAA and we are targeting resubmission of the MAA with these data in the second quarter of 2021 pending confirmation in presubmission meetings.

Valoctocogene roxaparovec has Orphan Drug designation from the FDA and the EMA. Valoctocogene roxaparovec has also been accepted for Priority Medicines (PRIME) scheme from the EMA. Additionally, the FDA has granted valoctocogene roxaparovec Breakthrough Therapy designation.

### *Vosoritide*

Vosoritide is an investigational, once daily injection analog of C-type Natriuretic Peptide (CNP) in development for the treatment of achondroplasia, the most common form of disproportionate short stature in humans. Vosoritide has been and is being tested only in children in the age range when their growth plates are still open, which is approximately 25% of people with achondroplasia. In June 2019, the *New England Journal of Medicine* published results from our Phase 2 open-label, dose-finding and extension study for vosoritide in children aged 5 to 14 years with achondroplasia receiving a continuous dose of 15 µg/kg/day. AGV increased from baseline in all cohorts during each 12-month interval by 1.10 to 2.34 cm/year through 42 months. In cohort 3, which received 15 µg/kg continuous dosing from baseline, the mean AGV derived between 30 and 42 months was 5.51 cm/year, representing a 1.46 cm/year change from baseline. On November 14, 2019, we announced that children in cohort 3 over 54 months achieved a statistically significant cumulative additional mean height gain of 9.0 cm compared to children, matched for age and gender, in a natural history achondroplasia dataset. In the global Phase 2 study of children with achondroplasia ages 0-5, enrollment of the first cohort (24-60 months) and the second cohort (6-24 months) is complete. Cohort 3 (0-6 months) is actively enrolling and close to completely enrolled.

On December 21, 2020, we announced results from our pivotal global Phase 3 randomized, double-blind, placebo-controlled study of vosoritide in approximately 121 children with achondroplasia ages 5-14 for two years. The data demonstrated that children in the open-label long-term extension of the Phase 3 study maintained an increase in AGV through the second year of continuous treatment. An analysis comparing 52 children who were randomized and treated with vosoritide for two years to 38 children from the run-in study who were randomized to receive placebo with an untreated observation period of two years showed improvement in one-year height change in the treated group relative to the untreated group. The one-year height change improvement in the second year of treatment, 1.79 cm, was similar to the one-year height change improvement in the first year of treatment, 1.73 cm. The cumulative height gain over the 2-year period for treated children was 3.52 cm more than untreated children.

On November 9, 2020, we announced an expansion of our clinical program for vosoritide with two new Phase 2 studies. The first study is sponsored by BioMarin to investigate the safety of vosoritide in infants with achondroplasia at risk of life-threatening foramen magnum compression. The second study, an investigator-initiated study sponsored by Children's National Hospital in Washington, D.C., will investigate vosoritide in children with selected genetic forms of short stature. Together, these two studies represent addressable patient populations of approximately 275,000.

Overall, vosoritide continues to have a favorable safety profile and has been generally well tolerated by participants across the Phase 2 and Phase 3 studies with no clinically significant blood pressure changes and a mild side effect profile.

On February 25, 2021, we announced that the FDA granted priority review designation for our NDA for vosoritide for the treatment of children with achondroplasia. On November 2, 2020, we announced that the FDA accepted our NDA for vosoritide for the treatment of children with achondroplasia and the FDA PDUFA target action date is August 20, 2021. When the FDA accepted

our NDA for vosoritide, the agency reiterated its position raised during the vosoritide Pediatric Advisory Committee and Endocrinologic and Metabolic Drugs Advisory Committee held in May 2018, recommending two, two-year placebo-controlled trials in different age groups. Although our vosoritide clinical program does not meet every element of the FDA's recommendation, we designed our studies of vosoritide in a manner that we believe can demonstrate efficacy and safety of the product candidate for the target patient population. However, the FDA may ultimately disagree with our position that our vosoritide trials support regulatory approval.

On August 13, 2020, we announced that the EMA validated our MAA for vosoritide for the treatment of children with achondroplasia. We anticipate an opinion from the CHMP on our MAA for vosoritide in June 2021.

Vosoritide has Orphan Drug designation from the FDA and the EMA.

#### *BMN 307*

BMN 307 is an AAV5 mediated gene therapy that is designed to normalize blood Phe concentration levels in patients with PKU. We tested a broad range of vector constructs and combinations to optimize the vector and increase potency, resulting in a 10-fold increase in potency for the selected vector. Treatment of mice in a validated PKU mouse model with BMN 307 showed a lifetime normalization of Phe and normalized neurotransmitter levels. BMN 307 will be evaluated to determine safety and whether a single dose of treatment can restore natural Phe metabolism, normalize plasma Phe levels, and enable a normal diet in patients with PKU. On January 13, 2020, we announced that both the FDA and the Medicines and Healthcare Products Regulatory Agency (MHRA) in the United Kingdom (U.K.) granted IND status and approved our Clinical Trial Application (CTA), respectively, for BMN 307 for the treatment of PKU. On September 24, 2020, we announced that we had dosed the first participant in the global PHEarless Phase 1/2 study of BMN 307. On February 25, 2021, we announced that we plan to dose escalate in the PHEarless Phase 1/2 study of BMN 307 based on encouraging Phe lowering and safety signals observed in study participants who were treated with the lowest dose.

BMN 307 has Orphan Drug designation from the FDA and the EMA and Fast Track designation from the FDA.

#### **Manufacturing**

We manufacture the active pharmaceutical ingredients (API) for Aldurazyme, Naglazyme, Palynziq, Vimizim and vosoritide in our production facilities located in Novato, California. We currently also manufacture the API for Brineura and Vimizim in our manufacturing facility in Shanbally, Cork, Ireland. These facilities have demonstrated compliance with current Good Manufacturing Practices (cGMPs) to the satisfaction of the FDA, the EC and health agencies in other countries for the commercial production of Naglazyme, Palynziq, and Vimizim. We believe that with our Novato, California facility and our Shanbally facility, we have ample manufacturing capacity to support anticipated commercial demand for Aldurazyme, Brineura, Naglazyme, Palynziq and Vimizim for at least the next five years.

We contract with third parties to manufacture Kuvan API and drug substance. All our drug product manufacturing (which includes vials, syringes, tablets, and powder) and most packaging operations are performed by 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 sales. 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.

In July 2017, we commissioned our commercial-scale gene therapy manufacturing facility, located in Novato, California, where we conduct cGMP production of valoctocogene roxaparvovec and BMN 307 to support clinical development activities and anticipated commercial demand. This facility has the potential to support the commercial production of multiple gene therapy products to meet global commercial demand, depending on dose and production mix. The facility's production processes have been developed in accordance with International Conference on Harmonisation Technical Requirements for Registration of Pharmaceuticals for Human Use facilitating worldwide registration with health authorities.

## **Raw Materials**

Raw materials and supplies required for the production of 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.

## **Sales and Marketing**

We have established a commercial organization, including a sales force, to support our product lines directly in the U.S., Europe, South America and certain other significant markets. For other selected markets, we have signed agreements with other companies to act as distributors of Brineura, Kuvan, Naglazyme and Vimizim. 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.

Genzyme 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 2021, the size of our sales force will be appropriate to effectively reach our target customers in markets where our products are directly marketed. The launch of any future products, if approved, will likely require expansion of our commercial organization, including our sales force, in the U.S. and international markets.

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

## **Customers**

Our Brineura, Kuvan, Naglazyme, Palynziq and Vimizim customers include a limited number of specialty pharmacies and end-users, such as hospitals and non-U.S. government agencies. We also sell Brineura, Kuvan, Naglazyme, Palynziq and Vimizim 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, such as those in Latin America, governments place large periodic orders for Naglazyme and Vimizim. The timing of these orders can be inconsistent and can create significant quarter to quarter variation in our revenue. Palynziq is currently distributed in the U.S. pursuant to the REMS program through a limited number of certified specialty pharmacies. During 2020, 45% of our net product revenues, excluding Aldurazyme, was generated by three customers. Genzyme is our sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third parties.

## **Competition**

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 competitors may have advantages over us due to greater financial or scientific resources, lower labor and other costs, or due to higher headcount and more robust organizational structures. 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 Kuvan (which faces generic competition, 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.

### **Commercial Products**

#### *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., Orchard Therapeutics Plc, RegenxBio Inc. and earlier stage product candidates, including a product candidate from Immusoft Corporation. Naglazyme, for the treatment of MPS VI, has potential competition from a clinical stage product candidate from Inventiva S.A. and other potential candidates in earlier stages. Vimizim, for the treatment of MPS IVA, has potential competition from a preclinical product candidate from Esteve Pharmaceuticals, S.A. and other potential candidates in earlier stages.

#### *Brineura*

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

#### *Kuvan and Palyzinq*

There are currently no other approved, non-generic drugs on the market in the U.S. or the EU for the treatment of PKU. However, two companies previously filed paragraph IV certifications and submitted ANDAs to produce sapropterin dihydrochloride tablets and powder. We entered into settlement agreements regarding Kuvan with both companies, which allowed these companies to market generic versions of sapropterin dihydrochloride. Generic versions of Kuvan first became available in the U.S. in October 2020. Please see "Risk Factors" included in Part I, Item 1A of this Annual Report on Form 10-K for more information regarding the settlement agreements and for a discussion of the risks posed by generic versions of Kuvan in the U.S. and international markets. Please see "Government Regulation – The Hatch-Waxman Act" in this Annual Report on Form 10-K for additional information regarding ANDAs. Kuvan and Palyzinq also have potential competition from clinical stage product candidates from Homology Medicines, Inc., Nestle Health Science, S.A., PTC Therapeutics, Inc. and Synlogic, Inc. and earlier stage product candidates, including product candidates from Generation Bio Co., LogicBio Therapeutics, Inc., Moderna Therapeutics, Inc., Sangamo Therapeutics, Inc. and SOM Innovation Biotech, S.A. BMN 307 is our clinical gene therapy program for PKU, and other companies are also developing gene therapy product candidates for PKU, as described in "Competition—Product Candidates—BMN 307" in this Annual Report on Form 10-K.

### **Major Product Candidates in Development**

#### *Valoctocogene roxaparvovec*

Valoctocogene roxaparvovec, a gene therapy product candidate for severe hemophilia A, could have competition from marketed recombinant factor VIII replacement therapies, a novel bispecific antibody marketed by the Roche Group, and clinical stage programs, including gene therapy product candidates under development by Bayer AG, Pfizer, Inc., the Roche Group, Sangamo Therapeutics, Inc., Takeda Pharmaceutical Company Ltd. and preclinical product candidates from other companies, including Freeline Therapeutics Ltd. and Uniqure N.V. In addition, Novo Nordisk A/S, Pfizer, Inc. and Sanofi S.A. are developing novel non-factor replacement product candidates in the clinic for the treatment of hemophilia A.

#### *Vosoritide*

Vosoritide, for the treatment of achondroplasia, could have competition from clinical stage products under development by Ascendis Pharma A/S and Pfizer, Inc., and preclinical product candidates from other companies, including QED Therapeutics, Inc.

#### *BMN 307*

BMN 307, a gene therapy product candidate for the treatment of PKU, has potential competition from clinical stage product candidates from PTC Therapeutics, Inc., Homology Medicines, Inc., Nestle Health Science, S.A., Synlogic, Inc. and earlier



stage product candidates, including product candidates from Generation Bio Co., LogicBio Therapeutics, Inc., Moderna Therapeutics, Inc., Sangamo Therapeutics, Inc. and SOM Innovation Biotech, S.A.

### BMN 331

BMN 331, a gene therapy product candidate for the treatment of hereditary angioedema, has potential competition from marketed therapies by BioCryst Pharmaceuticals, Inc., CSL Limited and Takeda Pharmaceutical Company Ltd., clinical stage product candidates from CSL Limited, Ionis Pharmaceuticals, Inc., Kalvista Pharmaceuticals, Inc. and Pharvaris GmbH and earlier stage product candidates from Intellia Therapeutics, Inc. and Regenxbio Inc.

### Patents and Proprietary Rights

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

The table below lists our outstanding patents and patent applications of primary importance for our products other than Aldurazyme 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, the last of our patents expired in November 2020. 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. In addition to patent protection, certain of our products are entitled to regulatory exclusivity in the U.S. and the EU. For more information regarding regulatory exclusivity for our products, see the summary of our commercial products under "Overview" in Part I, Item 1 of this Annual Report on Form 10-K.

Product	Territory	Patent No(s).	General Subject Matter	Patent Expiration
Brineura	U.S.	8,029,781	Method of treatment	July 3, 2023 <sup>(1)</sup>
		9,044,473	Method of treatment by administration into the cerebrospinal fluid	February 18, 2032
		10,279,015	Formulation; kit	May 5, 2036
	EU	1,673,104	Pharmaceutical composition	August 30, 2024
		16793229.2 <sup>(2)</sup>	Formulation	May 5, 2036
Kuvan	EU	3138566 <sup>(2)</sup>	Use for treating with once daily dosing regimen	November 7, 2024
		2545939; 3461503 <sup>(2)</sup>	Use for treating once daily after a meal	April 11, 2028
Naglazyme	U.S.	6,866,844	Compositions; methods of treatment; production; purification	November 7, 2022
		7,713,709	Antibody assays	July 20, 2028
	EU	1565209; 2327414	Compositions; pharmaceutical compositions; use to treat an enzyme deficiency	November 7, 2023
Palyzniq	U.S.	7,534,595	Composition; method of treating	August 16, 2027 / May 24, 2032 <sup>(3)</sup>
		10,221,408	Purification	February 3, 2031
		9,557,340	Antibody detection assay	July 30, 2029
	EU	2152868	Composition; pharmaceutical composition	May 23, 2028 / May 23, 2033 <sup>(4)</sup>
		2531209; 3025728	Formulation; purification	February 03, 2031
Vimizim	U.S.	8,128,925	Compositions; methods of treatment	April 10, 2030
		8,765,437	Purification; formulation; methods of treatment	January 10, 2032
	EU	2245145	Composition; use for treating	April 30, 2029
		2595650	Purification; composition; use for treating; formulation	July 22, 2031

- (1) Under a patent term extension (PTE) that has been granted.
- (2) Patent application.
- (3) We filed for a PTE for this patent, and if granted, the patent expiration will extend to May 24, 2032.
- (4) We applied for Supplementary Protection Certificate (SPC) for this patent, and we have received SPC to extend the patent expiration to May 23, 2033 in certain European countries, including Austria, Cyprus, Estonia, Hungary, Italy, Latvia, Lithuania, Luxembourg, Malta, Netherlands, Spain, Slovenia, and Sweden.

With respect to our clinical product candidates, we believe we have the necessary intellectual property rights to allowing 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. 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 present and future business has been, and will continue to be, subject to a variety of laws in the U.S. and other jurisdictions. In the U.S., failure to comply with applicable U.S. requirements may subject a company to a variety of administrative or judicial sanctions, such as FDA refusal to approve pending NDAs or BLAs, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties, and criminal prosecution.

Our products require approval from the FDA, the EMA and corresponding agencies in other countries before they can be marketed.

### ***Approval Process in the U.S. and EU***

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 or a CTA), 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. Currently, European clinical trial authorization applications must be submitted to the competent authority in each EU Member State in which the trial will be conducted. Under the new European Regulation on Clinical Trials, which is expected to take effect in 2021, there will be a centralized application procedure where one national authority takes the lead in reviewing the application and the other national authorities have only a limited involvement. Satisfaction of FDA and European 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.

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 EMA regulations and requirements, including good laboratory practices. The results of preclinical testing, along with other information, including information about product chemistry, manufacturing and controls and a proposed clinical trial protocol are submitted to 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, we may not start the clinical trial in the relevant jurisdiction.

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

The regulatory agencies may order the temporary halt or permanent discontinuation of a clinical trial at any time or impose other sanctions if they believe that the clinical trial is not being conducted in accordance with applicable requirements or presents



an unacceptable risk to the clinical trial patients. The study protocol and informed consent information for patients in clinical trials must also be submitted to an institutional review board (IRB) or ethics committee, for approval. 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 to support NDAs, BLAs or MAAs for marketing approval are typically conducted in three sequential phases, but the phases may overlap or be combined. In Phase 1, the initial introduction of the drug into healthy human subjects or patients, the drug is tested to assess metabolism, pharmacokinetics, pharmacological actions, side effects associated with increasing doses and, if possible, early evidence on effectiveness. Phase 2 usually involves trials in a limited patient population, to determine the effectiveness of the drug for a particular indication or indications, dosage tolerance and optimum dosage, and to identify common adverse effects and safety risks. If a compound demonstrates evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations, Phase 3 trials are 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 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), an MAA is prepared and, for all orphan designated products, is submitted to the EMA under the centralized application procedure. EC approval of the MAA under the centralized application procedure results in a single marketing authorization that is valid across the EEA. 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.

The FDA and the EMA initially review the applications for a threshold determination that it is sufficiently complete to permit substantive review, typically within 30-60 days. 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 is typically an additional ten months 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 an additional six months in duration. The review process may be extended by the FDA for three additional months to consider new information submitted during the review or clarification regarding information already provided in the submission. The FDA may also refer applications for novel products or products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations. After the FDA evaluates the information provided in the NDA/BLA, it issues an approval letter, or a complete response letter. A complete response letter outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed and the NDA/BLA has been resubmitted, the FDA will re-initiate review. If it is satisfied that the deficiencies have been addressed, the FDA will issue an approval letter.

Under the centralized procedure in the EU, the maximum timeframe for the evaluation of an MAA by the EMA is 210 days. This excludes so-called clock stops, during which additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP. At the end of the review period, the CHMP provides an opinion to the EC. If the opinion is favorable, the EC may then adopt a decision to grant marketing authorization. In the event of a negative opinion, the company may request a re-examination of the application within 15 days of receipt of the negative opinion. The company then has 60 days to provide the CHMP with detailed grounds for requesting the re-examination. Within 60 days of providing this information, the CHMP shall 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 EMA will typically inspect one or more clinical sites and/or the sponsor to assure compliance with GCP regulations and will inspect the facility or the facilities at which the drug is manufactured to ensure compliance with cGMPs regulations. Neither the FDA nor the EMA 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.

A marketing approval authorizes commercial marketing of the drug with specific prescribing information for specific indications. 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. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

## **Combination Products**

A combination product is a product comprising (i) two or more regulated components, i.e., drug/device, biologic/device, drug/biologic, or drug/device/biologic, that are physically, chemically, or otherwise combined or mixed and produced as a single entity; (ii) two or more separate products packaged together in a single package or as a unit and comprising drug and device products, device and biological products, or biological and drug products; (iii) a drug, device, or biological product packaged separately that according to its investigational plan or proposed labeling is intended for use only with an approved individually specified drug, device, or biological product where both are required to achieve the intended use, indication, or effect and where upon approval of the proposed product the labeling of the approved product would need to be changed, e.g., to reflect a change in intended use, dosage form, strength, route of administration, or significant change in dose; or (iv) any investigational drug, device, or biological product packaged separately that according to its proposed labeling is for use only with another individually specified investigational drug, device, or biological product where both are required to achieve the intended use, indication, or effect.

The FDA is divided into various branches, or Centers, by product type. Different Centers typically review drug, biologic, or device applications. In order to review an application for a combination product, the FDA must decide which Center should be responsible for the review. FDA regulations require that the FDA determine the combination product's primary mode of action, which is the single mode of a combination product that provides the most important therapeutic action of the combination product. The Center that regulates that portion of the product becomes the lead evaluator. When evaluating an application, a lead Center may consult other Centers but still retain complete reviewing authority, or it may collaborate with another Center, by which the Center assigns review of a specific section of the application to another Center, delegating its review authority for that section. Typically, the FDA requires a single marketing application submitted to the Center selected to be the lead evaluator, although the agency has the discretion to require separate applications to more than one Center. One reason to submit multiple applications is if the applicant wishes to receive some benefit that accrues only from approval under a particular type of application, like new drug product exclusivity. If multiple applications are submitted, each may be evaluated by a different lead Center.

## **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. Moreover, there is an increasing trend in the EU requiring public disclosure of development data, in particular clinical trial data. These data were traditionally regarded as confidential commercial information; however, under policies recently 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.

## **The Hatch-Waxman Act**

Upon approval of a drug through an NDA, applicants are required to submit to the FDA each patent that covers the applicant's product or FDA approved method of using this product. Those patents are then published in the FDA's Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential competitors in support of approval of an ANDA. Generally, an ANDA provides for marketing of a drug product that has the same active ingredients in the same strength(s), route of administration, and dosage form as the listed drug and has been shown through bioequivalence testing to be therapeutically equivalent to the listed drug. ANDA applicants are not required to conduct or submit results of preclinical or clinical tests to prove the safety or effectiveness of their drug product, other than the requirement for bioequivalence testing. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug, and can often be substituted by pharmacists under prescriptions written for the original listed drug.

The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. A certification that the new product will not infringe the already approved product's listed patents or that such patents are invalid is called a paragraph IV certification. If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired. Alternatively, for a patent covering an approved method of use, an ANDA applicant may submit a statement to the FDA that the company is not seeking approval for the covered use.

If the ANDA applicant has submitted a paragraph IV certification to the FDA, the applicant must also send notice of the paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the paragraph IV certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the ANDA applicant.

The ANDA application also will not be approved until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired. Federal law provides a period of five years following approval of a drug containing no previously approved active moiety, during which ANDAs for generic versions of those drugs cannot be submitted unless the submission contains a paragraph IV challenge to a listed patent, in which case the submission may be made four years following the original product approval. Federal law provides for a period of three years of exclusivity following approval of a listed drug that contains previously approved active ingredients but is approved in a new dosage form, route of administration or combination, or for a new condition of use, the approval of which was required to be supported by new clinical trials conducted by or for the sponsor, during which the FDA cannot grant effective approval of an ANDA based on that listed drug. Both of the five-year and three-year exclusivity periods, as well as any unexpired patents listed in the Orange Book for the listed drug, can be extended by six months if the FDA grants the NDA sponsor a period of pediatric exclusivity based on studies submitted by the sponsor in response to a written request.

### ***Exclusivity for Biologics***

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” or “interchangeable” with an FDA-licensed reference biological product. Biosimilarity sufficient to reference a prior FDA-licensed product requires that there be no differences in conditions of use, route of administration, dosage form, and strength, and no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency. Biosimilarity must be shown through analytical studies, animal studies, and at least one clinical study, absent a waiver from the Secretary of the U.S. Department of Health and Human Services. In order to meet the higher hurdle of interchangeability, a sponsor must demonstrate that the biosimilar product can be expected to produce the same clinical result as the reference product, and for a product that is administered more than once, that the risk of 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, though no interchangeable products have been approved to date. 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 exclusivity from the time of first licensure of the reference product and no application for a biosimilar can be submitted for four years from the date of 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 has exclusivity against a finding of interchangeability for other biologics for the same condition of use for the lesser of (i) one year after first commercial marketing of the first interchangeable biosimilar, (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 a patent lawsuit is ongoing within the 42-month period.

### ***Orphan Drug Designation***

Orphan drug designation is granted by the FDA and EMA 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. 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 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.

Orphan drug designation does not shorten the regulatory review and approval process. However, if an orphan drug later receives approval for the indication for which it has designation, the relevant regulatory authority may not approve any other applications to market the same drug for the same indication, except in very limited circumstances, for seven years in the U.S. and ten years in the EU (extendable to twelve years for medicines that have complied with an agreed pediatric investigation plan pursuant to Regulation 1901/2006) and, in addition, a range of other benefits during the development and regulatory review process are available in the EU, including scientific assistance for study protocols, authorization through the centralized marketing authorization procedure covering all member countries and a reduction or elimination of registration and marketing authorization fees. Among the benefits of orphan drug designation in the U.S. are tax credits for certain research and a waiver of the NDA/BLA application user fee. Orphan drug exclusive marketing rights may be lost under certain conditions, such as if the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug. In the EU, marketing authorization may be granted to a similar medicinal product with the same orphan indication during the regulatory exclusivity period with the consent of the marketing authorization holder for the original orphan medicinal product or if the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities. Marketing authorization may also be granted to a similar medicinal product with the same orphan indication if this medicinal product is safer, more effective or otherwise clinically superior to

the original orphan medicinal product. The period of market exclusivity may, in addition, be reduced to six years if, at the end of the fifth year, it can be demonstrated on the basis of available evidence that the criteria for its designation as an orphan medicine are no longer satisfied, for example if the original orphan medicinal product has become sufficiently profitable not to justify maintenance of market exclusivity.

### ***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.

### ***PRIME Designation***

The EMA launched its PRIME regulatory initiative 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.

### ***Pediatric Information***

Under the Pediatric Research Equity Act of 2007 (PREA), 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 Best Pharmaceuticals for Children Act (BPCA) provides sponsors of NDAs with an additional six-month period of market exclusivity for all unexpired patent or non-patent exclusivity on all forms of the drug containing the active moiety if the sponsor submits results of pediatric studies specifically requested by the FDA under BPCA within required timeframes. The BPCIA provides sponsors of BLAs an additional six-month extension for all unexpired non-patent market exclusivity on all forms of the biological containing the active moiety pursuant to the BPCA if the conditions under the BPCA are met.

In the EU, companies developing a new medicinal product must agree to a Paediatric Investigation Plan (PIP) with the EMA and must conduct pediatric clinical trials in accordance with that PIP, unless a deferral or waiver applies, (e.g., because the relevant disease or condition occurs only in adults). 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. Products that are granted a marketing authorization on the basis of the pediatric clinical trials conducted in accordance with the PIP 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 subject to specific conditions and is not automatically available when data in compliance with the PIP are developed and submitted.

### ***Fast Track Designation and Accelerated Approval***

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

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

Under the fast track program and the FDA's accelerated approval regulations, the FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate

endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions, or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of a Phase 4 or post-approval clinical trial to confirm the effect on the clinical endpoint. Failure to conduct a required post-approval study or confirm a clinical benefit through a post-marketing study will allow the FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by the FDA.

### **Post-Approval Regulatory Requirements**

Following approval, the FDA and the EMA will impose certain post-approval requirements related to a product. For instance, the FDA closely regulates the post-approval marketing and promotion of approved products, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the Internet. Moreover, if a company obtains original FDA approval for a product via the accelerated approval pathway, the company 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 could result in the withdrawal of the FDA's marketing approval for a product.

Approved 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 EMA, as applicable, before the change can be implemented. An NDA/BLA or MAA supplement for a new indication typically requires clinical data similar to that in the original application, and similar procedures and actions in reviewing NDA/BLA or MAA supplements as in reviewing NDAs/BLAs and MAAs.

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

### **Healthcare Reform**

The U.S. and some non-U.S. jurisdictions are considering or have enacted a number of legislative and regulatory proposals to change the healthcare system in ways that could affect our ability to sell our products profitably. For example, in the U.S., the PPACA is a sweeping measure intended to improve quality of care, constrain healthcare spending, and expand healthcare coverage within the U.S., primarily through the imposition of health insurance mandates on employers and individuals and expansion of the Medicaid program.

The PPACA also imposed a fee on certain manufacturers and importers of branded prescription drugs (excluding orphan drugs under certain conditions). The annual fee is apportioned among the participating companies based on each company's sales of qualifying products to, or use by, certain U.S. government programs during the preceding year. Other provisions of the law have also affected us and have increased certain of our costs. For example, the Medicaid rebate rate was increased and the volume of rebated drugs has been expanded to include beneficiaries in Medicaid managed care organizations. Among other things, the PPACA also expanded the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance, and now includes a 70% discount on brand name drugs for Medicare Part D participants in the coverage gap, or "donut hole." The law also revised the definition of "average manufacturer price" for reporting purposes. In addition, drug manufacturers are required to collect and report annually information on payments or transfers of value to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as investment interests held by physicians and their immediate family members during the preceding calendar year. Effective January 1, 2022, covered manufacturers will also be required to report on payments or transfers of value to physician assistants, nurse practitioners or clinical nurse specialists, anesthesiologist assistants, certified registered nurse anesthetists, and certified nurse-midwives. The



reported data are posted in searchable form on a public web site. Failure to submit required information may result in civil monetary penalties. It is still unclear the full impact that the PPACA will have on our business.

There have been executive, judicial and congressional challenges to certain aspects of the PPACA. For example, former U.S. President Trump signed several Executive Orders designed to delay the implementation of any certain provisions of the PPACA or otherwise circumvent some of the requirements for health insurance mandated by the PPACA. Concurrently, Congress considered legislation that would repeal or repeal and replace all or part of the PPACA. While Congress has not passed legislation repealing the PPACA in its entirety, it has enacted laws that modify certain provisions of the PPACA such as removing penalties for not complying with the PPACA's individual mandate to carry health insurance, delaying the implementation of certain ACA-mandated fees, and increasing the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D. Additionally, on December 14, 2018, a Texas U.S. District Court Judge ruled that the PPACA is unconstitutional in its entirety because the individual mandate was repealed by Congress as part of the Tax Cuts and Jobs Act. This case was ultimately appealed to the United States Supreme Court, and on November 10, 2020, the U.S. Supreme Court heard oral arguments in this case. It is unclear when a decision will be made. Although the U.S. Supreme Court has not yet ruled on the constitutionality of the PPACA, on January 28, 2021, President Biden issued an executive order to initiate a special enrollment period from February 15, 2021 through May 15, 2021 for purposes of obtaining health insurance coverage through the PPACA marketplace. The executive order also instructs certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including, among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the PPACA. It is unclear how the Supreme Court ruling, other such litigation, and the healthcare reform measures of the Biden administration will impact the PPACA and our business.

Other legislative changes have been proposed and adopted since the PPACA was enacted. These changes included the Budget Control Act of 2011, which caused aggregate reductions to Medicare payments to providers of up to 2% per fiscal year effective April 1, 2013 which, following passage of the Bipartisan Budget Act of 2015, will stay in effect through 2030 unless additional congressional action is taken. However, the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, and other COVID-19 relief legislation suspended the 2% Medicare sequester from May 1, 2020 through March 31, 2021. The Consolidated Appropriations Act, 2021 extended the suspension of the 2% Medicare sequester through March 31, 2021. Further, the American Taxpayer Relief Act of 2012, among other things, further reduced Medicare payments to several types of providers.

Additionally, there has been increasing legislative and enforcement interest in the U.S. with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. congressional inquiries and proposed and enacted state and federal 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. For example, at the federal level, the former Trump administration used several means to propose or implement drug pricing reform, including through federal budget proposals, executive orders and policy initiatives. It is unclear whether the Biden administration will work to reverse these measures or pursue similar policy initiatives. However, it is expected that the Biden administration will also focus on controlling drug costs. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, to encourage importation from other countries and bulk purchasing.

Moreover, former U.S. President Trump and President Biden both issued Executive Orders intended to favor government procurement from domestic manufacturers. Former President Trump issued an Executive Order specifically aimed at the procurement of pharmaceutical products, which instructed the federal government to develop a list of "essential" medicines and then buy those and other medical supplies that are manufactured, including the manufacture of the API, in the U.S. It is unclear whether this Executive Order or something similar will be implemented by the Biden Administration.

### ***Brexit and the Regulatory Framework in the U.K.***

Following the result of a referendum in 2016, the U.K. left the EU on January 31, 2020, commonly referred to as Brexit. Pursuant to the formal withdrawal arrangements agreed between the U.K. and the EU, the U.K. was subject to a transition period until December 31, 2020, or the Transition Period, during which EU rules continued to apply. The Trade and Cooperation Agreement which outlines the future trading relationship between the U.K. and the EU was agreed to in December 2020. While the Trade and Cooperation Agreement provides for the tariff-free trade of medicinal products between the U.K. and the EU there may be additional non-tariff costs to such trade which did not exist prior to the end of the Transition Period. Further, should the U.K. diverge from the EU from a regulatory perspective in relation to medicinal products, tariffs could be put into place in the future.

Since a significant proportion of the regulatory framework in the U.K. applicable to our business and our product candidates is derived from EU directives and regulations, Brexit has had, and will continue to have, a material impact upon the regulatory regime with respect to the development, manufacture, importation, approval and commercialization of our products and product candidates in the U.K. or the EU. Great Britain is no longer covered by the EU's procedures for obtaining the grant of marketing authorizations (Northern Ireland will be covered by the centralized authorization procedure and can be covered under the decentralized or mutual recognition procedures. A separate marketing authorization will be required to market drugs in Great

Britain. However, for two years from January 1, 2021, the U.K.'s regulator, the MHRA, may adopt decisions taken by the EC on the approval of new marketing authorizations through the centralized procedure, and the MHRA will have regard to marketing authorizations approved in a country in the EEA (although in both cases a marketing authorization will only be granted if any Great Britain-specific requirements are met). Various national procedures are now available to place a drug on the market in the U.K., Great Britain, or Northern Ireland, with the main national procedure having a maximum timeframe of 150 days (excluding time taken to provide any further information or data required). The data exclusivity periods in the U.K. are currently in line with those in the EU, but the Trade and Cooperation Agreement provides that the periods for both data and market exclusivity are to be determined by domestic law, and so there could be divergence in the future. It is currently unclear whether the MHRA in the U.K. is sufficiently prepared to handle the increased volume of marketing authorization applications that it is likely to receive. Any delay in obtaining, or an inability to obtain, any marketing approvals, would delay or prevent us from commercializing our product candidates in the U.K. or the EU and restrict our ability to generate revenue and achieve and sustain profitability.

Orphan designation in Great Britain following Brexit is essentially identical to the position in the EU, but is based on the prevalence of the condition in Great Britain. It is therefore possible that conditions that are currently designated as orphan conditions in Great Britain will no longer be and that conditions that are not currently designated as orphan conditions in the EU will be designated as such in Great Britain.

### **Other U.S. Regulatory Requirements**

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

The federal Anti-Kickback Statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. The PPACA amended the intent requirement of the federal Anti-Kickback and certain other criminal healthcare fraud statutes such that a person or entity no longer needs to have actual knowledge of these statutes or specific intent to violate them in order to commit a violation. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other. Although there are a number of statutory exceptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exceptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exception or safe harbor.

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

The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for, among other things, executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters.

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and their implementing regulations, also imposes obligations, including mandatory contractual terms, on certain types of individuals and entities, with respect to safeguarding the privacy, security and transmission of individually identifiable health information.

The federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the CMS information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) 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. Effective January 1, 2022, covered manufacturers will also be required to report on payments or transfers of value to physician assistants, nurse practitioners or clinical nurse specialists, anesthesiologist assistants, certified registered nurse anesthetists, and certified nurse-midwives during the previous year.

The majority of states also have statutes or regulations similar to the federal Anti-Kickback Statute and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer. Several states now require pharmaceutical companies to report expenses relating to the marketing and promotion of pharmaceutical products and to report gifts and payments to individual physicians in these states while other states prohibit various

other marketing-related activities. Other states require submission or disclosure of certain pricing information. Still other states require the posting of information relating to clinical studies and their outcomes. In addition, states including California, Connecticut, Nevada and Massachusetts require pharmaceutical companies to implement compliance programs or marketing codes. Currently, several 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.

### ***Approval Outside of the U.S./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 EMA 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.

### ***Anti-Corruption Legislation***

The U.S. Foreign Corrupt Practices Act (FCPA), to which we are subject, prohibits corporations and individuals from engaging in certain activities to obtain or retain business or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any non-U.S. government official, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity. Similar laws exist in other countries, such as the U.K., that restrict improper payments to public and private parties. Many countries have laws prohibiting these types of payments within the respective country. Historically, pharmaceutical companies have been the target of FCPA and other anti-corruption investigations and penalties.

### ***Pricing and Reimbursement***

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

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

Outside of the U.S. our products are paid for by a variety of payers, with governments being the primary source of payment. Reimbursement in the EU and many other territories must be negotiated on a country-by-country basis and in many countries the product cannot be commercially launched until 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 patient access to and 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 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 ANDAs, the rebate amount is 13% of the average manufacturer price (AMP) for the quarter. The AMP is the weighted average of prices paid to the manufacturer (1) directly by retail community pharmacies and (2) by wholesalers for drugs distributed to retail community pharmacies. For innovator products (i.e., drugs that are marketed under NDAs or BLAs), the rebate amount is the greater of 23.1% of the AMP for the quarter or the difference between such AMP and the best price for that same quarter. The best price is essentially the lowest price available to non-governmental entities. Innovator products may also be subject to an additional rebate that is based on the amount, if any, by which the product's AMP for a given quarter exceeds the inflation-adjusted baseline AMP, which for most drugs is the AMP for the first full quarter after launch. Since 2017, non-innovator products are also subject to an additional rebate.

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

A manufacturer must also participate in a federal program known as the 340B drug pricing program in order for federal funds to be available to pay for the manufacturer's drugs 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.

Federal law also requires that manufacturers report data on a quarterly basis to CMS regarding the pricing of drugs that are separately reimbursable under Medicare Part B. These are generally drugs, such as injectable products, that are administered "incident to" a physician service and are not generally self-administered. The pricing information submitted by manufacturers is the basis for reimbursement to physicians and suppliers for drugs covered under Medicare Part B. 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. The U.S. Department of Health and Human Services, (HHS), recently published an interim final rule that establishes a Most Favored Nation (MFN), Model for Medicare Part B drug payment. This regulation would substantially change the drug reimbursement landscape as it bases Medicare Part B payment for 50 selected drugs on prices in non-U.S. countries instead of average sales price (ASP), and establishes a fixed add-on payment in place of the current 6 percent (4.3 percent after sequestration) of ASP. The MFN drug payment amount is expected to be lower than the current ASP-based payment limit because U.S. drug prices are generally the highest in the world. Although the MFN Model interim final rule with comment period was effective as of November 27, 2020, and the model payment methodology was scheduled to begin on January 1, 2021, it faces uncertain prospects for implementation. To date, three federal courts have granted orders preventing implementation of the rule. In addition to these lawsuits, the Biden administration, which has implemented a regulatory freeze, or additional litigation challenging the regulation could delay or halt the implementation of the rule.

Medicare Part D provides prescription drug benefits for seniors and people with disabilities. Medicare Part D beneficiaries have a gap in their coverage (between the initial coverage limit and the point at which catastrophic coverage begins) where Medicare does not cover their prescription drug costs, known as the coverage gap. However, starting in 2020, Medicare Part D beneficiaries pay 25% of drug costs after they reach the initial coverage limit - the same percentage they were responsible for before they reached that limit - thereby closing the coverage gap. The cost of closing the coverage gap is being borne by innovator companies and the government through subsidies. Each manufacturer of drugs approved under NDAs or BLAs is required to enter into a Medicare Part D coverage gap discount agreement and provide a 70% discount on those drugs dispensed to Medicare beneficiaries in the coverage gap, in order for its drugs to be reimbursed by Medicare Part D.

#### *Federal Contracting/Pricing Requirements*

Manufacturers are also required to make their covered drugs, which are generally drugs approved under NDAs or BLAs, available to authorized users of the Federal Supply Schedule (FSS) of the General Services Administration. The law also requires manufacturers to offer deeply discounted FSS contract pricing for purchases of their covered drugs by the Department of Veterans Affairs, the Department of Defense, the Coast Guard, and the Public Health Service (including the Indian Health Service) in order for federal funding to be available for reimbursement or purchase of the manufacturer's drugs under certain federal programs. FSS

pricing to those four federal agencies for covered drugs must be no more than the Federal Ceiling Price (FCP), which is at least 24% below the Non-Federal Average Manufacturer Price (Non-FAMP) for the prior year. The Non-FAMP is the average price for covered drugs sold to wholesalers or other middlemen, net of any price reductions.

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

## **Human Capital**

As of December 31, 2020, we had 3,059 full-time employees worldwide, 1,343 of whom were in operations, 664 of whom were in research and development, 550 of whom were in sales and marketing and 502 of whom were in administration. Of the 3,059 full-time employees as of December 31, 2020, 2,162 employees were in the U.S. and Canada, and 897 employees were in other non-U.S. countries, including 718 in Europe and the Middle East, 117 in Latin America and 62 in Asia Pacific. We also leverage temporary workers to fill short-term positions for our business and manufacturing needs. As a result of the COVID-19 pandemic, a majority of our global workforce is currently working remotely. However, a portion of our employee base in the U.S. and Ireland continues to work onsite supporting manufacturing and laboratory operations.

### *Diversity, Equity and Inclusion*

At BioMarin, prejudice, racism and intolerance are unacceptable. We are committed to diversity, equity and inclusion (DEI) across all aspects of our organization, including hiring, promotion and development practices. As of December 31, 2020, racial and ethnic minorities represented 46% of our employees in the U.S. Globally, 50% of our workforce were women and 46% of our positions at director-level and above were held by women. We are committed to continuing our ongoing efforts to ensure diversity in all positions, including leadership.

We remain steadfast in our commitment to fostering a community that reflects equality and inclusiveness and working towards making BioMarin a place where every employee feels heard, respected and valued. This commitment is a cultural value, and we believe incorporating and encouraging different perspectives from employees of varied backgrounds and experiences helps us better achieve our business goals and objectives. In 2020, we formed a DEI Employee Advisory Committee to help us define our DEI roadmap and ensure that perspectives from employees of different age, tenure, gender, sexual orientation, race, ethnicity, level and location are considered in how we build the most inclusive environment. We also continue to support and increase the number of our employee resource groups that build community for employees from underrepresented groups.

We are honored to be recognized as a company of choice. In January 2021, we were recognized as a Best Place to Work for lesbian, gay, bisexual, transgender and queer (LGBTQ) Equality by the Human Rights Campaign, scoring 100% on their Corporate Equality Index, one of the foremost benchmarking surveys and reports in the U.S. measuring corporate policies and practices related to LGBTQ workplace equality.

### *Compensation, Benefits and Well-being*

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

In 2020, in response to the COVID-19 pandemic, we developed a comprehensive global plan that established clear and standardized COVID-19 policies, safety protocols and updates to all employees. We implemented significant changes that we determined were in the best interest of our employees as well as the communities in which we operate. These include having the majority of our employees work from home, while implementing additional safety measures for employees continuing critical onsite work. We also instituted a policy prohibiting all domestic and international non-essential travel for all employees. As part of our response to the changes implemented due to the COVID-19 pandemic, we established additional employee support programs to support the transition to remote working and to provide financial support and time-off for working parents. In addition, policies and programs were established to help support our onsite manufacturing and laboratory staff, including safety screening, onsite COVID-19 testing and financial support for employees who incurred additional expenses related to higher commuting costs, fitness facility closures, personal purchase of supplies and equipment, and increased childcare demands.

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

#### *Professional Growth and Development*

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

#### *Patient and Community Connections*

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

#### **Other Information**

We were incorporated in Delaware in October 1996. Our principal executive offices are located at 770 Lindero 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](http://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](http://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

### **The COVID-19 pandemic could materially adversely affect our business, results of operations, and financial condition.**

On March 11, 2020, the World Health Organization declared the COVID-19 outbreak a pandemic. The COVID-19 pandemic has resulted in travel restrictions, quarantines, “work-from-home” and “shelter-in-place” orders and extended shutdown of certain businesses around the world, including in many countries in which we operate. We experienced an impact on our product revenues and development programs in 2020 from the COVID-19 pandemic, and we expect that the outbreak will continue to adversely impact our financial results and our business generally. Ongoing and future effects of the COVID-19 pandemic (or any future pandemic) on all aspects of our business and operations, including revenues, expenses, reserves and allowances, manufacturing, clinical trials and research and development costs, and the duration of such effects, are highly uncertain and difficult to predict. We anticipate that the COVID-19 pandemic will likely continue to have an adverse impact on our business, results of operations, and financial condition.

The continued spread of COVID-19 has adversely affected and will likely continue to adversely impact our product development programs, including preclinical study and clinical trial operations. We have been, and will likely continue to be, unable to initiate or continue conducting clinical trials as originally planned due to the prioritization of hospital resources toward the outbreak, difficulty in recruiting and retaining healthcare providers and staff due to their diversion toward treating COVID-19 patients or their heightened exposure to COVID-19, potential unwillingness of patients to enroll or continue in trials for fear of exposure to COVID-19 at sites, or the inability of patients to comply with clinical trial protocols as quarantines or travel restrictions impede patient movement or otherwise interrupt healthcare services. For example, we experienced delays in certain clinical trials and have had to reevaluate expected timelines for those trials. In addition, we rely on independent clinical investigators, contract research organizations (CROs) and other third-party service providers to assist us in managing, monitoring and otherwise carrying out our preclinical studies and clinical trials, and the outbreak may affect their ability to devote sufficient time and resources to our programs or to travel to sites to perform work for us. Additionally, the COVID-19 pandemic has delayed, and may continue to postpone, necessary regulatory inspections and other interactions with regulators regarding our products in development, which could delay review or approval of our regulatory submissions.

COVID-19 could adversely affect our ability to source materials and supplies and successfully manufacture and distribute our product candidates and products. The outbreak could result in reduced operations of third-party suppliers of raw materials and supplies upon whom we rely or otherwise limit our ability to obtain sufficient materials and supplies necessary for production of our therapies. Our manufacturing facilities and those of our contract manufacturers are located in areas impacted by the COVID-19 pandemic, which may result in delays or disruptions in our ability to produce product candidates and products. If we or any third party in our supply or distribution chain are adversely impacted by the COVID-19 pandemic, including as a result of required closures, staffing shortages, production slowdowns and disruptions in delivery systems, our operations may be disrupted, limiting our ability to manufacture and distribute our product candidates for clinical trials and research and development operations and our products for commercial sales.

Our commercial operations have also been, and will likely continue to be, adversely impacted by the COVID-19 pandemic. Many of our products are administered via infusions in a clinic or hospital setting and/or by a healthcare professional. Treating COVID-19 patients has become the priority for many healthcare facilities and workers, so it has become, and may continue to be, difficult for some of our patients to receive our therapies that are administered by infusion. Although we are working with our patient community and healthcare providers to find alternative arrangements where necessary, such as providing infusions at home, the revenue from doses of our products that are missed by patients and the lost revenue from delayed treatment starts for new patients will never be recouped. Moreover, some patients may choose to skip infusions because they do not want to risk exposure to COVID-19 by having a healthcare provider administer the therapy at a healthcare facility or at home. The pandemic has also hindered our ability to find new patients and start treating newly found patients, and it has limited our sales force’s ability to promote our products to distributors, hospitals, clinics, doctors and pharmacies, which could adversely affect our revenues and results of operations. In addition, the COVID-19 pandemic could adversely affect our workforce and the employees of companies with which we do business, thereby disrupting our business operations. We have implemented work-from-home policies for employees whose jobs do not require them to be onsite. Increased reliance by us and the companies with which we do business on personnel working from home may negatively impact productivity, increase cyber security risk, create data accessibility issues, increase the risk for communication disruptions, or otherwise disrupt or delay normal business operations. For our employees whose jobs require them to be onsite, we have taken precautions to avoid the spread of COVID-19 among our employees, but we cannot guarantee our workforce will not face an outbreak that could adversely impact our operations.

While the long-term economic impact and the duration of the COVID-19 pandemic may be difficult to predict, the widespread pandemic has resulted in, and may continue to 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 convertible notes. In addition, a recession, further market correction or depression resulting from the COVID-19 pandemic could materially adversely affect our business and the value of our common stock and convertible notes.

To the extent the COVID-19 pandemic adversely affects our business and financial results, it 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 substantial indebtedness, our need to generate sufficient cash flows to service our indebtedness and finance our operations, our ability to comply with the covenants contained in the agreements that govern our indebtedness and the volatility of our stock price.

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

All of our products target diseases with 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 profitability. For Brineura, Naglazyme and Vimizim in particular, we must market worldwide to achieve significant market penetration of the product. In addition, because the number of potential patients in each disease population is small, it is not only important to find patients who begin therapy to achieve significant market penetration of the product, but we also need to be able to maintain these patients on therapy for an extended period of time. Due to the expected costs of treatment for our products, we may be unable to maintain or obtain sufficient market share at a price high enough to justify our product development efforts and manufacturing expenses.

**If we fail to 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 patients to need treatment for extended periods, and for some products throughout the lifetimes of the patients. We expect that most families of patients will not be capable of paying for this treatment themselves. There will be no commercially viable market for our products without coverage and reimbursement from third-party payers. Additionally, even if there is a commercially viable market, if the level of reimbursement is below our expectations, our revenue and gross margins 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. Coverage and reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. If coverage and reimbursement are not available or reimbursement is available only to limited levels, we may not successfully commercialize any product candidate for which we obtain marketing approval or continue to market any product that has already been commercialized.

Reimbursement in the European Union (EU) and many other territories must be negotiated on a country-by-country basis and in many countries the product cannot be commercially launched until 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 margins may be adversely affected.

**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 revenue 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) or commercialize their products before we do. With respect to valoctocogene roxaparvovec, if the product candidate is approved, we will face a highly developed and competitive market for hemophilia A treatments. As we commercialize valoctocogene roxaparvovec, if approved, we may face intense competition from large pharmaceutical companies with extensive resources and established relationships in the hemophilia A community. If we do not compete successfully, our revenue would be adversely affected, and we may be unable to generate sufficient sales to recover our expenses related to the development of a product program or to justify continued marketing of a product.

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

Even if our product candidates are approved, if doctors elect a course of treatment which does not include our products, this decision would reduce demand for our products and adversely affect revenues. For example, if gene therapy becomes widely used as a treatment of genetic diseases, the use of enzyme replacement therapy, such as Aldurazyme, Naglazyme, and Vimizim in MPS diseases, could be greatly reduced. Moreover, if we obtain regulatory approval for valoctocogene roxaparvovec, the commercial success of valoctocogene roxaparvovec will still depend, in part, on the acceptance of physicians, patients and healthcare payers of gene therapy products in general, and our product candidate in particular, as medically necessary, cost effective and safe. Changes in treatment method can be caused by the introduction of other companies' products or the development of new technologies or surgical procedures which may not directly compete with ours, but which have the effect of changing how doctors decide to treat a disease.

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

Our future growth and development depend in part on our ability to successfully develop new products from our research and development activities. The development of biopharmaceutical products is very expensive and time intensive and involves a great degree of risk. The outcomes of research and development programs, especially for innovative biopharmaceuticals, 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 may target diseases that we are also targeting, and have already entered into partnering and licensing arrangements with academic research institutions, reducing the pool of available opportunities.

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

**The sale of generic versions of Kuvan by generic manufacturers has adversely affected and may continue to adversely affect our revenue and results of operations.**

The Drug Price Competition and Patent Term Restoration Act of 1984, known as the Hatch-Waxman Act, permits the Food and Drug Administration (FDA) to approve abbreviated new drug applications (ANDAs) for generic versions of branded drugs. We refer to this process as the ANDA process. The ANDA process permits competitor companies to obtain marketing approval for a drug with the same active ingredient as a branded drug, but does not generally require the conduct and submission of clinical efficacy studies for the generic product. In place of such clinical studies, an ANDA applicant usually needs only to submit data demonstrating that its product is bioequivalent to the branded product.

Pursuant to the Hatch-Waxman Act, companies were permitted to file ANDA applications for proposed generic versions of Kuvan at any time after December 2011. We own several patents that cover Kuvan, and we have listed those patents in conjunction with that product in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book).



The Hatch-Waxman Act requires an ANDA applicant seeking FDA approval of its proposed generic product prior to the expiration of our Orange Book-listed patents to certify that the applicant believes that our patents are invalid or will not be infringed by the manufacture, use or sale of the drug for which the application has been submitted (a paragraph IV certification) and notify us of such certification (a paragraph IV notice). Upon receipt of a paragraph IV notice, the Hatch-Waxman Act allows us, with proper basis, to bring an action for patent infringement against the ANDA filer, asking that the proposed generic product not be approved until after our patents expire. If we commence a lawsuit within 45 days from receipt of the paragraph IV notice, the Hatch-Waxman Act provides a 30-month stay, during which time the FDA cannot finally approve the generic's application. If the litigation is resolved in favor of the ANDA applicant during the 30-month stay period, the stay is lifted and the FDA may approve the ANDA if it is otherwise ready for approval. The discovery, trial and appeals process in such a lawsuit is costly, time consuming, and may result in generic competition if the ANDA applicant prevails.

Between 2014 and 2016 we received paragraph IV notice letters from two pharmaceutical companies notifying us that each had filed ANDAs seeking approval of proposed generic versions of Kuvan. We filed lawsuits alleging patent infringement against each company, and between 2015 and 2017 we entered into settlement agreements with the companies that resolved the patent litigation in the U.S. The settlement agreements granted the companies non-exclusive licenses to our Kuvan-related patents to allow them to market generic versions of sapropterin dihydrochloride in the U.S. for the indications approved for Kuvan beginning on October 1, 2020, or earlier under certain circumstances. Generic versions of Kuvan first became available in the U.S. in October 2020, and as expected, our U.S. sales of Kuvan in the fourth quarter of 2020 significantly declined as compared to prior periods. We expect that the continued availability of generic versions of sapropterin dihydrochloride in the U.S. will continue to adversely affect our U.S. sales of Kuvan.

Any future ANDA or related legal proceeding could have an adverse impact on our stock price, and litigation to enforce our patents has, and is likely to continue to, cost a substantial amount and require significant management attention. If the patents covering Kuvan and its use are not upheld in litigation, or if any ANDA filer we bring suit against is found to not infringe our asserted patents, the resulting generic competition following the expiration of regulatory exclusivity would have a material adverse effect on our revenue and results of operations. Moreover, generic competition following the settlements described above could have a material adverse effect on our revenue and results of operations.

We also face generic competition for Kuvan in certain non-U.S. countries, and there is a process equivalent to the ANDA process under Article 10 of Directive 2001/83/EC in the EU. Our ability to successfully market and sell Kuvan in many countries in which we operate is based upon patent rights or certain regulatory forms of exclusivity, or both. The scope of our patent rights and regulatory exclusivity for Kuvan vary from country to country and are dependent on the availability of meaningful legal remedies in each country. If our patent rights and regulatory exclusivity for Kuvan are successfully challenged, expire, or otherwise terminate in a particular country, the resulting generic competition could have a material adverse effect on our revenue and results of operations.

**If we do not achieve our projected development goals in the timeframes we announce and expect, the commercialization of our product candidates may be delayed 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, in many cases for reasons beyond our control. If we do not meet these milestones as publicly announced, the commercialization of our products may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

### Regulatory Risks

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

We must obtain regulatory approval to market and sell our product candidates. For example, in the U.S., we must obtain FDA approval for each product candidate that we intend to commercialize, and in Europe we must obtain approval from the European Medicines Agency (EMA). The FDA and EMA approval processes are typically lengthy and expensive, and approval is never certain. 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 agency will mean that other agencies will also approve the same product candidate. Similarly, 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 and 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 EMA approval. Moreover, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Approval by the FDA or EMA 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 EMA. 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 EMA 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.

Because of the risks and uncertainties in pharmaceutical development, our product candidates could take a significantly longer time to gain regulatory approval than we expect or may never gain approval. We also rely on independent third-party CROs to file some of our non-U.S. marketing applications and 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 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. In addition, the FDA, the EMA and other international regulatory authorities have substantial discretion over the approval process for pharmaceutical products. These regulatory agencies may not agree that we have demonstrated the requisite level of product safety and efficacy to grant approval and may require 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.

With respect to valoctocogene roxaparvovec, we may experience challenges specific to gene therapy that cause significant delays or unanticipated costs, or that cannot be solved. Although numerous companies are currently advancing gene therapy product candidates through clinical trials, the FDA has only approved a very small number of vector-based gene therapy products thus far. Moreover, there are very few approved gene therapy products outside the U.S. As a result, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for valoctocogene roxaparvovec in any jurisdiction. Regulatory requirements governing gene and cell therapy products are still evolving and may continue to change in the future. For example, in October 2020, it was reported that the Director of the Center for Biologics Evaluation and Research, the center of the FDA responsible for reviewing marketing applications for gene therapies, stated that the FDA will assess the importance of durability of effect differently for a gene therapy that treats a disease that has no other available therapies versus a condition for which there are multiple approved treatments. Regulatory review agencies 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 treatment candidate or lead to significant post-approval studies, limitations or restrictions. For example, on August 18, 2020 the FDA issued a Complete Response Letter (CRL) to our BLA for valoctocogene roxaparvovec for the treatment of adults with severe hemophilia A. In the CRL, the FDA introduced a new request for two-year follow-up safety and efficacy data on all study participants from our ongoing Phase 3 study of valoctocogene roxaparvovec. The trial will not complete two years of follow-up until November 2021. Continued delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring valoctocogene roxaparvovec to market could have a negative effect on our business and financial condition. Even if we do obtain regulatory approval, ethical, social and legal concerns about gene therapy arising in the future could result in additional regulations restricting or prohibiting sale of our product.

In addition, some of our product candidates are intended to be used in combination with a delivery device, such as an injector or other delivery system. Medical products containing a combination of new drugs, biological products or medical devices may be regulated as "combination products" in the U.S. A combination product generally is defined as a product consisting of components from two or more regulatory categories (e.g., drug/device, device/biologic, drug/biologic). 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. Our product candidates intended for use with such devices, 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 or clearances. Where approval of the drug or biologic product and device is sought under a single application, the increased complexity of the review process may delay approval. The FDA review process and criteria are not well-established areas, which could also lead to delays in the approval process. In addition, because these delivery 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 and 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 agencies 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. For example, when the FDA accepted our new drug application for vosoritide in late 2020, the agency reiterated its position raised during the vosoritide Pediatric Advisory Committee and Endocrinologic and Metabolic Drugs Advisory Committee held in May 2018, recommending two, two-year placebo-controlled trials in different age groups. Although our vosoritide clinical program does not meet every element of the FDA's recommendation, we designed our studies of vosoritide in a manner that we believe can demonstrate efficacy and safety of the product candidate for the target patient population. However, the FDA may ultimately disagree with our position that our vosoritide trials support regulatory approval. Moreover, sometimes different regulatory agencies 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 and other non-U.S. 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 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 revenue 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.**

Aldurazyme, Brineura, Kuvan, Naglazyme and Vimizim have received regulatory approval to be commercially marketed and sold in the U.S., the EU and certain other countries, and Palynziq has received regulatory approval to be commercially marketed in the U.S. and the EU. Any product for which we have obtained regulatory approval, or for which we obtain regulatory approval in the future, along with the manufacturing processes and practices, post-approval clinical research, product labeling, advertising and promotional activities for such product, are subject to continual requirements of, and review by, the FDA, the EMA and other comparable international 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.

Moreover, promotional communications with respect to prescription drugs, including biologics, are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. In particular, a

product may not be promoted for uses that are not approved by the FDA as reflected in the product's approved labeling. The FDA and other agencies 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 will not be able to promote any products we develop for indications or uses for which they are not approved.

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. In addition, the FDA often requires post-marketing testing and surveillance to monitor the effects of products. The FDA, the EMA and other comparable international regulatory agencies 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:

- restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials;
- restrictions on 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;
- refusal 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 EMA or any other comparable international regulatory agency withdraws its approval of a product, we will be unable to generate revenue 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 use in the targeted human patients in order to receive regulatory approval for commercial sale. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials, and favorable data from interim analyses do not ensure the final results of a trial

will be favorable. From time to time, we have and may in the future publish or report preliminary, initial or interim data from our clinical trials, such as the data we have announced from the GENER-8-1 study for valoctocogene roxaparvovec. 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.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services reportable to the FDA or other regulatory authority. If the FDA or other regulatory authority concludes that a financial relationship between us and a principal investigator has created a conflict of interest, the FDA or other regulatory authority may question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized.

Similar rules governing clinical trials to those in place in the United States apply in the EU and the U.K. Following the U.K.'s exit from the EU, commonly referred to as Brexit, and the end of the transition period that was in place until the end of 2020, clinical trials that take place in the U.K. are seen as trials that have taken place in a "third country" and will only be considered during the course of a marketing authorization application if they are carried out on a basis that is in line with the regulations governing clinical trials in the EU. Clinical trials in the EU must be conducted in accordance with the requirements of the EU Clinical Trials Directive, as implemented in national law by individual member states, and applicable good clinical practice standards. The EU Clinical Trials Directive is due to be replaced with the new EU Clinical Trials Regulation (EU CTR) at the end of 2021. Should the U.K. not seek to align its regulations governing clinical trials with the EU CTR then trials that take place in the U.K. may carry less weight than they currently do when applying for a marketing authorization in the EU.

**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 revenue and results of operations.**

We expect that coverage and reimbursement may be increasingly restricted in all the markets in which we sell our products. The escalating cost of healthcare has led to increased pressure on the healthcare industry to reduce costs. In particular, drug pricing by pharmaceutical companies has recently come under increased scrutiny and continues to be subject to intense political and public debate in the U.S. and abroad. Governmental and private third-party payers have proposed healthcare reforms and cost reductions. A number of federal and state proposals to control the cost of healthcare, including the cost of drug

treatments, have been made in the U.S. Specifically, there have been several recent U.S. congressional inquiries and proposed bills and enacted legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. Further, Congress and the executive branch have each indicated that they will continue to seek new legislative and/or administrative measures to control drug costs. In some international markets, the government controls the pricing, which can affect the profitability of drugs. Current government regulations and possible future legislation regarding healthcare may affect coverage and reimbursement for medical treatment by third-party payers, which may render our products not commercially viable or may adversely affect our future revenues and gross margins.

International operations are also generally subject to extensive price and market regulations, and there are many proposals for additional cost-containment measures, including proposals that would directly or indirectly impose additional price controls or mandatory price cuts or reduce the value of our intellectual property portfolio. As part of these cost containment measures, some countries have imposed and continue to propose revenue caps limiting the annual volume of sales of our products. Some of these caps are significantly below the actual demand in certain countries, and if the trend regarding revenue caps continues, our future revenues and gross margins may be adversely affected.

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 revenue and results of operations.

**Government healthcare reform could increase our costs and adversely affect our revenue 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. For example, the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 (the PPACA) is a sweeping measure intended to, among other things, expand healthcare coverage within the U.S., primarily through the imposition of health insurance mandates on employers and individuals and expansion of the Medicaid program. Several provisions of the law have affected us and increased certain of our costs. Since its enactment, there have been executive, judicial and congressional challenges to certain aspects of the PPACA. For example, the U.S. Supreme Court is currently reviewing the U.S. Court of Appeals for the 5th Circuit ruling that that the individual mandate was unconstitutional and to determine the constitutionality of the PPACA in its entirety, although it is unclear when a decision will be made. It is also unclear how such litigation will 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.

We anticipate that the PPACA, as well as 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. Recently there has been heightened governmental scrutiny in countries worldwide over the manner in which manufacturers set prices for their marketed products.

In the U.S., there have been several recent congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug 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. For example, at the federal level, during the former Trump administration there were multiple executive orders issued, initiatives implemented and calls for legislation from Congress to reduce drug prices, increase competition and reduce out of pocket costs of drugs for patients. The likelihood of implementation of any of the former Trump administration healthcare reform initiatives is uncertain, particularly in light of the new Biden administration and its implementation of a regulatory freeze. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payers. In addition, individual states in the U.S. have also increasingly passed legislation and implemented regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, 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. Further, it is possible that additional governmental action is taken in response to the COVID-19 pandemic.

Likewise, in many EU countries, 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 products and any approved 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 countries 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 revenue and results of operations. Moreover, in order to obtain reimbursement for our products in some countries, we may be required to conduct clinical trials that compare the cost-effectiveness of our products to other available therapies.

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.

For more information regarding government healthcare reform, see “Government Regulation - Health Reform” in Part I, Item 1 of this Annual Report on Form 10-K for the year ended December 31, 2020.

**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. 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 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 conditions, 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. 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 stated condition for a period of seven years. Orphan drug exclusive marketing rights may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug. In addition, the FDA may approve another drug during a period of orphan drug exclusivity if the second drug is found to be clinically superior to the first drug. In the EU, a ten-year period of market exclusivity (extendable to twelve years for medicines that have complied with an agreed pediatric investigation plan pursuant to Regulation 1901/2006) is available. Orphan drug marketing exclusivity may be lost in the EU if a manufacturer is unable to supply sufficient quantities and marketing authorization may also be granted to a similar medicinal product with the same orphan indication if this medicinal product is safer, more effective or otherwise clinically superior to the original orphan medicinal product. The period of market exclusivity may, in addition, be reduced to six years if, at the end of the fifth year, it can be demonstrated on the basis of available evidence that the criteria for its designation as an orphan medicine are no longer satisfied, for example if the original orphan medicinal product has become sufficiently profitable not to justify maintenance of market exclusivity. Because the extent and scope of patent protection for some of our products is limited, orphan drug designation 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 to maintain a competitive position. If we do not obtain orphan drug exclusivity for our products that do not have broad patent protection, 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 exclusivity and could also potentially be blocked from approval of certain product candidates until the competitor product’s orphan drug exclusivity period expires. Moreover, with respect to certain biologics and gene therapies, it is uncertain how similarity between product candidates designed to treat the same rare disease or condition may affect such product candidates’ orphan drug exclusivities. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition and the same drug can be approved for different conditions and potentially used off-label in the orphan indication. Even after an orphan drug is approved and granted orphan drug exclusivity, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is safer or more effective or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process.

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

Our Aldurazyme, Brineura, Naglazyme, Palynziq and Vimizim products are regulated by the FDA as biologics under the Federal Food, Drug, and Cosmetic Act (the FDC Act) and the Public Health Service Act (the PHS Act). Biologics require the submission of a BLA and approval 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 approval of biological products that are demonstrated to be “biosimilar” or “interchangeable” with an FDA-approved biological product. A similar abridged marketing authorization process is available to biosimilar products in the EU. In order to meet the standard of interchangeability, a sponsor must demonstrate that the biosimilar product can be expected to produce the same clinical result as the reference product, and for a product that is administered more than once, that the risk of switching between the reference product and biosimilar product is not greater than the risk of maintaining the patient on the reference product. The BPCIA establishes a period of 12 years of exclusivity for reference products. In Europe, a medicinal product containing a new active substance benefits from eight years of data exclusivity, during which biosimilar applications referring to the data of that product may not be accepted by the regulatory authorities, and a further two years of market exclusivity, during which such biosimilar products may not be placed on the market. The two-year period may be extended to three years if during the first eight years a new therapeutic indication with significant clinical benefit over existing therapies is approved. Our products approved under BLAs in the U.S. or Marketing Authorization Applications (MAAs) in Europe, as well as products in development that may be approved under those regimes in the future, could be reference products for biosimilar marketing applications.

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

Changes in funding levels of government agencies can affect their ability to hire and retain key personnel and carry out their normal functions that support our business. For example, the ability of the FDA to timely review and approve INDs or marketing authorizations for our product candidates may be hindered by a lack of resources and qualified personnel. In addition, funding of other 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 government agencies to function normally and support our operations. For example, the U.S. federal government has shut down repeatedly since 1980, including for a period of 35 days beginning on December 22, 2018. During a shutdown, certain regulatory 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.

#### **Risks Related to Valoctocogene Roxaparvec**

**Our valoctocogene roxaparvec program is based on a gene therapy approach, which, as a novel technology, presents additional development and treatment risks in relation to our other, more traditional drug development programs.**

In addition to the risks set forth in this Risk Factors section associated with developing more traditional pharmaceutical drugs, there are additional, unique development and treatment risks associated with gene therapy products like our product candidate valoctocogene roxaparvec. The goal of gene therapy is to be able to correct an inborn genetic defect through one-time administration of therapeutic genetic material containing non-defective gene copies. The gene copies are designed to reside permanently in a patient, allowing the patient to produce an essential protein or ribonucleic acid (RNA) molecule that a healthy person would normally produce. There is a risk, however, that the new gene copies will produce too little or too much of the desired protein or RNA. Although a one-time administration of a gene therapy product like our product candidate valoctocogene roxaparvec is intended to correct an inborn genetic defect for the entire lifetime of a patient, there is a risk that the therapeutic effect will not be durable and production of the desired protein or RNA will decrease over time or cease entirely. Because the treatment is irreversible, there may be challenges in managing side effects, particularly those caused by potential overproduction of the desired protein. Adverse effects would not be able to be reversed or relieved by stopping dosing, and we may have to develop additional clinical safety procedures. Furthermore, because the new gene copies are designed to reside permanently in a patient, there is a risk that they will disrupt other normal biological molecules and processes, including other healthy genes, and we may not learn the nature and magnitude of these side effects until long after clinical trials have been completed.

**As compared to our other, more traditional products, our gene therapy product candidate valoctocogene roxaparvec, if approved, may present additional problems with respect to the pricing, coverage, and reimbursement and acceptance of the product candidate.**

In addition to the risks set forth in this Risk Factors section associated with commercializing more traditional pharmaceutical drugs, there are additional, unique commercial risks associated with gene therapy products like our product candidate valoctocogene roxaparvec. Due to the relative novelty of gene therapy and the potential to provide extended duration therapeutic treatment with a one-time administration, we face uncertainty with respect to the pricing, coverage and reimbursement of valoctocogene roxaparvec, if approved. In order to recover our research and development costs and commercialize this one-time treatment on a profitable basis, we expect the cost of a single administration of valoctocogene roxaparvec to be substantial. Therefore, we expect that coverage and reimbursement by governments and other third-party payers will be essential for the vast majority of patients to be able to afford valoctocogene roxaparvec. Accordingly, sales of valoctocogene roxaparvec, if

approved, will depend substantially, both domestically and internationally, on the extent to which its cost will be paid by third-party payers. Even if coverage is provided, the reimbursement amounts approved by third-party payers may not be high enough to allow us to realize sufficient revenues from our investment in the development of valoctocogene roxaparovec.

We also face uncertainty as to whether gene therapy will gain the acceptance of the public or the medical community. Even if we obtain regulatory approval for valoctocogene roxaparovec, the commercial success of valoctocogene roxaparovec will depend, in part, on the acceptance of physicians, patients and third-party payers of gene therapy products in general, and our product candidate in particular, as medically necessary, cost-effective and safe. In particular, our success will depend upon physicians prescribing our product candidate in lieu of existing treatments they are already familiar with and for which greater clinical data may be available. Moreover, physicians and patients may delay acceptance of valoctocogene roxaparovec until the product candidate has been on the market for a certain amount of time. Negative public opinion or more restrictive government regulations could have a negative effect on our business and financial condition and may delay or impair the successful commercialization of, and demand for, valoctocogene roxaparovec.

**We have implemented a data access plan for valoctocogene roxaparovec, which restricts our management's review of emerging data from these trials. Without access to ongoing data, management does not have the ability to adjust the trials based on such emerging data, which could adversely impact the ultimate outcome of these trials.**

In order to preserve the scientific integrity of the valoctocogene roxaparovec trials and to allow us to only report on data at intervals that we believe will be meaningful to investors, we have implemented a data access plan related to the ongoing open label trials, which is designed to significantly mirror blinded trials. Pursuant to this plan, the ongoing emerging data are generally not collected by us, with the exception that certain specific data points are collected and reviewed by a small group of medical personnel monitoring and managing the trial, and then, only to the extent necessary to allow them to perform their monitoring responsibilities. As we disclose and publicly discuss prior data from these trials, such discussions do not incorporate any of the currently emerging data that are being collected and reviewed by personnel monitoring the trial and, accordingly, this prior data may differ significantly from more recent data that are only available to such personnel. Further, because our management does not have access to any of the ongoing data and does not have the ability to adjust the trials based on such emerging data, the data access plan could adversely impact the ultimate outcome of the trials.

### Financial and Financing Risks

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

Since we began operations in March 1997, we have been engaged in substantial research and development and capital investments, and we have operated at a net loss for each year since our inception, with the exception of 2008, 2010 and 2020. Our future profitability and cash flows depend on our marketing and selling of our products, the receipt of regulatory approval of our product candidates, our ability to successfully manufacture and market any products, either by ourselves or jointly with others, our spending on our development programs, the impact of any possible future business development transactions and other risks set forth in this Risk Factors section. The extent of our future losses and the timing of profitability and positive cash flows are highly uncertain. If we fail to become profitable or are unable to sustain profitability and positive cash flows on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

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

As of December 31, 2020, we had cash, cash equivalents and investments totaling \$1.4 billion and debt obligations of \$1.1 billion (undiscounted), which consisted of our 0.599% senior subordinated convertible notes due in 2024 (the 2024 Notes) and our 1.25% senior subordinated convertible notes due in 2027 (the 2027 Notes). The 2024 Notes and the 2027 Notes (collectively, the Notes), if not converted, will be required to be repaid in cash at maturity in August 2024 and May 2027, respectively. We will need cash not only to pay the ongoing interest due on the Notes during their term, but also to repay the principal amount of the Notes if not converted.

In January 2016, we terminated our License and Commercialization Agreement with Ares Trading, S.A. (Merck Serono). Pursuant to the Termination and Transition Agreement related to Kuvan and the Termination Agreement related to Palynziq, we are obligated to make certain payments to Merck Serono if sales and development milestones are achieved. The remaining milestone payments that may become payable include up to a maximum of €60 million, in cash, if future sales milestones are met with respect to Kuvan and Palynziq.

We may require additional financing to fund the repayment of the Notes, future milestone payments and our future operations, including the commercialization of our products and product candidates currently under development, preclinical studies and clinical trials, and potential licenses and acquisitions. We may be unable to raise additional financing due to a variety of factors, including our financial condition, the status of our product programs, and the general condition of the financial markets. If



we fail to raise any necessary additional financing we may have to delay or terminate some or all of our product development programs and our financial condition and operating results will be adversely affected.

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

- our ability to successfully market and sell our products;
- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities the progress and success of our preclinical studies and clinical trials (including studies and the manufacture of materials);
- the timing, number, size and scope of our preclinical studies and clinical trials;
- the time and cost necessary to obtain regulatory approvals and the costs of post-marketing studies which may be required by regulatory authorities;
- the progress of research programs carried out by us;
- our possible achievement of development and commercial milestones under agreements with third parties, such as the Kuvan and Palynziq milestones under the termination agreements with Merck Serono;
- 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 Genzyme's (Genzyme) 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 securities will result in additional dilution to our stockholders. Furthermore, additional financing may not be available in amounts or on terms satisfactory to us or at all. This could result in the delay, reduction or termination of our research, which could harm our business.

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

As of December 31, 2020, we had \$1.1 billion (undiscounted) principal amount of indebtedness, including \$495.0 million (undiscounted) principal amount of indebtedness under the 2024 Notes and \$600.0 million (undiscounted) principal amount of indebtedness under the 2027 Notes. In October 2018, we also entered into an unsecured credit agreement (the 2018 Credit Facility) with Bank of America, N.A., as the administrative agent, swingline lender and a lender, Citibank N.A. as letter of credit issuer and each of Merrill Lynch, Pierce, Fenner & Smith Incorporated, Citibank, N.A. and Wells Fargo Securities, LLC as joint lead arrangers and joint bookrunners, providing up to \$200.0 million in revolving loan commitments. 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 2018 Credit Facility contains, and any future indebtedness that we may incur may contain, financial and 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 2018 Credit Facility, the outstanding borrowings thereunder could become immediately due and payable, the 2018 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 indentures governing the Notes. If we default under any of the Notes, such Notes could become immediately due and payable and it could lead to defaults under the other Notes and/or the 2018 Credit Facility.

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

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

In addition, we also may borrow up to \$200.0 million in revolving loans under the 2018 Credit Facility, which would be required to be repaid in cash at maturity on October 19, 2021.

## Manufacturing Risks

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

Before we can begin commercial manufacture of our products, regulatory authorities must approve marketing applications that identify manufacturing facilities operated by us or our contract manufacturers that have passed regulatory inspection and manufacturing processes that are acceptable to the regulatory authorities. In addition, our pharmaceutical manufacturing facilities are continuously subject to scheduled and unannounced inspection by the FDA and international regulatory authorities, before and after product approval, to monitor and ensure compliance with cGMP and other regulations. Our manufacturing facility in the U.S. has been approved by the FDA and the European Commission (EC) for the manufacture of Palynziq, and it has been approved by the FDA, the EC, and health agencies in other countries for the manufacture of Aldurazyme, Brineura, Naglazyme and Vimizim. Our manufacturing facility in Shanbally, Cork, Ireland has been approved by the FDA, the EC, and health agencies in other countries for the manufacture of Vimizim, and it has been approved by the FDA and the EMA as a formulated bulk drug substance manufacturing and quality control facility for Brineura. 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 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 or international regulatory inspection.

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

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

Due to the complexity of manufacturing our product candidates and products, we may not be able to manufacture sufficient quantities. Our inability to produce enough of our product candidate at acceptable costs may result in the delay or termination of development programs. With respect to our commercial portfolio, we may not be able to manufacture our products successfully with a commercially viable process or at a scale large enough to support their respective commercial markets or at acceptable margins.

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.

With respect to valoctocogene roxaparvovec, gene therapy products are relatively novel and complex and have only in limited cases been manufactured at scales sufficient for pivotal trials and commercialization. Few pharmaceutical contract manufacturers specialize in gene therapy products and those that do are still developing appropriate processes and facilities for large-scale production. We invested a considerable amount of capital building our own commercial gene therapy manufacturing facility, which may be subject to significant impairment if our gene therapy programs are unsuccessful. As we develop, seek to optimize and operate the valoctocogene roxaparvovec manufacturing process, we will likely face technical and scientific challenges, considerable capital costs, and potential difficulty in recruiting and hiring experienced, qualified personnel. There may also be unexpected technical or operational issues during clinical or commercial manufacturing campaigns. As a result, we could experience manufacturing delays that prevent us from completing our clinical studies in a timely manner, if at all, or commercializing valoctocogene roxaparvovec on a profitable basis, if at all.

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

Even a developed manufacturing process can encounter difficulties. Problems may arise during manufacturing for a variety of reasons, including human error, mechanical breakdowns, problems with raw materials and cell banks, malfunctions of internal information technology systems, and other events that cannot always be prevented or anticipated. Many of the processes include biological systems, which add significant complexity, as compared to chemical synthesis. We expect that, from time to time, consistent with biotechnology industry expectations, certain production lots will fail to produce product that meets our quality control release acceptance criteria. To date, our historical failure rates for all of our product programs, including Aldurazyme, Brineura, Naglazyme, Palynziq and Vimizim, 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 commercial 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 and local laws and regulations governing the use, generation, manufacture, storage, handling and disposal of hazardous materials and wastes resulting from their use. We incur significant costs in complying with these laws and regulations.

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

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

- timing, scheduling and prioritization of production by our contract manufacturers or a breach of our agreements by our contract manufacturers;
- labor interruptions;
- changes in our sources for manufacturing;
- the timing and delivery of shipments;

- our failure to locate and obtain replacement suppliers and manufacturers as needed on a timely basis; and
- conditions affecting the cost and availability of raw materials.

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 Genzyme 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 Genzyme 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 Genzyme 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 Genzyme 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 Genzyme'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 Genzyme's interest, we may be held in breach of the agreement and may lose any claim to the rights to Aldurazyme and the related intellectual property and regulatory approvals. We would then effectively be prohibited from developing and commercializing Aldurazyme. If this happened, not only would our product revenues decrease, but our share price would also decline.

#### **Risks Related to International Operations**

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

A significant portion of the sales of Aldurazyme, Brineura, Kuvan, Naglazyme and Vimizim are generated from countries other than the U.S. Similarly, we expect a significant portion of the sales of Palynziq to be generated from countries other than the U.S. 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;
- political and economic instability;
- diminished protection of intellectual property in some countries outside of the U.S.;
- trade protection measures and import or export licensing requirements;
- difficulty in staffing and managing international operations;
- differing labor regulations and business practices;
- potentially negative consequences from changes in or interpretations of tax laws;
- changes in international medical reimbursement policies and programs;
- financial risks such as longer payment cycles, difficulty collecting accounts receivable, exposure to fluctuations in foreign currency exchange rates and potential currency controls imposed by non-U.S. governments;
- regulatory and compliance risks that relate to maintaining accurate information and control over sales and distributors' and service providers' activities that may fall within the purview of the Foreign Corrupt Practices Act (the FCPA); and
- rapidly evolving global laws and regulations relating to data protection and the privacy and security of commercial and personal information.

Any of these factors may, individually or as a group, have a material adverse effect on our business and results of operations.

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 revenue 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 revenue in these countries.**

We make a significant portion of our international sales of Naglazyme and Vimizim through special access or "named patient" programs, which do not require full product approval, and we expect a significant portion of our international sales of Brineura will also be through such programs. The specifics of the programs vary from country to country. Generally, special approval must be obtained for each patient. The approval normally requires an application or a lawsuit accompanied by evidence of medical need. Generally, the approvals for each patient must be renewed from time to time.

These programs are not well defined in some countries and are subject to changes in requirements and funding levels. 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 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 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 and maintain a full product approval, 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.

**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. 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, be renewed in the future or that we will remain in compliance. 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., the United Kingdom (U.K.) Bribery Act and other similar laws in other countries in which we do business. We operate in a number of countries that are recognized to have a reputation for corruption and pose an increased risk of corrupt practices. We also regularly interact with government regulators in many countries, including those that are considered higher risk for corruption, in order to secure regulatory approval to manufacture and distribute our products. The anti-corruption and anti-bribery laws to which we are subject generally prohibit companies and their intermediaries from making improper payments to non-U.S. 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. 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 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.

**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 net assets, are exposed to changes in foreign exchange rates. As we operate in multiple foreign currencies, including the Euro, the Brazilian Real, the Great British Pound, the Canadian Dollar and several other currencies, changes in those currencies relative to the U.S. Dollar (USD) will impact our revenues and expenses. If the USD were to weaken against another currency, assuming all other variables remained constant, our revenues would increase, having a positive impact on earnings, and our overall expenses would increase.

having a negative impact on earnings. Conversely, if the USD were to strengthen against another currency, 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.

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

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

### Intellectual Property Risks

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

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

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

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

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



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

Under policies recently adopted in the EU, clinical trial data submitted to the EMA in MAAs that were traditionally regarded as confidential commercial information are now subject to public disclosure. Subject to our ability to review and redact a narrow sub-set of confidential commercial information, the new EU policies will result in the EMA's public disclosure of certain of our clinical study reports, clinical trial data summaries and clinical overviews for recently completed and future MAA submissions. The move toward public disclosure of development data 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, and enabling competitors to use our data to gain approvals for their own products.

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

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

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

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

We may also support and collaborate in research conducted by government organizations, hospitals, universities or other educational institutions. These research partners may be unwilling to grant us any exclusive rights to technology or products derived from these collaborations.

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 may be volatile, and an investment in our stock could suffer a decline in value.**

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 will fluctuate due to factors including:

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

Furthermore, the stock markets have recently experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many companies. In some cases, these fluctuations have been unrelated or disproportionate to the operating performance of those companies. In the past, companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. For example, in September 2020, after a substantial drop in our stock price that followed an announcement providing a regulatory update regarding valoctocogene roxaparvovec, 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 and misleading statements. We may be the target of additional litigation of this type in the future as well. Securities litigation against us could result in substantial costs and divert our management's time and attention from other business concerns, which could harm our business.

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

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

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

**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.

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

The terms of the Notes require us to repurchase the Notes in the event of a fundamental change. A takeover of us would trigger options by the respective holders of the applicable Notes to require us to repurchase such Notes. This may have the effect of delaying or preventing a takeover of us that would otherwise be beneficial to our stockholders or investors in the Notes.

**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 director, officer or other employee of BioMarin to us or our stockholders;
- any claim or cause of action against us or any of our 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 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 employees.**

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

Because of the specialized scientific and managerial 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. 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 this intense competition, 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. If we are unsuccessful in our recruitment and retention efforts, our business may be harmed.

### **Our success depends on our ability to manage our growth.**

Product candidates that we are currently developing or may license or acquire in the future may be intended for patient populations that are significantly larger than any of the patient populations we currently target. In order to continue development and marketing of these products, if approved, we will need to significantly expand 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 and corruption risks and our management may be unable to manage successfully future market opportunities or our relationships with customers and other third parties.

### **Changes in tax laws or regulations that are 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 enacted in 2017, informally titled the Tax Cuts and Jobs Act (TCJA), enacted many significant changes to the U.S. tax laws, including changes in corporate tax rates, the application of certain tax credits (including a reduction of tax credits under the Orphan Drug Act), the deductibility of expenses, the utilization of net operating losses (NOLs) and other deferred tax assets, and the taxation of non-U.S. earnings. Future guidance from the Internal Revenue Service and other tax authorities with respect to the TCJA may affect us, and certain aspects of the TCJA could be repealed or modified in future legislation. For example, the Coronavirus Aid, Relief, and Economic Security Act, or CARES Act, enacted on March 27, 2020, modified certain provisions of the TCJA, including provisions relating to NOL utilization. In addition, it is uncertain if and to what extent various states will conform to the TCJA, the CARES Act, or any newly enacted federal tax legislation. The impact of changes under the TCJA, the CARES Act, or future reform legislation could increase our future U.S. tax expense and could have a material adverse impact on our business and financial condition.

Moreover, changes in the tax laws of non-U.S. jurisdictions could arise, including as a result of the base erosion and profit shifting (BEPS) project that was undertaken by the Organization for Economic Co-operation and Development (OECD), or other initiatives led by the European Commission. The OECD, which represents a coalition of member countries including the U.S. and other countries in which we have operations, made several recommendations with the aim of addressing tax avoidance and ensuring that profits are taxed where economic activities generating the profits are performed and where value is created. These

changes, as adopted by countries, may increase tax uncertainty and may adversely affect our provision for income taxes, results of operations and cash flows. It is not uncommon for taxing authorities in different countries to have conflicting views, for instance, with respect to, among other things, the manner in which the arm's length standard is applied for transfer pricing purposes, or with respect to the valuation of intellectual property. If tax authorities successfully challenge our transfer prices as not reflecting arm's length transactions, they could require us to adjust our transfer prices and thereby reallocate our income to reflect these revised transfer prices, resulting in a higher tax liability. In addition, if a country from which income is reallocated does not agree with the reallocation, both that country and the other country to which the income was allocated could tax the same income, potentially resulting in double taxation. If tax authorities were to allocate income to a higher tax jurisdiction, subject our income to double taxation or assess interest and penalties, it would increase our consolidated tax liability, which could adversely affect our business, financial condition, results of operations and cash flows.

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

Federal and state false claims laws, including the civil False Claims Act, 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, recent healthcare reform legislation has 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 outside of the European Economic Area (EEA) are likely to remain uncertain for the foreseeable future. In the United States, California recently enacted the California Consumer Privacy Act (CCPA), which took effect on January 1, 2020. The CCPA gives California consumers expanded rights to access and delete their personal information, opt out of certain personal information sales, and receive detailed information about how their personal information is used. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. The CCPA will be expanded substantially on January 1, 2023 when the California Privacy Rights Act of 2020 (CPRA), becomes fully operative. The CPRA will, among other things, give consumers the ability to limit use of information deemed to be sensitive, increase the maximum penalties for violations concerning consumers under age 16, expand an individual's private right of action and establish the California Privacy Protection Agency to implement and enforce the new law and impose administrative fines. Aspects of the CCPA and CPRA and their interpretation and enforcement remain uncertain. The potential effects of the CCPA and CPRA are far-reaching and may require us to modify our data processing practices and policies and to incur substantial costs and expenses in an effort to comply.

The European Regulation 2016/679, known as the General Data Protection Regulation (GDPR), as well as EU Member State implementing legislations, apply to the processing of personal data of individuals located in the EEA, including health-related

information, by companies located in the EEA, or in certain circumstances, by companies located outside of the EEA. These laws impose strict obligations on the ability to collect, record, store, disclose, use and transmit personal data, including health-related information. These include several requirements relating to (i) obtaining, in some situations, the consent of the individuals to whom the personal data relates, (ii) the information provided to the individuals about how their personal information is used, (iii) ensuring the security and confidentiality of the personal data, (iv) the obligation to notify regulatory authorities and affected individuals of personal data breaches, (v) extensive internal privacy governance obligations, and (vi) obligations to honor rights of individuals in relation to their personal data (for example, the right to access, correct and delete their data). The GDPR prohibits the transfer of personal data to countries outside of the EEA, such as the United States, which are not considered by the European Commission to provide an adequate level of data protection. Switzerland has adopted similar restrictions. Potential pecuniary fines for noncompliant companies may be up to the greater of €20 million or 4% of annual global revenue. The GDPR has increased our responsibility and liability in relation to personal data that we process and has increased our compliance costs.

The GDPR and other European data protection laws generally restrict the transfer of personal information from Europe, including the EEA and Switzerland, to the U.S. and most other countries unless the parties to the transfer have implemented specific safeguards to protect the transferred personal information. One of the primary safeguards allowing U.S. companies to import personal information from Europe has been the European Commission's Standard Contractual Clauses (SCCs). However, the Court of Justice of the EU issued a decision that called into question whether the SCCs can lawfully be used for transfers of personal information from Europe to the United States or most other countries. At present, there are few, if any, viable alternatives to the SCCs, on which we have relied for personal information transfers from Europe to the United States and other "third countries." The Court of Justice of the EU ruled that subject to their containing "effective mechanisms" to ensure compliance with the protections offered by EU law, namely implementation of transfer impact assessments regarding country surveillance laws, the SCCs were generally upheld. New SCCs will be published in the coming months and will require implementation within 12 months from publication. Compliance with the regulations covering the transfer of personal information from Europe to the U.S. has increased our compliance costs.

Following the U.K.'s withdrawal from the EU on January 31, 2020 and the end of the transitional arrangements agreed to between the U.K. and EU, the GDPR no longer has effect in law in the U.K. However, the data protection obligations of the GDPR continue to apply to U.K.-related processing of personal data in substantially unvaried form under the so-called "UK GDPR" (i.e., the GDPR as it continues to form part of law in the U.K. However, going forward, there will be increasing scope for divergence in application, interpretation and enforcement of the data protection law as between the U.K. and EEA. Furthermore, the relationship between the U.K. and the EEA in relation to certain aspects of data protection law remains unclear, which could expose us to two parallel regimes, each of which potentially authorizes similar fines and other potentially divergent enforcement actions for certain violations. Also in the event of a violation of the GDPR affecting data subjects across the U.K. and the EEA, we could potentially be investigated by, and ultimately fined by the U.K. Information Commissioner's Office and the supervisory authority in each and every EEA Member State where data subjects have been affected by such violation.

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) and teaching hospitals, as well as investment and ownership interests held by such physicians and their immediate family members during the preceding calendar year. Effective January 1, 2022, manufacturers will also be required to report on payments or transfers of value, and ownership and investment interests held, during the previous year to physician assistants, nurse practitioners, clinical nurse specialists, anesthesiologist assistants, certified registered nurse anesthetists, and certified nurse-midwives. In addition, there has been a recent trend of increased state regulation of payments made to physicians. Certain states and/or local jurisdictions mandate implementation of compliance programs, compliance with the Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers and the Pharmaceutical Research and Manufacturers of America (PhRMA) Code on Interactions with Healthcare Professionals, the registration of pharmaceutical sales representatives and/or the tracking and reporting of gifts, compensation and other remuneration to physicians, marketing expenditures, and drug pricing. Likewise, in many non-U.S. countries there is an increasing focus on the relationship between drug companies and healthcare practitioners. Recently enacted non-U.S. legislation creates reporting obligations on payments, gifts and benefits made to these professionals; however, implementing regulations enacting such laws are still pending and subject to varying interpretations by courts and government agencies. The shifting regulatory environment and the need to implement systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the costs of maintaining compliance and the possibility that we may violate one or more of the requirements and be subject to fines or sanctions.

Due to the breadth of the healthcare and privacy and data protection laws described above, the narrowness of available statutory and regulatory exceptions and safe harbors and the increased focus by law enforcement agencies 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, diminished profits and future earnings, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-

compliance with these laws, curtailment of our operations, and debarment, suspension or exclusion from participation in government healthcare programs, any of which could adversely affect our business, financial condition and results of operations.

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

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

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

We rely significantly on our information technology systems to effectively manage and maintain our operations, inventory and internal reports, to manufacture and ship products to customers and to timely invoice them. Any failure, inadequacy or interruption of that infrastructure or security lapse of that technology, including cybersecurity incidents or attacks, could harm our ability to operate our business effectively. Our ability to manage and maintain our operations, inventory and internal reports, to manufacture and ship our products to customers and timely invoice them depends significantly on our enterprise resource planning, production management and other information systems. Our technology systems, including our cloud technologies, continue to increase in multitude and complexity, making them potentially vulnerable to breakdown, cyberattack and other disruptions. Potential problems and interruptions associated with the implementation of new or upgraded technology systems or with maintenance or adequate support of existing systems could disrupt or reduce the efficiency of our operations and expose us to greater risk of security breaches. Cybersecurity incidents, including phishing attacks and attempts to misappropriate or compromise confidential or proprietary information or sabotage enterprise IT systems are becoming increasingly frequent and more sophisticated. Cybersecurity incidents resulting in the failure of our enterprise resource planning system, production management or other systems to operate effectively or to integrate with other systems, or a breach in security or other unauthorized access or unavailability of these systems, have occurred in the past and may affect our ability in the future to manage and maintain our operations, inventory and internal reports, and result in delays in product fulfillment and reduced efficiency of our operations.

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

We have experienced and may continue to experience cybersecurity incidents. Although to our knowledge we have not experienced any material incident or interruption to date, if such an 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 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.

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

The occurrence of an earthquake or other catastrophic disaster could cause damage to our facility and equipment, or that of our third-party manufacturers or single-source suppliers, which could materially impair the ability for us or our third-party manufacturers to manufacture our products and product candidates. Our Galli Drive facility, located in Novato, California, is currently our only manufacturing facility for Aldurazyme, Naglazyme and Palynziq and is one of two manufacturing facilities for Brineura and Vimizim. Our gene therapy manufacturing facility is also located in Novato, California, and it is currently our only manufacturing facility to support valoctocogene roxaparvovec clinical development activities and the anticipated commercial demand for valoctocogene roxaparvovec, if approved. These facilities are located in the San Francisco Bay Area near known earthquake fault zones and are vulnerable to significant damage from earthquakes. We, the third-party manufacturers with whom we contract and our single-source suppliers of raw materials, which include many of our critical raw materials, are also vulnerable to damage from other types of disasters, including fires, explosions, floods, and similar events. If any disaster were to occur, or any terrorist or criminal activity caused significant damage to our facilities or the facilities of our third-party manufacturers and suppliers, our ability to manufacture our products, or to have our products manufactured, could be seriously, or potentially completely, impaired, and our commercialization efforts and revenue 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 significant delays in the manufacture of our products and adversely impact our commercial operations and revenues. Pacific Gas and Electric Company, the electric utility in the San Francisco Bay Area where many of our facilities are located, commenced widespread blackouts during the fall of 2019 to avoid and contain wildfires sparked during strong wind events by downed power lines or equipment failures. While we have not experienced damage to our facilities or material disruption to our operations as a result of these power outages, ongoing blackouts, particularly if prolonged or frequent, could impact our business going forward. 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 and foreign currency exchange rates and overall economic conditions and uncertainties, including those resulting from the current and future conditions in the global financial markets. For instance, if inflation or other factors were to significantly increase our business costs, 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 counter parties 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 revenue 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. 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.

### Item 1B. Unresolved Staff Comments

None.

### Item 2. Properties

The following table contains information about our significant owned and leased properties as of December 31, 2020:

Location	Approximate Square Feet	Use	Lease Expiration Date
San Rafael facility, San Rafael, California	407,300	Corporate headquarters, laboratory and office	Owned property
Several facilities in Novato, California	293,300	Clinical and commercial manufacturing, laboratory and office	Owned property
Several leased facilities in Novato, California	203,700	Office and warehouse	2023
Shanbally facility, Cork, Ireland	203,500	Manufacturing, laboratory and office	Owned property

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

### Item 3. Legal Proceedings

On September 25, 2020, a purported shareholder class action lawsuit was filed against us, our Chief Executive Officer, our President of Worldwide Research and Development and our Executive Vice President and Chief Financial Officer in the United States District Court in the Northern District of California, alleging violations under Sections 10(b) and 20(a) of the Securities Exchange Act of 1934. The complaint alleges that the Company made materially false or misleading statements regarding the clinical trials and Biologics License Application (BLA) for valoctocogene roxaparovec by purportedly failing to disclose that differences between the Company's Phase 1/2 and Phase 3 clinical studies limited the ability of the Phase 1/2 study to support valoctocogene roxaparovec's durability of effect and, as a result, that it was foreseeable that the FDA would not approve the BLA without additional data. The complaint seeks an unspecified amount of damages, pre-judgment and post-judgment interest, attorneys' fees, expert fees, and other costs. In December 2020, the court hearing the case appointed Arbejdsmarkedets Tillægspension as lead plaintiff. The lead plaintiff filed an amended complaint in February 2021, dropping our Chief Financial Officer as a defendant, and asserting that the Company misled investors about the progress of the FDA's review of our BLA for valoctocogene roxaparovec. Our motion to dismiss the complaint is due on April 22, 2021. We believe that the claims have no merit and we intend to vigorously defend this action.

### 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**

We did not sell any unregistered securities during the year ended December 31, 2020 that were not previously disclosed in a Quarterly Report on Form 10-Q or in a Current Report on Form 8-K.

#### **Issuer Purchases of Equity Securities**

None.

#### **Holders**

As of February 12, 2021, there were 39 holders of record of 181,829,680 outstanding shares of our common stock.

#### **Performance Graph**

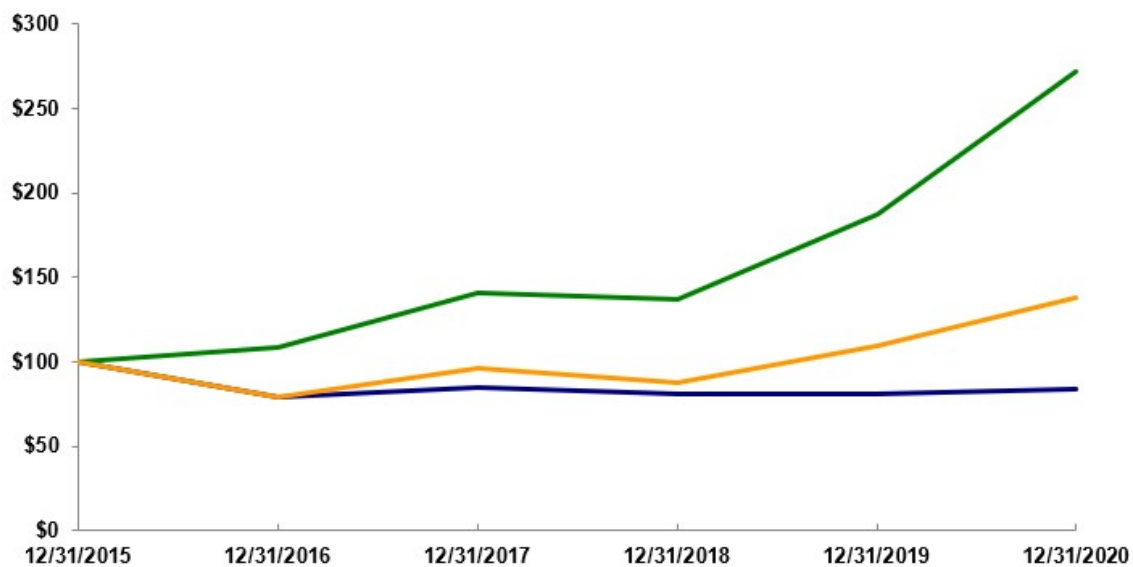
*The following is not deemed "filed" with the Securities and Exchange Commission and is not to be incorporated by reference into any filing we make under the Securities Act of 1933, as amended, whether made before or after the date hereof and irrespective of any general incorporation by reference language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

The following graph shows the value of an investment in BioMarin common stock, the Nasdaq Composite Index and the Nasdaq Biotechnology Index, 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 both the Nasdaq Composite Index and the Nasdaq Biotechnology Index. 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.

### COMPARISON OF FIVE-YEAR CUMULATIVE TOTAL RETURN\*

Among BioMarin Pharmaceutical Inc., the Nasdaq Composite Index and the NASDAQ Biotechnology Index



— BioMarin Pharmaceutical Inc. — Nasdaq Composite Index — Nasdaq Biotechnology Index

\* \$100 invested on December 31, 2015 in stock or index, including reinvestment of dividends.

	2015	2016	2017	2018	2019	2020
BioMarin Pharmaceutical Inc.	\$ 100.00	\$ 79.08	\$ 85.12	\$ 81.28	\$ 80.71	\$ 83.71
Nasdaq Composite Index	\$ 100.00	\$ 108.87	\$ 141.13	\$ 137.12	\$ 187.44	\$ 271.64
Nasdaq Biotechnology Index	\$ 100.00	\$ 78.65	\$ 95.67	\$ 87.19	\$ 109.08	\$ 137.90

**Item 6. Selected Consolidated Financial Data**

We derived the selected consolidated statements of operations data for the years ended December 31, 2020, 2019 and 2018 and the selected consolidated balance sheet data as of December 31, 2020 and 2019 from the audited Consolidated Financial Statements appearing elsewhere in this Annual Report on Form 10-K. We derived the selected consolidated statements of operations data for the years ended December 31, 2017 and 2016 and the selected consolidated balance sheet data as of December 31, 2018, 2017 and 2016 from audited Consolidated Financial Statements not included in this Annual Report on Form 10-K. The information set forth below is not necessarily indicative of results of future operations, and should be read in conjunction with Part II, Item 7, "Management's Discussion and Analysis of Financial Condition and Results of Operations," and the Consolidated Financial Statements and related notes thereto included in Item 15 of this Annual Report on Form 10-K to fully understand factors that may affect the comparability of the information presented below:

	Years Ended December 31, (In millions, except for per share data)				
	2020 <sup>(1)</sup>	2019 <sup>(1)</sup>	2018 <sup>(1)</sup>	2017	2016
<b>Consolidated Statements of Operations data:</b>					
Total revenues	\$ 1,860.5	\$ 1,704.0	\$ 1,491.2	\$ 1,313.6	\$ 1,116.9
Total costs and expenses	\$ 1,897.2	\$ 1,804.5	\$ 1,614.7	\$ 1,328.3	\$ 1,920.3
Loss from operations	\$ (36.8)	\$ (100.5)	\$ (123.5)	\$ (14.7)	\$ (803.4)
Provision for (benefit from) income taxes	\$ (901.4)	\$ (71.0)	\$ (65.5)	\$ 81.2	\$ (200.8)
Net Income (loss)	\$ 859.1	\$ (23.8)	\$ (77.2)	\$ (117.0)	\$ (630.2)
Net income (loss) per share, basic	\$ 4.75	\$ (0.13)	\$ (0.44)	\$ (0.67)	\$ (3.80)
Net income (loss) per share, diluted	\$ 4.53	\$ (0.13)	\$ (0.44)	\$ (0.67)	\$ (3.81)
Weighted average common shares outstanding, basic	180.8	179.0	177.1	174.4	166.0
Weighted average common shares outstanding, diluted	191.7	179.0	177.3	174.4	166.2
	December 31, (In millions)				
	2020	2019	2018	2017	2016
<b>Consolidated Balance Sheets data:</b>					
Cash, cash equivalents and investments	\$ 1,350.9	\$ 1,165.8	\$ 1,320.2	\$ 1,781.7	\$ 1,362.4
Total assets	\$ 5,848.0	\$ 4,690.0	\$ 4,427.1	\$ 4,633.1	\$ 4,023.7
Convertible senior notes, net	\$ 1,075.1	\$ 848.1	\$ 830.4	\$ 1,174.5	\$ 683.2
Other long-term obligations	\$ 174.3	\$ 148.9	\$ 105.5	\$ 194.4	\$ 157.3
Total stockholders' equity	\$ 4,106.0	\$ 3,122.4	\$ 2,967.9	\$ 2,808.7	\$ 2,766.3

(1) Refer to "Management's Discussion and Analysis of Financial Condition and Results of Operations — Results of Operations" in Part II, Item 7 of this Annual Report on Form 10-K for additional discussion of our operating results and changes in our financial position.

**Quarterly Financial Data (unaudited)**

You should read the following tables presenting our unaudited quarterly results of operations in conjunction with the Consolidated Financial Statements and related notes contained elsewhere in this Annual Report on Form 10-K. We have prepared this unaudited information on the same basis as our audited Consolidated Financial Statements. Our quarterly operating results have fluctuated in the past and may continue to do so in the future as a result of a number of factors, including, but not limited to, the timing and nature of research and development activities.

	Three Months Ended (In millions, except per share data, unaudited)			
	March 31,	June 30,	September 30,	December 31,
<b>2020:</b>				
Total revenues	\$ 502.1	\$ 429.5	\$ 476.8	\$ 452.1
Net income (loss)	\$ 81.4	\$ (29.2)	\$ 784.8	\$ 22.1
Net income (loss) per share, basic	\$ 0.45	\$ (0.16)	\$ 4.33	\$ 0.12
Net income (loss) per share, diluted	\$ 0.44	\$ (0.16)	\$ 4.01	\$ 0.12
<b>2019:</b>				
Total revenues	\$ 400.7	\$ 387.8	\$ 461.1	\$ 454.4
Net income (loss)	\$ (56.5)	\$ (37.4)	\$ 55.0	\$ 15.0
Net income (loss) per share, basic	\$ (0.32)	\$ (0.21)	\$ 0.31	\$ 0.08
Net income (loss) per share, diluted	\$ (0.32)	\$ (0.21)	\$ 0.30	\$ 0.08

Refer to "Management's Discussion and Analysis of Financial Condition and Results of Operations - Results of Operations" in Part II, Item 7 of this Annual Report on Form 10-K for discussion of our operating results.

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

The following Management's Discussion and Analysis of Financial Condition and Results of Operations (MD&A) is intended to help the reader understand our results of operations and financial condition. MD&A is provided as a supplement to, and should be read in conjunction with, our audited Consolidated Financial Statements and the accompanying notes to the Consolidated Financial Statements and other disclosures included in this Annual Report on Form 10-K, including the disclosures under "Risk Factors" in Part I, Item 1A of this Annual Report on Form 10-K. In particular, we encourage you to review the risk factor related to the impact of the coronavirus pandemic, "The COVID-19 pandemic could materially adversely affect our business, results of operations and financial condition." 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 global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare diseases and medical conditions. We select product candidates for diseases and conditions that represent a significant unmet medical need, have well-understood biology and provide an opportunity to be first-to-market or offer a significant benefit over existing products.

Our portfolio consists of several commercial products and multiple clinical and preclinical product candidates for the treatment of various diseases. A summary of our commercial products, as of December 31, 2020, is provided below:

Commercial Products	Indication	United States Orphan Drug Exclusivity Expiration <sup>(1)</sup>	United States Biologic Exclusivity Expiration <sup>(2)</sup>	European Union Orphan Drug Exclusivity Expiration <sup>(1)</sup>
Aldurazyme (laronidase)	MPS I <sup>(3)</sup>	Expired	Expired	Expired
Brineura (cerliponase alfa)	CLN2 <sup>(4)</sup>	2024	2029	2027
Kuvan (sapropterin dihydrochloride)	PKU <sup>(5)</sup>	Expired	Not Applicable	Expired
Naglazyme (galsulfase)	MPS VI <sup>(6)</sup>	Expired	Expired	Expired
Palynziq (pegvaliase-pqpz) <sup>(7)</sup>	PKU	2025	2030	2029
Vimizim (elosulfase alpha)	MPS IVA <sup>(8)</sup>	2021	2026	2024

(1) See "Government Regulation—Orphan Drug Designation" in Part I, Item 1 of this Annual Report on Form 10-K for further discussion

(2) See "Government Regulation—Healthcare Reform" in Part I, Item 1 of this Annual Report on Form 10-K for further discussion

(3) For the treatment of Mucopolysaccharidosis I (MPS I)

(4) For the treatment of late infantile neuronal ceroid lipofuscinosis type 2 (CLN2)

(5) For the treatment of phenylketonuria (PKU)

(6) For the treatment of Mucopolysaccharidosis VI (MPS VI)

(7) For adult patients with PKU

(8) For the treatment of Mucopolysaccharidosis IV Type A (MPS IVA)

**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
(In millions of U.S. Dollars, except as otherwise disclosed)

A summary of our on-going major development programs as of December 31, 2020, is provided below:

Major Product Candidates in Development	Target Indication	U.S. Orphan Designation	EU Orphan Designation	Stage
Valoctocogene roxaparvovec	Severe Hemophilia A	Yes	Yes	Clinical Phase 3
Vosoritide	Achondroplasia	Yes	Yes	Clinical Phase 3
BMN 307	PKU	Yes	Yes	Clinical Phase 1/2

*Uncertainty Relating to the COVID-19 Pandemic*

The outbreak of novel coronavirus disease (COVID-19) continues to affect economies and business around the world. Our global revenue sources, mostly in the form of demand interruptions such as missed patient infusions and delayed treatment starts for new patients, and our overall business operations were impacted by COVID-19 during the year ended December 31, 2020 and we anticipate a continued impact on our financial results in fiscal year 2021. The extent and duration of such effects are highly 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 related to the impact of the coronavirus pandemic, "The COVID-19 pandemic could materially adversely affect our business, results of operations and financial condition." described in "Risk Factors" in Part I, Item 1A of this Annual Report on Form 10-K.

*Business Developments*

We continued to grow our commercial business and advance our product candidate pipeline during 2020. We believe that the combination of our internal research programs, acquisitions and partnerships will allow us to continue to develop and commercialize innovative therapies for people with serious and life-threatening rare diseases and medical conditions. Below is a summary of key business developments:

**Continued Emphasis on Research and Development**

- Vosoritide - In February 2021, we announced that the FDA granted priority review designation for our New Drug Application (NDA) for vosoritide, an investigational, once daily injection analog of C-type Natriuretic Peptide (CNP) for children with achondroplasia, the most common form of disproportionate short stature in humans.

In December 2020, we announced results from our pivotal global Phase 3 randomized, double-blind, placebo-controlled study of vosoritide in approximately 121 children with achondroplasia ages 5-14 for two years. The data demonstrated that children in the open-label long-term extension of the Phase 3 study maintained an increase in Annual Growth Velocity through the second year of continuous treatment. An analysis comparing 52 children who were randomized and treated with vosoritide for two years to 38 children from the run-in study who were randomized to receive placebo with an untreated observation period of two years showed improvement in one-year height change in the treated group relative to the untreated group. The one-year height change improvement in the second year of treatment, 1.79 cm, was similar to the one-year height change improvement in the first year of treatment, 1.73 cm. The cumulative height gain over the 2-year period for treated children was 3.52 cm more than untreated children.

In 2020, marketing applications for vosoritide were validated and accepted by the EMA and FDA, respectively. The Committee for Medicinal Products for Human Use (CHMP) opinion is expected in Europe in June 2021. The U.S. NDA for vosoritide is under review by the FDA with a Prescription Drug User Fee Act (PDUFA) Target Action Date of August 20, 2021. Vosoritide has Orphan Drug designation from the FDA and EMA.
- BMN 307 - In February 2021, we announced that we plan to dose escalate participants in PHEarless, the Phase 1/2 study of BMN 307 our gene therapy candidate for PKU based on encouraging Phe lowering and safety signals observed in study participants who were treated with the lowest dose. Both the FDA and EMA have granted BMN 307 Orphan Drug Status. Additionally, the FDA has granted fast track designation to BMN 307. All subjects participating in the PHEarless study are receiving product made at commercial scale from our gene therapy manufacturing facility.
- Valoctocogene roxaparvovec – In January 2021, we announced topline results from our ongoing global Phase 3 study of valoctocogene roxaparvovec, an investigational gene therapy for the treatment of adults with severe hemophilia A. All 134 study participants received a single dose of valoctocogene roxaparvovec and completed a year or more of follow-up. Data from the study in the pre-specified primary analysis for Annualized Bleeding Rate (ABR) showed that a single dose of valoctocogene roxaparvovec significantly reduced ABR by 84% compared

**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
**(In millions of U.S. Dollars, except as otherwise disclosed)**

with prior treatment with prophylactic Factor VIII infusions from 4.8 (median 2.8) bleeding episodes per year at baseline to 0.8 (median 0.0). These results were from a pre-specified group of 112 participants in a non-interventional prospective baseline observational study with a median follow-up of 60.1 weeks after dosing with valoctocogene roxaparvovec. Of the 112 rollover study participants, 80% were bleed-free starting at week five after treatment.

Valoctocogene roxaparvovec also significantly reduced the mean annualized Factor VIII usage in the rollover population by 99%, from 135.9 (median 128.6) to 2.0 (median 0.0) infusions per year. At the end of the first year post-infusion with valoctocogene roxaparvovec, 132 participants in the modified intent-to-treat (miTT) population had a mean endogenous Factor VIII expression level of 42.9 (SD 45.5, median 23.9) IU/dL, as measured by the chromogenic substrate assay, supporting the clinical benefits observed with significant reduction of bleeding episodes and Factor VIII infusion rate. Factor VIII expression declined at a slower rate compared to the Phase 1/2 study, and remained in a range to provide hemostatic efficacy. In the 17 participants who comprise a subset of the miTT population and had been dosed at least two years prior to the data cut date, Factor VIII expression declined from a mean of 42.2 (SD 50.9, median 23.9) IU/dL at the end of year one to a mean of 24.4 (SD 29.2, median 14.7) IU/dL at the end of year two with continued hemostatic efficacy demonstrated by a mean ABR of 0.9 (median 0.0) bleeding episodes per year

In the United States, the Food and Drug Administration (FDA) recommended that we complete the Phase 3 study and submit two-year follow-up safety and efficacy data on all study participants. We plan to meet with FDA to review the two-year data request and share the Phase 3 results announced in January 2021. We are targeting resubmission of the Marketing Authorization Application (MAA) with these results to the European Medicines Agency (EMA) in the second quarter of 2021 pending confirmation in presubmission meetings.

- **BMN 255** - In January 2021, we announced that we filed an Investigational New Drug application (IND) for BMN 255, a small molecule for the treatment of a subset of chronic renal disease. BMN 255 was driven by genetic discoveries for both mechanism and for identifying individuals for treatment.
- **Palynziq** - In October 2020 we announced that the FDA approved our supplemental BLA to increase the maximum allowable dose of Palynziq Injection for treatment of adults with PKU to 60 mg daily. Previously, the maximum dose was 40 mg daily. In the Phase 3 PRISM studies, 19% of study participants required a 60 mg dose to achieve adequate response to Palynziq.  
Palynziq is indicated to reduce blood Phe concentrations in adults with PKU, who have uncontrolled blood Phe concentrations greater than 600 µmol/L on existing management. Palynziq, a PEGylated recombinant phenylalanine ammonia lyase enzyme, is the first and only approved enzyme substitution therapy to target the underlying cause of PKU by helping the body to break down Phe.
- **BMN 331** - In July 2020, we began IND-enabling studies for our third gene therapy product candidate, BMN 331, for the treatment of hereditary angioedema.
- **BMN 351** - IND-enabling studies are underway for BMN 351, an oligonucleotide therapy that has demonstrated a high-level of protein expression in pre-clinical models for the treatment of Duchenne Muscular Dystrophy. We intend to determine the timing of a potential IND filing at the end of the year based on the results of these studies.
- **Gene therapy manufacturing facility** - In January 2020, we announced significant improvements in productivity at our gene therapy facility which has the potential to support the commercial production of multiple gene therapy products to meet global commercial demand, depending on dose and production mix.

### *2021 Outlook*

In 2021, we will continue to focus on our key operating objectives which include continued progression of our product pipeline and continued uptake of our commercial products. From a research and development (R&D) perspective, we expect to continue to invest in our various ongoing clinical studies which support both our commercial products and research and early development pipeline. We expect to move forward on a number of late-stage clinical studies for new product candidates and plan to file marketing applications for various therapeutic areas.

From a commercial perspective, we expect to continue to build-out our commercial organization to support the commercialization of Palynziq and incur pre-commercialization expenses related to vosoritide.

While the continued impact of the COVID-19 pandemic on the global business environment remains uncertain, we expect the conditions and effects of the pandemic on our business will persist. We expect significant declines in Kuvan revenues in the U.S. due to the October 2020 loss of market exclusivity and plan to implement strategies to retain market share aimed to mitigate a complete loss of market share. We expect to experience continued growth in our base business for the other commercial products.



**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
(In millions of U.S. Dollars, except as otherwise disclosed)

We continue to monitor conditions in the macroeconomic environment that could affect our ability to achieve our goals, such as the continuing impact of the COVID-19 pandemic on all aspects of our business, changes in the reimbursement and payer landscape, changes in economic conditions in certain key markets, particularly in Europe and Latin America, patent expiration of competitive products and the launch of generic competitors, government pricing pressures internationally and the potential volatility in foreign currency exchange rates. We will adjust our business processes, as appropriate, to attempt to mitigate these risks to our business.

We expect that our product pipeline investments and expanding commercial infrastructure will enable us to execute on our 2021 operating objectives.

#### 2020 Financial Highlights

Key components of our results of operations include the following:

	Years Ended December 31,		
	2020	2019	2018
Total revenues	\$ 1,860.5	\$ 1,704.0	\$ 1,491.2
Cost of sales	\$ 524.3	\$ 359.5	\$ 315.3
R&D expense	\$ 628.1	\$ 715.0	\$ 696.3
Selling, general and administrative (SG&A) expense	\$ 737.7	\$ 680.9	\$ 604.4
Gain on sale of nonfinancial assets	\$ (59.5)	\$ (25.0)	\$ (50.0)
Benefit from income taxes	\$ (901.4)	\$ (71.0)	\$ (65.5)
Net income (loss)	\$ 859.1	\$ (23.8)	\$ (77.2)

The increase in Net Income for the year ended December 31, 2020 as compared to 2019 was primarily attributed to:

- an increase in the benefit from income taxes primarily due to the completion of an intra-entity transfer of certain intellectual property rights to an Irish subsidiary, to better align ownership of the intellectual property rights with how our business operates, resulting in a tax benefit of \$835.1 million based on the fair value of the transferred intellectual property rights;
- decreased R&D expense primarily due to lower clinical activity spend for valoctocogene roxaparvovec and for tralesenidase alfa which was licensed to a third-party in the fourth quarter of 2019; and
- an increase in the gain of nonfinancial assets due primarily to the divestiture and sale of Firdapse in 2020; partially offset by
- decreased gross profits due primarily to \$87.2 million of inventory charges recorded in 2020 related to pre-launch valoctocogene roxaparvovec inventory reserves due to regulatory responses received in the third quarter of 2020 requesting additional data extending anticipated regulatory approval timelines; and
- higher SG&A expense primarily due to an increase in pre-commercial activities related to valoctocogene roxaparvovec and vosoritide.

See "Results of Operations" below for additional information related to the Net Income (Loss) fluctuations presented above.

Our cash, cash equivalents and investments totaled \$1.4 billion as of December 31, 2020, compared to \$1.2 billion as of December 31, 2019. We have historically financed our operations primarily through our cash flows from operating activities and the issuance of common stock and convertible debt. We will be highly dependent on our net product revenues to supplement our current liquidity and fund our operations for the foreseeable future. We may in the future elect to supplement this with further debt or equity offerings or commercial borrowing. Further, depending on market conditions, our financial position and performance and other factors, we may in the future choose to use a portion of our cash, cash equivalents or investments to repurchase our convertible debt or other securities. See "Financial Position, Liquidity and Capital Resources" below for a further discussion of our liquidity and capital resources.

#### Critical Accounting Policies, Estimates and Judgments

In preparing our Consolidated Financial Statements in accordance with U.S. GAAP and pursuant to the rules and regulations promulgated by the Securities and Exchange Commission (the SEC), we make assumptions, judgments and estimates

**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
**(In millions of U.S. Dollars, except as otherwise disclosed)**

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.

The full extent to which the ongoing COVID-19 pandemic will directly or indirectly impact our business, results of operations and financial condition, including revenues, expenses, reserves and allowances, manufacturing, clinical trials and research and development costs will depend on future developments that continue to remain highly uncertain at this time. As events continue to evolve and additional information becomes available, our estimates may change materially in future periods.

While our significant accounting policies are more fully described in Note 3 to our accompanying Consolidated Financial Statements included in this Annual Report on Form 10-K, we believe the critical accounting policies below reflect the more significant judgments and estimates used in the preparation of our Consolidated Financial Statements.

- Revenue Recognition and Related Allowances
- Inventory Produced Prior to Regulatory Approval
- Valuation of Goodwill and Acquired Intangible Assets
- Valuation of Contingent Consideration
- Income Taxes

Historically, our assumptions, judgments and estimates relative to our critical accounting policies have not differed materially from actual results.

***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 Genzyme (Genzyme) depending on sales volume, which is included in Net Product Revenues in our Consolidated Statements of Operations. We recognize our best estimate of the entire revenue that we expect to receive when the product is released and control is transferred to Genzyme. We record Aldurazyme net product revenues based on the estimated variable consideration payable when the product is sold through by Genzyme. Actual amounts of consideration ultimately received may differ from our estimates. Differences between the estimated variable consideration to be received from Genzyme 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, in addition to 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 include amounts paid to Medicaid, other government programs, certain managed care providers, as well as foreign government rebates. Rebates, 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 historical experience, customer mix, current contractual and statutory obligations, specific known market events and trends and industry data. We evaluate our customer mix to estimate which sales will be subject to these revenue dilutive items and consider changes to government program guidelines that would impact the actual rebates and/or our estimates of which sales qualify for such rebates.

We update our estimates and assumptions each quarter based on actual historical experience, current contractual and statutory requirements, specific known market events and trends and forecasted customer buying and payment patterns and record any necessary adjustments to our reserves 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.

**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
(In millions of U.S. Dollars, except as otherwise disclosed)

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, 2020	\$ 114.4	\$	239.9	\$	(249.9)	\$	104.4
Year ended December 31, 2019	80.7		198.1		(164.4)		114.4
Year ended December 31, 2018	\$ 63.3	\$	151.6	\$	(134.2)	\$	80.7

#### ***Inventory Produced Prior to Regulatory Approval***

When future commercialization for a product candidate is considered probable and management believes that material uncertainties related to the ultimate regulatory approval have been significantly reduced and we expect to realize economic benefit in the future, we capitalize pre-launch or pre-qualification manufacturing costs prior to regulatory approval. For inventories that are capitalized in preparation of product launch, management considers a number of factors based on the information available at the time, including the product candidate's current status in the drug development and regulatory approval process, results from the related pivotal clinical trial, results from meetings with relevant regulatory agencies prior to the filing of regulatory applications, historical experience, as well as potential impediments to the approval process such as product safety or efficacy, as well as commercialization and market trends.

In applying the lower of cost or net realizable value to pre-launch inventory, we estimate a range of likely commercial prices based on our comparable commercial products and consider the product candidate's stability data for all of the pre-approval production to date to determine whether there is adequate expected shelf life for the capitalized pre-launch production costs. If the criteria for capitalizing inventory produced prior to regulatory approval are not met, we recognize such costs as R&D expense in the period incurred. As of December 31, 2020, there were no pre-launch inventory costs on our Consolidated Balance Sheets.

#### ***Valuation of Goodwill and Acquired Intangible Assets***

We have recorded goodwill and acquired intangible assets primarily related to in-process research and development (IPR&D) projects through acquisitions accounted for as business combinations. When identifiable intangible assets, including IPR&D, are acquired, we determine the fair value of these assets as of the acquisition date. Discounted cash flow models are typically used in these valuations if quoted market prices are not available, and the models require significant estimates and assumptions including but not limited to:

- estimating the time and resources needed to complete the development and approval of product candidate;
- estimating future cash flows from product sales;
- developing appropriate probability of success rates for unapproved product candidates considering their stages of development;
- projecting timing of regulatory approval; and
- risks related to the viability of and potential alternative treatments in any future target markets.

Goodwill represents the excess of purchase price over fair value of net assets acquired in a business combination accounted for by the acquisition method of accounting and is not amortized, but subject to impairment testing. We review our goodwill and indefinite lived intangible assets for impairment annually in the fourth quarter, or more frequently if warranted by events or changes in circumstances indicate that the carrying amount may not be recoverable.

We assess goodwill impairment by comparing the fair value of our single 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 will be recorded.

We assess impairment of indefinite-lived intangible assets first by performing a qualitative assessment. If the qualitative assessment indicates that it is more likely than not that the fair value of our indefinite-lived intangible assets is less than its carrying amount, then we will perform a quantitative assessment and record an impairment loss. We assess definite-lived intangible assets for recoverability when there is an indication of impairment 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. As of December 31, 2020, we had no indefinite-lived intangible assets on our Consolidated Balance Sheets.

**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
**(In millions of U.S. Dollars, except as otherwise disclosed)**

**Valuation of Contingent Consideration**

Significant estimates and judgments are required in determining the acquisition fair value of any contingent obligations incurred in connection with an acquisition. We estimate the fair value of contingent consideration utilizing a probability-based income approach inclusive of an estimated discount rate. Each period we reassess the fair value of the contingent consideration associated with certain acquisitions and record increases in the fair value as contingent consideration expense and record decreases in the fair value as a reduction of contingent consideration expense. Changes in the fair value of the contingent consideration can result from changes to one or multiple inputs including the estimated probability with respect to regulatory approval, changes in the assumed timing of when milestones are likely to be achieved and changes in assumed discount periods and rates. Accordingly, subsequent changes in the underlying facts and circumstances could result in changes to our estimates and assumptions, which could have a material impact on the estimated future fair values of contingent consideration.

We believe the fair value used to record contingent consideration incurred in connection with business combinations is based on reasonable estimates and assumptions given the facts and circumstances as of the related valuation date.

**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 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. In addition, significant judgment is required in determining our worldwide provision for income taxes. For example, the 2020 income tax benefit included a \$835.1 million deferred tax benefit related to an intra-entity transfer of certain intellectual property rights to an Irish subsidiary. The transaction resulted in a step-up of the Irish tax-deductible basis, and the Irish subsidiary recognized a deferred tax asset for the book and tax basis difference of the transferred intellectual property rights. The tax deductions related to the amortization of these intangible assets will be recognized in the future and any amortization not deducted for tax purposes will be carried forward indefinitely under Irish tax laws. We expect to be able to realize the deferred tax asset resulting from this transaction and have not recorded a valuation allowance as of December 31, 2020. Management is not aware of any potential changes that would have a material effect on our Consolidated Financial Statements.

**Recent Accounting Pronouncements**

See Note 4 to our accompanying Consolidated Financial Statements for a full description of recent accounting pronouncements and our expectation of their impact, if any, on our results of operations and financial condition.

**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
(In millions of U.S. Dollars, except as otherwise disclosed)

**Results of Operations**
**Net Income (Loss)**

Net Income (Loss) consisted of the following:

	Years Ended December 31,			2020 vs. 2019	2019 vs. 2018
	2020	2019	2018		
Total revenues	\$ 1,860.5	\$ 1,704.0	\$ 1,491.2	\$ 156.5	\$ 212.8
Cost of sales	524.3	359.5	315.3	164.8	44.2
R&D expense	628.1	715.0	696.3	(86.9)	18.7
SG&A expense	737.7	680.9	604.4	56.8	76.5
Intangible asset amortization and contingent consideration	66.7	74.1	48.8	(7.4)	25.3
Gain on sale of nonfinancial assets	(59.5)	(25.0)	(50.0)	(34.5)	25.0
Other income (expense), net	(5.5)	5.7	(19.1)	(11.2)	24.8
Benefit from income taxes	(901.4)	(71.0)	(65.5)	(830.4)	(5.5)
<b>Net income (loss)</b>	<b>\$ 859.1</b>	<b>\$ (23.8)</b>	<b>\$ (77.2)</b>	<b>\$ 882.9</b>	<b>\$ 53.4</b>

Our Net Income for the year ended December 31, 2020 was \$859.1 million, compared to Net Loss of \$23.8 million and \$77.2 million for the years ended December 31, 2019 and 2018, respectively. The changes in Net Income (Loss) were primarily a result of the following:

**Net Product Revenues**

Net Product Revenues consisted of the following:

	Years Ended December 31,			2020 vs. 2019	2019 vs. 2018
	2020	2019	2018		
Net product revenues by product:					
Vimizim	\$ 544.4	\$ 544.3	\$ 482.0	\$ 0.1	\$ 62.3
Kuvan	457.7	463.4	433.6	(5.7)	29.8
Naglazyme	391.3	374.3	345.9	17.0	28.4
Palynziq	171.0	86.9	12.2	84.1	74.7
Brineura	110.2	72.0	39.9	38.2	32.1
Firdapse	1.2	\$ 22.3	\$ 21.7	\$ (21.1)	\$ 0.6
Total net product revenues marketed by the Company	\$ 1,675.8	\$ 1,563.2	\$ 1,335.3	\$ 112.6	\$ 227.9
Aldurazyme net product revenues marketed by Sanofi Genzyme	130.1	97.8	135.1	32.3	(37.3)
<b>Total net product revenues</b>	<b>\$ 1,805.9</b>	<b>\$ 1,661.0</b>	<b>\$ 1,470.4</b>	<b>\$ 144.9</b>	<b>\$ 190.6</b>

**2020 compared to 2019**

The increase in Net Product Revenues for the year ended December 31, 2020 as compared to 2019 was primarily attributed to the following:

- Palynziq: the increase was attributed to a combination of revenue from more patients achieving maintenance dosing and new patients initiating therapy in the U.S.;
- Brineura: the increase was primarily attributed to growth in the number of patients in all regions;
- Aldurazyme: the increase was attributed to timing of product fulfillment to Genzyme;

**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
**(In millions of U.S. Dollars, except as otherwise disclosed)**

- Naglazyme: the increase was primarily attributed to increased orders from the Middle East and growth in North America; partially offset by
- Firdapse: the decrease was primarily attributed to the divestiture and sale of the Firdapse business in January 2020; and
- Kuvan: the decrease was primarily attributed to the U.S. loss of market exclusivity in October 2020 resulting in generic competition.

**2019 compared to 2018**

The increase in Net Product Revenues for the year ended December 31, 2019 as compared to 2018 was primarily attributed to the following:

- Palyngiq: the increase was attributed to a combination of revenue from more patients achieving maintenance dosing and new patients initiating therapy in the U.S. as the product launched in the third quarter of 2018;
- Vimizim: the increase was primarily attributed to increased sales volume due to patient growth in the U.S. and orders from Latin America;
- Brineura: the increase was primarily attributed to growth in the number of patients in all regions;
- Kuvan: the increase was primarily attributed to increased sales volume due to patient growth in North America and in European markets;
- Naglazyme: the increase was primarily attributed to increased sales volume driven by orders from Latin America; partially offset by
- Aldurazyme: the decrease was primarily attributed to timing of product fulfillment to Genzyme.

In certain countries, such as in Latin America, governments place large periodic orders for Naglazyme and Vimizim. The timing of these large government orders can be inconsistent and can create significant period to period variation in our revenues.

We anticipate the COVID-19 pandemic will have a continued impact on future Net Product Revenues in 2021 as many of our products are administered via infusions in a clinic or hospital setting and/or by a healthcare professional. Although we are working with our patient community and health care providers to find alternative arrangements where necessary, such as providing infusions at home, the revenue from the doses of our products that are missed by patients and the lost revenue from delayed treatment starts for new patients will never be recouped. See the risk factor "The COVID-19 pandemic could materially adversely affect our business, results of operations and financial condition" in "Risk Factors" included in Part I, Item 1A of this Annual Report on Form 10-K for additional information.

In October 2020, we lost U.S. market exclusivity for Kuvan which adversely affected revenues in the fourth quarter of 2020. We have been preparing for this loss of exclusivity which will continue to result in a reduction in market share and adversely affect our revenue and results of operations in 2021. See the risk factor "The sale of generic versions of Kuvan by generic manufacturers may adversely affect our revenues and results of operations" in "Risk Factors" included in Part I, Item 1A of this Annual Report on Form 10-K for additional information. Additionally, the responses received from the FDA and EMA requesting additional safety and efficacy data from our valoctocogene roxaparvovec Phase 3 studies have extended our anticipated regulatory approval timelines which will have a negative impact on net product revenue growth for 2021.

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

	Years Ended December 31,			2020 vs. 2019	2019 vs. 2018
	2020	2019	2018		
Sales denominated in USD	\$ 1,063.2	\$ 932.6	\$ 891.7	\$ 130.6	\$ 40.9
Sales denominated in foreign currencies	742.7	728.4	578.7	14.3	149.7
Total net product revenues	<u>\$ 1,805.9</u>	<u>\$ 1,661.0</u>	<u>\$ 1,470.4</u>	<u>\$ 144.9</u>	<u>\$ 190.6</u>

The net impact of foreign currency exchange rates on product sales denominated in currencies other than USD during 2020 and 2019 was unfavorable by \$23.3 million and \$24.8 million respectively, which was primarily driven by a weakening, relative to the USD, of currencies in Latin American markets such as Brazil, Colombia and Argentina.

**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
(In millions of U.S. Dollars, except as otherwise disclosed)

See "Quantitative and Qualitative Disclosures about Market Risk" in Part II, Item 7A of this Annual Report on Form 10-K for information on currency exchange rate risk related to our revenues.

**Royalty and Other Revenues**

Royalty revenues include royalties earned on net sales of products sold and milestones achieved by licensees or sublicensees. Other revenues include revenues earned from the sale of licenses of our intellectual property and rental income associated with the tenants in our facilities.

	Years Ended December 31,			2020 vs. 2019	2019 vs. 2018
	2020	2019	2018		
Royalty revenues	\$ 38.6	\$ 39.7	\$ 19.3	\$ (1.1)	\$ 20.4
Other revenues	16.0	3.3	1.6	12.7	1.7
<b>Total Royalty and Other revenues</b>	<b>\$ 54.6</b>	<b>\$ 43.0</b>	<b>\$ 20.9</b>	<b>\$ 11.6</b>	<b>\$ 22.1</b>

The 2020 increase in Royalty and Other Revenues compared to 2019 was primarily due to license revenues earned from the third-party that licensed tralesenidase alfa from us.

The 2019 increase in Royalty and Other Revenues compared to 2018 was primarily due to a one time license payment from a third party due to their achievement of a regulatory milestone and royalty revenues earned from Catalyst Pharmaceutical Partners, Inc. (Catalyst) on net product sales of Firdapse in North America, which was approved for sale in November 2018. See Note 20 to our accompanying Consolidated Financial Statements for additional information regarding our license and collaboration agreements.

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

**Cost of Sales and Product Gross Margin**

Cost of Sales includes raw materials, personnel and 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, and internal and external final formulation and packaging costs. Cost of Sales also includes royalties payable to third parties based on sales of our products and charges for inventory valuation reserves.

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

	Years Ended December 31,			2020 vs. 2019	2019 vs. 2018
	2020	2019	2018		
Total net product revenues	\$ 1,805.9	\$ 1,661.0	\$ 1,470.4	\$ 144.9	\$ 190.6
Cost of sales	\$ 524.3	\$ 359.5	\$ 315.3	\$ 164.8	\$ 44.2
Product gross margin	71.0 %	78.4 %	78.6 %	(7.4)%	(0.2)%

Cost of Sales increased for 2020 compared to 2019 primarily due to \$87.2 million of inventory charges recorded in 2020 related to pre-launch valoctocogene roxaparvec inventory as a result of anticipated regulatory approval delays. Product gross margin for 2020 compared to 2019 decreased primarily due to higher costs of sales and product mix as there were higher sales of lower margin products.

The 2019 increase in Cost of Sales compared to 2018 was primarily attributable to increased sales volumes. Product gross margin was relatively flat in 2019 compared to 2018.

We expect total product gross margin to remain near 75% over the next twelve months.

**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 approved products. R&D expense primarily includes preclinical and clinical studies, personnel



**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
(In millions of U.S. Dollars, except as otherwise disclosed)

and raw materials costs associated with manufacturing clinical product, quality control and assurance, other R&D activities, facilities and regulatory costs.

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, and capitalize the costs incurred related to those activities if it is determined that recoverability is highly likely and therefore future revenues are expected. When regulatory approval and the likelihood of future revenues for a product candidate are less certain, the related manufacturing costs are expensed as R&D expenses. We did not have any pre-launch or pre-qualification manufacturing activities capitalized as of December 31, 2020. See Note 8 to our accompanying Consolidated Financial Statements for additional information regarding our inventory.

R&D expense consisted of the following:

	Years Ended December 31,			2020 vs. 2019	2019 vs. 2018
	2020	2019	2018		
Research and early development	\$ 164.9	\$ 79.4	\$ 68.2	\$ 85.5	\$ 11.2
Vosoritide	130.8	120.9	89.3	9.9	31.6
Valoctocogene roxaparovec	116.2	192.8	161.7	\$ (76.6)	\$ 31.1
Other approved products	83.2	104.1	117.5	(20.9)	(13.4)
BMN 307	72.6	89.2	18.1	(16.6)	71.1
Palynziq	50.5	71.7	94.8	(21.2)	(23.1)
Tralesinidase alfa	1.4	27.4	82.2	(26.0)	(54.8)
Other	8.5	29.5	64.5	(21.0)	(35.0)
<b>Total R&amp;D expense</b>	<b>\$ 628.1</b>	<b>\$ 715.0</b>	<b>\$ 696.3</b>	<b>\$ (86.9)</b>	<b>\$ 18.7</b>

**2020 compared to 2019**

The decrease in R&D expense primarily comprised the following:

- a decrease in costs related to valoctocogene roxaparovec, due primarily to lower clinical activity spend;
- a decrease in tralesinidase alfa costs as the program was licensed to a third-party in the fourth quarter of 2019;
- a decrease in clinical study costs related to Palynziq due to the 2019 completion of the studies supporting European regulatory approval;
- a decrease in expenses related to approved products primarily due to completion of Brineura extension studies;
- a decrease in clinical manufacturing costs related to BMN 307; partially offset by
- an increase in early stage research program spending due primarily to the \$26.3 million payment made for preclinical programs licensed from a third party and costs related to BMN 331 for the treatment of hereditary angioedema.

We expect R&D expense to increase in future periods compared to 2020, primarily due to increased spending on preclinical activities for our research and early development programs while we continue to develop our later stage programs.

**2019 compared to 2018**

The increase in R&D expense primarily comprised the following:

- an increase in preclinical and manufacturing activities related to our BMN 307 development program, including clinical manufacturing costs; and
- an increase in clinical activity related to our late-stage product candidates valoctocogene roxaparovec and vosoritide; partially offset by
- a decrease in tralesinidase alfa clinical manufacturing costs; and

**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
(In millions of U.S. Dollars, except as otherwise disclosed)

- a decrease in costs related to Palynziq, for which capitalization of manufacturing costs began in the second quarter of 2018 following FDA approval.

**Selling, General and Administrative**

Sales and marketing (S&M) expense primarily consisted 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 consisted of corporate support and other administrative expenses, including employee-related expenses.

SG&A expenses consisted of the following:

	Years Ended December 31,			2020 vs. 2019	2019 vs. 2018
	2020	2019	2018		
S&M expense	\$ 403.4	\$ 382.2	\$ 324.2	\$ 21.2	\$ 58.0
G&A expense	334.3	298.7	280.2	35.6	18.5
Total SG&A expense	<u>\$ 737.7</u>	<u>\$ 680.9</u>	<u>\$ 604.4</u>	<u>\$ 56.8</u>	<u>\$ 76.5</u>

S&M expenses by product were as follows:

	Years Ended December 31,			2020 vs. 2019	2019 vs. 2018
	2020	2019	2018		
PKU Products (Kuvan and Palynziq)	\$ 127.5	\$ 137.2	\$ 118.6	\$ (9.7)	\$ 18.6
MPS Products (Aldurazyme, Naglazyme and Vimizim)	108.1	119.2	115.8	(11.1)	3.4
Valoctocogene roxaparvovec	87.3	50.3	17.0	37.0	33.3
Brineura	37.8	45.4	44.2	(7.6)	1.2
Vosoritide	34.5	13.3	7.3	21.2	6.0
Other	8.2	16.8	21.3	(8.6)	(4.5)
Total S&M expense	<u>\$ 403.4</u>	<u>\$ 382.2</u>	<u>\$ 324.2</u>	<u>\$ 21.2</u>	<u>\$ 58.0</u>

**2020 compared to 2019**

The increase in S&M expense was primarily a result of an increase in pre-commercial activities related to valoctocogene roxaparvovec and vosoritide, partially offset by decreases in MPS Products, PKU Products and Brineura.

The increase in G&A expense was primarily due to employee-related expenses due to a higher headcount and the unfavorable impact of foreign exchange revaluation of non-USD denominated assets and liabilities.

We expect SG&A expense to increase in future periods as a result of preparing to launch new products and support of our global business as it grows.

**2019 compared to 2018**

The increase in S&M expense was primarily a result of an increase in pre-commercialization activities related to valoctocogene roxaparvovec and commercialization activities in support of the continued U.S. expansion and EU commercial launches of Palynziq.

The increase in G&A expense was primarily due to consulting fees and higher employee related expenses to support our growth.

**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
(In millions of U.S. Dollars, except as otherwise disclosed)

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

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

	Years Ended December 31,			2020 vs. 2019	2019 vs. 2018
	2020	2019	2018		
Changes in the fair value of contingent consideration (gain) / loss	\$ 4.5	\$ 20.6	\$ 18.5	\$ (16.1)	\$ 2.1
Amortization of intangible assets	62.2	53.5	30.3	8.7	23.2
Total intangible asset amortization and contingent consideration	\$ 66.7	\$ 74.1	\$ 48.8	\$ (7.4)	\$ 25.3
Gain on sale of nonfinancial assets	\$ 59.5	\$ 25.0	\$ 50.0	\$ 34.5	\$ (25.0)

**2020 compared to 2019**

Fair value of contingent consideration: the 2020 increase in the fair value of contingent consideration is lower than the increase in 2019 due primarily to the achievement in 2019 of the Palynziq EU regulatory approval milestone.

Amortization of intangible assets: the increase in 2020 was due to the Palynziq intangible assets that were in service for the entire year compared to seven months in 2019.

Gain on Sale of Nonfinancial Assets: we recognized a gain of \$59.5 million in 2020 due to the divestiture and sale of Firdapse compared to \$25.0 million in 2019 due to the achievement of milestones by third parties, related to previously sold intangible assets. See Note 6 to the accompanying Consolidated Financial Statements for additional information.

**2019 compared to 2018**

Fair value of contingent consideration: the increase in the fair value of the contingent consideration in 2019 was primarily attributable to changes in the estimated probability of achieving development milestones, related to our Palynziq program which received EU marketing approval in May 2019.

Amortization of intangible assets: the increase in 2019 was due to the Palynziq IPR&D assets that were placed into service following EU marketing approval in May 2019.

Gain on Sale of Nonfinancial Assets: we recognized a gain of \$25.0 million in 2019 compared to \$50.0 million in 2018 due to the achievement of milestones by third parties, 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.

	Years Ended December 31,			2020 vs. 2019	2019 vs. 2018
	2020	2019	2018		
Interest income	\$ 16.6	\$ 22.7	\$ 22.8	\$ (6.1)	\$ (0.1)

The decrease in Interest Income during 2020 compared to 2019 was primarily due to lower interest rates. Interest Income during 2019 was flat compared to 2018.

We expect Interest Income to be lower over the next 12 months due to lower interest rates and yields on our cash equivalents and investments.

**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
(In millions of U.S. Dollars, except as otherwise disclosed)

### Interest Expense

We incur Interest Expense primarily on our convertible debt.

	Years Ended December 31,			2020 vs. 2019	2019 vs. 2018
	2020	2019	2018		
Total interest expense	\$ 29.3	\$ 23.5	\$ 43.7	\$ 5.8	\$ (20.2)

The increase in Interest Expense in 2020 compared to 2019 was primarily due to the issuance of the 1.25% senior subordinated convertible notes due in 2027 (the 2027 Notes) in the second quarter of 2020 partially offset by the maturity of our 1.50% senior subordinated convertible notes which matured in October 2020 (the 2020 Notes).

The decrease in Interest Expense in 2019 compared to 2018 was primarily due to the maturity of our 0.75% senior subordinated convertible notes, which matured in October 2018.

We expect Interest Expense to be lower over the next 12 months due to the maturity of the 2020 Notes. See Note 13 to our accompanying Consolidated Financial Statements for additional information regarding our debt.

### Benefit from Income Taxes

We recognized an income tax benefit of \$901.4 million, \$71.0 million and \$65.5 million in 2020, 2019 and 2018, respectively. Benefit from Income Taxes for 2020, 2019 and 2018 consisted of state, federal and foreign current tax expense which was offset by tax benefits related to stock option exercises and deferred tax benefits from federal orphan drug credits, federal R&D credits and California R&D credits. The Benefit from Income Taxes for the years ended December 31, 2020 and 2019 were further impacted by the following items:

- In the third quarter of 2020, we completed an intra-entity transfer of certain intellectual property rights to an Irish subsidiary where our ex-U.S. regional headquarters are located and we have significant manufacturing and commercial operations, to better align ownership of intellectual property rights with how the business operates. The transaction did not result in a taxable gain; however, our Irish subsidiary recognized a deferred tax asset for the book and tax basis difference of the transferred intellectual property rights. As a result, we recognized a deferred tax asset of \$835.1 million and related tax benefit in the third quarter based on the fair value of the transferred intellectual property rights. The tax deductions related to the amortization of these intangible assets will be recognized in the future and any amortization not deducted for tax purposes will be carried forward indefinitely under Irish tax laws. We expect to be able to realize the deferred tax asset resulting from this transaction and have not recorded a valuation allowance as of December 31, 2020.
- 2019 included a tax benefit of \$29.6 million for the release of the valuation allowance against the net deferred tax asset of our Dutch subsidiary. In the second quarter of 2019, we determined that it is more likely than not that the deferred tax assets, including net operating losses and tax credit carryforwards of our Dutch subsidiary, will be realized. In making this determination, we analyzed our recent history of earnings, forecasts of future earnings and cumulative earnings for the last three years of our Dutch subsidiary and we reached the conclusion that it was appropriate to release the valuation allowance reserve.
- On March 27, 2020, The Coronavirus Aid, Relief and Economic Security Act (CARES Act) was signed into law lifting certain limitations originally imposed by the Tax Cuts and Jobs Act of 2017, including the calculation of certain deductions and treatment of net operating losses. On June 29, 2020, California enacted legislative changes (California AB 85) which suspended California net operating loss utilization for years 2020 through 2022. The enactment of both the CARES Act and California AB 85 did not result in any material adjustments to our income tax provision for the year ended December 31, 2020, or to our net deferred tax assets as of December 31, 2020.

### Financial Position, Liquidity and Capital Resources

As of December 31, 2020, we had \$1.4 billion in cash, cash equivalents, and investments which includes net proceeds of \$535.8 million from our May 2020 convertible debt offering, net of the repurchase of our common stock. We believe our net product revenues from our commercial products, cash, cash equivalents and investments, potentially supplemented by proceeds from equity or debt financings and loans, or collaborative agreements with corporate partners will be sufficient to satisfy our liquidity requirements for the next 12 months. We may require additional financing to fund the repayment of our convertible debt, future milestone payments and our future operations, including the commercialization of our products and product candidates currently

**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
(In millions of U.S. Dollars, except as otherwise disclosed)

under development, preclinical studies and clinical trials, and potential licenses and acquisitions. 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 timing and mix of our funding options 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 elect to settle all or a portion of our convertible debt in cash. Our ability to raise additional capital may also be adversely impacted by potential worsening global economic conditions and the recent disruptions to, and volatility in, financial markets in the U.S. and worldwide resulting from the ongoing COVID-19 pandemic.

In managing our liquidity needs in the U.S., we do not rely on unrepatriated earnings as a source of funds. As of December 31, 2020, \$248.2 million of our \$1.4 billion balance of cash, cash equivalents and investments was held in non-U.S. subsidiaries, a significant portion of which is required to fund the liquidity needs of these non-U.S. subsidiaries. See Note 18 to our accompanying Consolidated Financial Statements for additional discussion regarding income taxes.

We are mindful that conditions in the current macroeconomic environment could affect our ability to achieve our goals. We sell our products in 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 and adverse effects of the impact of the ongoing COVID-19 pandemic 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 liquidity and capital resources as of December 31, 2020, 2019 and 2018 were as follows:

	2020	2019	2018	2020 vs. 2019	2019 vs. 2018
Cash and cash equivalents	\$ 649.2	\$ 437.4	\$ 494.0	\$ 211.7	\$ (56.6)
Short-term investments	416.2	316.4	590.3	99.9	(273.9)
Long-term investments	285.5	412.0	235.9	(126.5)	176.1
Cash, cash equivalents and investments	<u>\$ 1,350.9</u>	<u>\$ 1,165.8</u>	<u>\$ 1,320.2</u>	<u>\$ 185.1</u>	<u>\$ (154.4)</u>
Total convertible debt, net	\$ 1,075.1	\$ 848.1	\$ 830.4	\$ 227.0	\$ 17.7

Our cash flows for each of the years ended December 31, 2020, 2019 and 2018 are summarized as follows:

	2020	2019	2018	2020 vs. 2019	2019 vs. 2018
Cash & cash equivalents at the beginning of the period	\$ 437.4	\$ 494.0	\$ 598.0	\$ (56.6)	\$ (104.0)
Net cash provided by operating activities	85.4	48.3	20.2	37.1	28.1
Net cash provided by (used in) investing activities	(53.6)	(31.0)	264.4	(22.6)	(295.4)
Net cash provided by (used in) financing activities	181.1	(74.7)	(388.0)	255.8	313.3
Foreign exchange impact	(1.1)	0.8	(0.6)	(1.9)	1.4
Cash & cash equivalents at the end of the period	<u>\$ 649.2</u>	<u>\$ 437.4</u>	<u>\$ 494.0</u>	<u>\$ 211.8</u>	<u>\$ (56.6)</u>
Short-term and long-term investments	<u>701.7</u>	<u>728.4</u>	<u>826.2</u>	<u>(26.7)</u>	<u>(97.8)</u>
Cash, cash equivalents and investments	<u>\$ 1,350.9</u>	<u>\$ 1,165.8</u>	<u>\$ 1,320.2</u>	<u>\$ 185.1</u>	<u>\$ (154.4)</u>

**Cash Provided by Operating Activities**

Net cash provided by operating activities during 2020 was \$85.4 million, compared to net cash provided by operating activities of \$48.3 million during 2019. The change in net cash provided by operating activities in 2020 compared to 2019 was due

**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
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to increased cash receipts from our customers, licensees and sublicensees, due in part to higher sales. The increase was partially offset by timing of payments made to vendors and due to increased payments for inventory purchases.

Net cash provided by operating activities during 2019 was \$48.3 million, compared to net cash provided by operating activities of \$20.2 million during 2018. The change in net cash provided by operating activities in 2019 compared to 2018 was primarily attributed to the timing of payments to vendors and cash receipts from customers.

***Cash Provided by (Used in) Investing Activities***

Net cash used in investing activities during 2020 was \$53.6 million, compared to net cash used in investing activities of \$31.0 million during 2019. The change in net cash used in investing activities in 2020 compared to 2019 was primarily attributable to a decrease of \$82.0 million in proceeds from net maturities and sales of investments partially offset by an increase in proceeds from the sale of nonfinancial assets and lower purchases of property, plant and equipment.

Net cash used in investing activities during 2019 was \$31.0 million, compared to net cash provided by investing activities of \$264.4 million during 2018. The change in net cash used in investing activities in 2019 compared to 2018 was primarily due to a decrease of \$250.8 million in proceeds from net maturities and sales of investments, lower cash receipts from milestone payments related to previously sold intangible assets and an increase in cash used in the purchase of intangible assets.

***Cash Provided by (Used in) Financing Activities***

Net cash provided by financing activities during 2020 was \$181.1 million, compared to net cash used in financing activities of \$74.7 million during 2019. The increase in net cash provided by financing activities in 2020 compared to 2019 was primarily attributed to the net proceeds from the issuance of the 2027 Notes and exercises of awards under our equity incentive plans, partially offset by the cash settlement of the 2020 Notes and the repurchase of our common stock.

Net cash used in financing activities during 2019 was \$74.7 million, compared to net cash used in financing activities of \$388.0 million during 2018. The decrease in net cash used in financing activities in 2019 compared to 2018 was primarily due to the settlement, in 2018, of the 2018 Notes which did not recur in 2019.

***Other Information***

Our \$1.1 billion (undiscounted) of total convertible debt as of December 31, 2020 will impact our liquidity due to the semi-annual cash interest payments. As of December 31, 2020, our indebtedness consisted of the 2027 Notes and our 0.599% senior subordinated convertible notes due in 2024 (the 2024 Notes and together with the 2027 Notes, the Notes), which, if not converted, will be required to be repaid in cash at maturity in August 2024 and May 2027, respectively. We will need cash not only to pay the ongoing interest due on the Notes during their term, but also to repay the principle amount of the Notes if not converted.

Our 2020 Notes matured on October 15, 2020 and were settled in cash for approximately \$375.0 million. No shares were issued in connection with the settlement as the Company's share price did not exceed the conversion price of \$94.15, as measured over a 25-day averaging period, and the capped call transaction entered into concurrently with the issuance of the 2020 Notes was not triggered. The Company incurred no gain or loss upon the extinguishment of the 2020 Notes. See Note 13 to our accompanying Consolidated Financial Statements for additional discussion on our outstanding debt.

In October 2018, we entered into an unsecured revolving credit facility of up to \$200.0 million (the 2018 Credit Facility). The 2018 Credit Facility includes a letter of credit subfacility and a swingline loan subfacility and is also intended to finance ongoing working capital needs and for other general corporate purposes. The 2018 Credit Facility matures on October 19, 2021 at which time all outstanding amounts become due and payable. The 2018 Credit Facility contains financial covenants requiring us to maintain a minimum interest coverage ratio and a minimum liquidity requirement. As of December 31, 2020, there were no outstanding amounts due on nor any usage of the 2018 Credit Facility and we were in compliance with all covenants. See Note 13 to our accompanying Consolidated Financial Statements for additional discussion.

***Funding Commitments***

We cannot estimate with certainty the cost to complete any of our product development programs. Additionally, except as disclosed under "Overview" above, we cannot precisely estimate the time to complete any of our product development programs or when we expect to receive net cash inflows from any of our product development programs. Please see "Risk Factors" included in

**Management's Discussion and Analysis of Financial Condition and Results of Operations (continued)**  
**(In millions of U.S. Dollars, except as otherwise disclosed)**

Part I, Item 1A of this Annual Report on Form 10-K, for a discussion of the reasons we are unable to estimate such information, and in particular the following risk factors:

*Business and Operational Risks:*

- *The COVID-19 pandemic could materially affect our business, results of operations, and financial condition;*
- *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 revenue could be adversely affected.*
- *If we do not achieve our projected development goals in the timeframes we announce and expect, the commercialization of our product candidates may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.*

*Regulatory Risks:*

- *If we fail to obtain regulatory approval to commercially market and sell our product candidates, or if approval of our product candidates is delayed, we will be unable to generate revenue 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;*

*Manufacturing Risks:*

- *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;*

Our investment in our product development programs and continued development of our existing commercial products has a major impact on our operating performance. Our R&D expenses for the period since inception as of December 31, 2020 for certain of our key programs were as follows:

	<b>Since Program Inception</b>
Palyzinq	\$ 739.9
Valoctocogene roxaparvovec	\$ 710.0
Vosoritide	\$ 570.7
BMN 307	\$ 179.9
Other approved products	\$ 1,524.8

We may need or elect to increase our spending above our current long-term plans to be able to achieve our long-term goals. This may increase our capital requirements, including: costs associated with the commercialization of our products; additional clinical trials; investments in the manufacturing of our commercial products; preclinical studies and clinical trials for our product candidates; potential licenses and other acquisitions of complementary technologies, products and companies; and general corporate purposes.

Our future capital requirements will depend on many factors, including, but not limited to:

- our ability to successfully market and sell our products;
- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities;
- the progress and success of our preclinical studies and clinical trials (including the manufacture of materials for use in such studies and trials);
- the timing, number, size and scope of our preclinical studies and clinical trials;
- the time and cost necessary to obtain regulatory approvals and the costs of post-marketing studies which may be required by regulatory authorities; and
- the progress of research programs carried out by us.



### Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements that are currently material or reasonably likely to be material to our consolidated financial position or results of operations.

### Contractual and Commercial Obligations

We have contractual and commercial obligations under our convertible debt, operating leases and other obligations related to R&D activities, purchase commitments, licenses and sales royalties with annual minimums. Our contractual obligations as of December 31, 2020 are presented in the table below.

	Payments Due within				Total
	1 Year or Less	>1 -3 Years	> 3 - 5 Years	More Than 5 Years	
2024 Notes and related interest	\$ 3.0	\$ 5.9	\$ 498.0	\$ —	\$ 506.9
2027 Notes and related interest	7.5	15.0	15.0	611.3	648.8
Leases	14.4	22.9	12.2	16.8	66.3
R&D and purchase commitments	139.6	2.2	1.0	—	142.8
Total	\$ 164.5	\$ 46.0	\$ 526.2	\$ 628.1	\$ 1,364.8

As of December 31, 2020, we were also subject to contingent payments totaling approximately \$662.8 million upon achievement of certain development and regulatory activities and commercial sales milestones if they occur before certain dates in the future. Of this amount, \$235.0 million related to an early stage development programs licensed from a third party in the second quarter of 2020, \$73.6 million related to the acquisition of certain rights and other assets with respect to Kuvan and Palynziq from Merck Serono and \$237.7 million related to programs that are no longer being developed.

As of December 31, 2020, we have recorded \$60.1 million of contingent consideration on our Consolidated Balance Sheet, all of which was long-term. See Note 21 to our accompanying Consolidated Financial Statements for additional discussion on our commitments.

In addition, our liability for unrecognized tax benefits has been excluded from the above contractual and commercial obligations table as the nature and timing of future payments, if any, cannot be reasonably estimated. As of December 31, 2020, our liability for unrecognized tax benefits was \$182.6 million. See Note 18 to our accompanying Consolidated Financial Statements for additional discussion on our income taxes.

## 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 and Asia Pacific. As a result, our financial results may be significantly affected by factors such as the impact of the COVID-19 pandemic in these regions, 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 made in the respective foreign currency decreases. Conversely, when the USD weakens against these currencies, the relative value of such sales 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 2020, approximately 41% of our net product sales were denominated in foreign currencies and 21% 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 may enter into forward foreign currency exchange 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, 2020, we had open forward contracts with net notional amounts of \$678.9 million. A hypothetical 10% adverse movement in foreign currency exchange rates compared with the USD relative to exchange rates as of December 31, 2020 would have resulted in a reduction in the value received over the remaining life of these contracts by approximately \$70.9 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, 2020, 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 \$9.1 million. We expect to continue to enter into transactions based in foreign currencies that could be impacted by changes in exchange rates.

### **Interest Rate Market Risk**

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

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

We have outstanding \$495.0 million (undiscounted) of the 2024 Notes and \$600.0 million (undiscounted) of the 2027 Notes. The interest rates on these notes are fixed and therefore they do not expose us to risk related to rising interest rates. As of December 31, 2020, the fair value of our convertible debt was \$1.2 billion.

As of December 31, 2020, our investment portfolio did not include any investments with significant exposure to countries that face economic volatility and weakness. Although not predictive in nature, we believe a hypothetical 100 basis point threshold reflects a reasonably possible near-term change in interest rates. Based on our investment portfolio and interest rates at December 31, 2020, period ending, we believe that a 100 basis point increase in interest rates could result in a potential loss in fair

value of our investment portfolio of approximately \$5.9 million. Changes in interest rates may affect the fair value of our investment portfolio. However, we will not recognize such gains or losses in our Consolidated Statements of Operations unless the investments are sold or we determine that the declines in the investment's fair values below the cost basis are a result of a credit loss, which, if any, are reported in Other Income, 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, 2020 (in millions):

	Expected Maturity						Total
	2021	2022	2023	2024	2025	Thereafter	
Available-for-sale debt securities	\$ 430.3	\$ 169.5	\$ 105.8	\$ 9.9	\$ —	\$ 0.2	\$ 715.7
Average interest rate	0.3 %	0.3 %	0.3 %	0.4 %	— %	5.4 %	0.3 %

#### **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, 2020.

##### ***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, 2020. 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, 2020 was effective at the reasonable assurance level.

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

***Changes in Internal Control Over Financial Reporting***

There were no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act, during our most recently completed quarter that have materially affected or are reasonably likely to materially affect our internal control over financial reporting. We continue to utilize the Committee of Sponsoring Organizations of the Treadway Commission (COSO) 2013 Framework on internal control. We believe that our ability to maintain an effective internal control environment has not been impacted by the COVID-19 pandemic. We are continually monitoring and assessing the COVID-19 situation on our internal controls to minimize the impact on their design and operating effectiveness.

***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**

None.

### Part III

#### **Item 10. Directors, Executive Officers and Corporate Governance**

The information required by this Item regarding our directors, executive officers and corporate governance is incorporated into this section by reference to the sections captioned “Election of Directors” and “Executive Officers” in the proxy statement for our 2021 annual meeting of stockholders.

#### **Item 11. Executive Compensation**

The information required by this Item regarding executive compensation is incorporated into this section by reference to the section captioned “Executive Compensation” in the proxy statement for our 2021 annual meeting of stockholders.

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

The information required by this Item regarding security ownership of our beneficial owners, management and related stockholder matters is incorporated into this section by reference to the section captioned “Security Ownership of Certain Beneficial Owners and Management” in the proxy statement for our 2021 annual meeting of stockholders.

The information required by this Item regarding the securities authorized for issuance under our equity compensation plans is incorporated into this section by reference to the section captioned “Equity Compensation Plan Information” in the proxy statement for our 2021 annual meeting of stockholders.

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

The information required by this Item regarding certain relationships, related transactions and director independence is incorporated into this section by reference to the sections captioned “Other Board Governance Information — Transactions with Related Persons, Promoters and Certain Control Persons,” “Other Board Governance Information — Review, Approval and Ratification of Transactions with Related Parties” and “Director Independence” in the proxy statement for our 2021 annual meeting of stockholders.

#### **Item 14. Principal Accountant Fees and Services**

The information required by this Item regarding our principal accountant fees and services is incorporated into this section by reference to the section captioned “Independent Registered Public Accounting Firm” in the proxy statement for our 2021 annual meeting of stockholders.

Part IV

Item 15. Exhibits, Financial Statement Schedules

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<b>Exhibit Number</b>	<b>Description</b>
2.1	<a href="#">Amended and Restated Termination and Transition Agreement, dated as of December 23, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on January 7, 2016 as Exhibit 2.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC.</a>
2.2	<a href="#">Termination and Transition Agreement, dated as of October 1, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on January 7, 2016 as Exhibit 2.3 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC.</a>
2.3	<a href="#">First Amendment, dated as of December 12, 2016, to the Amended and Restated Termination and Transition Agreement, dated as of December 23, 2015 and effective as of October 1, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on February 27, 2017 as Exhibit 2.6 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC.</a>
2.4	<a href="#">Asset Purchase Agreement by and between Novartis Pharma AG, BioMarin Pharmaceutical Inc. and BioMarin Commercial Ltd., dated as of November 21, 2017, previously filed with the SEC on February 26, 2018 as Exhibit 10.47 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated by reference herein.</a>
3.1	<a href="#">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.</a>
3.2	<a href="#">Amended and Restated Bylaws of BioMarin Pharmaceutical Inc., previously filed with the SEC on December 18, 2020 as Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.</a>
4.1	<a href="#">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.</a>
4.2	<a href="#">First Supplemental Indenture, dated August 11, 2017, between the Company and Wilmington Trust, National Association, as Trustee (including the form of 0.599% Senior Subordinated Convertible Note due 2024), previously filed with the SEC on August 11, 2017 as Exhibit 4.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.</a>
4.3	<a href="#">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.</a>
4.4	<a href="#">Description of Capital Stock</a>
10.1†	<a href="#">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.</a>
10.2†	<a href="#">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.</a>
10.3†	<a href="#">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.</a>
10.4†	<a href="#">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.</a>
10.5†	<a href="#">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.</a>



10.6†	<a href="#">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.</a>
10.7†	<a href="#">Amended and Restated Employment Agreement with Jean-Jacques Bienaimé effective December 13, 2016 previously filed with the SEC on December 19, 2016 as Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.</a>
10.8	<a href="#">License Agreement dated July 30, 2004, between BioMarin Pharmaceutical Inc. and Daiichi Suntory Pharma Co., Ltd., as amended by Amendment No. 1 to License Agreement dated November 19, 2004, previously filed with the SEC on March 16, 2005 as Exhibit 10.25 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.</a>
10.9	<a href="#">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.</a>
10.10	<a href="#">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.</a>
10.11	<a href="#">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.</a>
10.12	<a href="#">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.</a>
10.13†	<a href="#">BioMarin Pharmaceutical Inc. 2012 Inducement Plan, adopted May 8, 2012, previously filed with the SEC on May 9, 2012 as Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.</a>
10.14†	<a href="#">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.</a>
10.15†	<a href="#">Form of Stock Options Agreement for the BioMarin Pharmaceutical Inc. 2012 Inducement Plan, previously filed with the SEC on August 2, 2012 as Exhibit 10.13 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference.</a>
10.16	<a href="#">Contract of Purchase and Sale and Joint Escrow Instructions, dated December 17, 2013, for the San Rafael Corporate Center, by and among BioMarin Pharmaceutical Inc., through its wholly-owned subsidiary, California Corporate Center Acquisition, LLC, SR Corporate Center Phase One, LLC, and SR Corporate Center Phase Two, previously filed with the SEC on February 26, 2014 as Exhibit 10.68 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference.</a>
10.17†	<a href="#">BioMarin Pharmaceutical Inc. 2014 Inducement Plan, adopted December 17, 2014, previously filed with the SEC on December 23, 2014 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.</a>
10.18†	<a href="#">Form of Stock Options Agreement for the BioMarin Pharmaceutical Inc. 2014 Inducement Plan, previously filed with the SEC on March 2, 2015 as Exhibit 10.60 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference.</a>
10.19†	<a href="#">Form of Agreement Regarding Restricted Share Units for the BioMarin Pharmaceutical Inc. 2014 Inducement Plan, previously filed with the SEC on March 2, 2015 as Exhibit 10.61 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference.</a>
10.20†	<a href="#">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.</a>
10.21	<a href="#">Settlement and License Agreement among BioMarin Pharmaceutical Inc., Merck &amp; Cie, Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd., dated September 14, 2015, previously filed with the SEC on November 2, 2015 as Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.</a>

10.22	<a href="#">Settlement and License Agreement among BioMarin Pharmaceutical Inc., Merck &amp; Cie and Par Pharmaceutical, Inc., dated as of April 12, 2017, previously filed with the SEC on November 13, 2017 as Exhibit 10.1 to the Company's Amendment No. 1 to Quarterly Report on Form 10-Q/A (File No. 000-26727), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.</a>
10.23†	<a href="#">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.</a>
10.24†	<a href="#">BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, as adopted April 10, 2017 and amended April 12, 2019, previously filed with the SEC on August 2, 2019 as Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference.</a>
10.25†	<a href="#">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.</a>
10.26†	<a href="#">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.</a>
10.27†	<a href="#">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.</a>
10.28†	<a href="#">BioMarin Pharmaceutical Inc. Summary of Independent Director Compensation, previously filed with the SEC on October 31, 2017 as Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference.</a>
10.29†	<a href="#">BioMarin Pharmaceutical Inc. Summary of Independent Director Compensation, previously filed with the SEC on November 4, 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.</a>
10.30	<a href="#">Credit Agreement by and among BioMarin Pharmaceutical Inc., as the Borrower, Bank of America, N.A., as Administrative Agent, Swing Line Lender and a Lender, and Citibank N.A. as L/C Issuer, and the Lenders party thereto, dated as of October 19, 2018, previously filed with the SEC on February 28, 2019 as Exhibit 10.48 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated by reference herein.</a>
10.31	<a href="#">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.</a>
10.32*†	<a href="#">Part-Time Employment Agreement with Robert A. Baffi, Ph.D. effective January 1, 2021.</a>
21.1*	<a href="#">Subsidiaries of BioMarin Pharmaceutical Inc.</a>
23.1*	<a href="#">Consent of KPMG LLP, Independent Registered Public Accounting Firm for BioMarin Pharmaceutical Inc.</a>
24.1*	<a href="#">Power of Attorney (Included in Signature Page to this Report)</a>
31.1*	<a href="#">Certification of Chief Executive Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.</a>
31.2*	<a href="#">Certification of Chief Financial Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.</a>
32.1*	<a href="#">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.</a>
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 Quarterly Report on Form 10-K for the year ended December 31, 2020, 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, 2020 and December 31, 2019, (ii) Consolidated Statements of Operations for the years ended December 31, 2020, 2019 and 2018, (iii) Consolidated Statements of Comprehensive Income (Loss) for the years ended December 31, 2020, 2019 and 2018, (iv) Consolidated Statements of Stockholders' Equity for the years ended December 31, 2020, 2019 and 2018, (v) Consolidated Statements of Cash Flows for the years ended December 31, 2020, 2019 and 2018, 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, 2021

By: \_\_\_\_\_ /S/ BRIAN R. MUELLER  
**Brian R. Mueller**  
**Executive Vice President, Chief Financial Officer**

**POWER OF ATTORNEY**

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Jean-Jacques Bienaimé 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 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:

<b>Signature</b>	<b>Title</b>	<b>Date</b>
<hr/> <i>/S/ JEAN-JACQUES BIENAIMÉ</i> <b>Jean-Jacques Bienaimé</b>	Chairman and Chief Executive Officer (Principal Executive Officer)	February 26, 2021
<hr/> <i>/S/ BRIAN R. MUELLER</i> <b>Brian R. Mueller</b>	Executive Vice President, Chief Financial Officer (Principal Financial Officer)	February 26, 2021
<hr/> <i>/S/ ANDREA L. ACOSTA</i> <b>Andrea L. Acosta</b>	Group Vice President, Chief Accounting Officer (Principal Accounting Officer)	February 26, 2021
<hr/> <i>/S/ ELIZABETH MCKEE ANDERSON</i> <b>Elizabeth McKee Anderson</b>	Director	February 26, 2021
<hr/> <i>/S/ WILLARD H. DERE, M.D.</i> <b>Willard H. Dere, M.D.</b>	Director	February 26, 2021
<hr/> <i>/S/ MICHAEL G. GREY</i> <b>Michael G. Grey</b>	Director	February 26, 2021
<hr/> <i>/S/ ELAINE J. HERON</i> <b>Elaine J. Heron</b>	Director	February 26, 2021
<hr/> <i>/S/ ROBERT J. HOMBACH</i> <b>Robert J. Hombach</b>	Director	February 26, 2021
<hr/> <i>/S/ V. BRYAN LAWLIS</i> <b>V. Bryan Lawlis</b>	Director	February 26, 2021
<hr/> <i>/S/ RICHARD A. MEIER</i> <b>Richard A. Meier</b>	Lead Independent Director	February 26, 2021
<hr/> <i>/S/ DAVID PYOTT</i> <b>David Pyott</b>	Director	February 26, 2021
<hr/> <i>/S/ DENNIS J. SLAMON</i> <b>Dennis J. Slamon</b>	Director	February 26, 2021

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## Report of Independent Registered Public Accounting Firm

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

### *Opinion on the Consolidated Financial Statements*

We have audited the accompanying consolidated balance sheets of BioMarin Pharmaceutical Inc. and subsidiaries (the Company) as of December 31, 2020 and December 31, 2019, the related consolidated statements of operations, comprehensive income (loss), stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2020, and the related notes (collectively, the consolidated financial statements). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2020 and December 31, 2019, and the results of its operations and its cash flows for each of the years in the three-year period ended December 31, 2020, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2020, based on criteria established in *Internal Control – Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission, and our report dated February 26, 2021 expressed an unqualified opinion on the effectiveness of the Company's internal control over financial reporting.

### *Change in Accounting Principle*

As discussed in Note 3 to the consolidated financial statements, the Company has changed its method of accounting for Leases as of January 1, 2019 due to the adoption of Accounting Standards Codification 842 (ASC 842), *Leases*.

### *Basis for Opinion*

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

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. Our audits 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. We believe that our audits provide a reasonable basis for our opinion.

### *Critical Audit Matters*

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

### *Evaluation of variable consideration relating to Aldurazyme product sales*

As described in Notes 3 and 15 to the consolidated financial statements, during the year ended December 31, 2020 the Company recognized \$130 million in Aldurazyme net product revenue. Under its arrangement with Genzyme Corporation (Genzyme), a wholly owned subsidiary of Sanofi, the Company receives payments ranging from 39.5% to 50% on worldwide net Aldurazyme sales by Genzyme, depending on Genzyme's sales volume. The Company estimates this variable consideration based on the amount that it expects to be entitled to from Genzyme's sales of Aldurazyme. The Company recognizes this revenue upon satisfying the product performance obligation, which is when the product is shipped to Genzyme 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 Genzyme sales volume and average price per vial involved a high degree of



**Report of Independent Registered Public Accounting Firm**

subjective auditor judgment due to the nature of available supporting evidence being limited to historical sales and price data related to these assumptions. 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 Genzyme'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 sales volume and price per vial of product sold by Genzyme. We also compared the Company's current-period forecasts of future Genzyme sales volume and average price per vial to Genzyme's historical sales volume and price per vial. We performed a sensitivity analysis to assess the impact of changes in these key assumptions on the Company's estimate of variable consideration.

*Evaluation of the fair value of the underlying intangible assets utilized to recognize a deferred tax asset*

As discussed in Note 18 to the consolidated financial statements, the Company completed an intra-entity transfer of certain intellectual property rights to an Irish subsidiary during the year ended December 31, 2020, resulting in the recognition of a deferred tax asset and a related income tax benefit of \$835.1 million.

We identified the evaluation of the fair value of the underlying intangible assets utilized to recognize a deferred tax asset as a critical audit matter. A high degree of auditor judgment was required to evaluate certain assumptions utilized by the Company in estimating the fair value of the underlying intangible assets. These assumptions included product-specific projected revenues and the inclusion or exclusion of a terminal value for each product.

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 over the Company's process to estimate the fair value of the underlying intangible assets. This included controls over the development of product-specific projected revenues and the inclusion or exclusion of terminal value for each product. We compared the product-specific projected revenue data to historical revenue data and compared the estimated fair value of each intangible asset to industry reports. We involved professionals with specialized skills and knowledge who assisted in evaluating the Company's inclusion or exclusion of terminal value for each product by assessing the likelihood and potential magnitude of future competitor products.

/s/ KPMG LLP

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

San Francisco, California  
February 26, 2021

## Report of Independent Registered Public Accounting Firm

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

### *Opinion on Internal Control Over Financial Reporting*

We have audited BioMarin Pharmaceutical Inc. and subsidiaries' (the Company) internal control over financial reporting as of December 31, 2020, 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 Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2020, based on criteria established in *Internal Control – Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the consolidated balance sheets of the Company as of December 31, 2020 and December 31, 2019, the related consolidated statements of operations, comprehensive income (loss), stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2020, and the related notes (collectively, the consolidated financial statements), and our report dated February 26, 2021 expressed an unqualified opinion on those consolidated financial statements.

### *Basis for Opinion*

The Company's management is responsible 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 internal control over financial reporting based on our audit. We are a public accounting firm registered with the 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 audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. 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 audit also included performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

### *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.

/s/ KPMG LLP

San Francisco, California  
February 26, 2021

**BIOMARIN PHARMACEUTICAL INC.**  
**CONSOLIDATED BALANCE SHEETS**  
December 31, 2020 and 2019

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

	December 31, 2020	December 31, 2019
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 649,158	\$ 437,446
Short-term investments	416,228	316,361
Accounts receivable, net	448,351	377,404
Inventory	698,548	680,275
Other current assets	129,934	130,657
<b>Total current assets</b>	<b>2,342,219</b>	<b>1,942,143</b>
Noncurrent assets:		
Long-term investments	285,473	411,978
Property, plant and equipment, net	1,032,471	1,010,868
Intangible assets, net	417,271	456,580
Goodwill	196,199	197,039
Deferred tax assets	1,432,150	549,422
Other assets	142,237	122,009
<b>Total assets</b>	<b>\$ 5,848,020</b>	<b>\$ 4,690,039</b>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable and accrued liabilities	\$ 492,548	\$ 570,621
Short-term convertible debt, net	—	361,882
<b>Total current liabilities</b>	<b>492,548</b>	<b>932,503</b>
Noncurrent liabilities:		
Long-term convertible debt, net	1,075,145	486,238
Long-term contingent consideration	60,130	50,793
Other long-term liabilities	114,195	98,124
<b>Total liabilities</b>	<b>1,742,018</b>	<b>1,567,658</b>
Stockholders' equity:		
Common stock, \$0.001 par value: 500,000,000 shares authorized; 181,740,999 and 179,838,114 shares issued and outstanding, respectively	182	180
Additional paid-in capital	4,993,407	4,832,707
Company common stock held by Nonqualified Deferred Compensation Plan (the NQDC)	(9,839)	(9,961)
Accumulated other comprehensive income (loss)	(16,139)	20,164
Accumulated deficit	(861,609)	(1,720,709)
<b>Total stockholders' equity</b>	<b>4,106,002</b>	<b>3,122,381</b>
<b>Total liabilities and stockholders' equity</b>	<b>\$ 5,848,020</b>	<b>\$ 4,690,039</b>

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

**BIOMARIN PHARMACEUTICAL INC.**  
**CONSOLIDATED STATEMENTS OF OPERATIONS**  
**Years Ended December 31, 2020, 2019 and 2018**  
(In thousands of U.S. Dollars, except per share amounts)

	2020	2019	2018
<b>REVENUES:</b>			
Net product revenues	\$ 1,805,861	\$ 1,661,043	\$ 1,470,356
Royalty and other revenues	54,594	43,005	20,856
Total revenues	<u>1,860,455</u>	<u>1,704,048</u>	<u>1,491,212</u>
<b>OPERATING EXPENSES:</b>			
Cost of sales	524,272	359,466	315,264
Research and development	628,116	715,007	696,328
Selling, general and administrative	737,669	680,924	604,353
Intangible asset amortization and contingent consideration	66,658	74,108	48,791
Gain on sale of nonfinancial assets	(59,495)	(25,000)	(50,000)
Total operating expenses	<u>1,897,220</u>	<u>1,804,505</u>	<u>1,614,736</u>
<b>LOSS FROM OPERATIONS</b>	<u>(36,765)</u>	<u>(100,457)</u>	<u>(123,524)</u>
Equity in the loss of BioMarin/Genzyme LLC	(6)	(587)	(553)
Interest income	16,610	22,748	22,831
Interest expense	(29,309)	(23,460)	(43,664)
Other income, net	7,148	6,945	2,205
<b>LOSS BEFORE INCOME TAXES</b>	<u>(42,322)</u>	<u>(94,811)</u>	<u>(142,705)</u>
Benefit from income taxes	(901,422)	(70,963)	(65,494)
<b>NET INCOME (LOSS)</b>	<u>\$ 859,100</u>	<u>\$ (23,848)</u>	<u>\$ (77,211)</u>
<b>NET INCOME (LOSS) PER SHARE, BASIC</b>	<u>\$ 4.75</u>	<u>\$ (0.13)</u>	<u>\$ (0.44)</u>
<b>NET INCOME (LOSS) PER SHARE, DILUTED</b>	<u>\$ 4.53</u>	<u>\$ (0.13)</u>	<u>\$ (0.44)</u>
Weighted average common shares outstanding, basic	<u>180,804</u>	<u>179,039</u>	<u>177,061</u>
Weighted average common shares outstanding, diluted	<u>191,678</u>	<u>179,039</u>	<u>177,268</u>

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

**BIOMARIN PHARMACEUTICAL INC.**  
**CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME (LOSS)**  
**Years Ended December 31, 2020, 2019 and 2018**  
**(In thousands of U.S. Dollars)**

	2020	2019	2018
<b>NET INCOME (LOSS)</b>	\$ 859,100	\$ (23,848)	\$ (77,211)
<b>OTHER COMPREHENSIVE INCOME (LOSS):</b>			
Available-for-sale debt securities:			
Unrealized holding gain (loss) arising during the period, net of tax impact of \$(227), \$(1,640) and \$(413), respectively.	749	5,482	1,391
Less: reclassifications to net income (loss), net of tax impact of \$(127), \$0 and \$0, respectively.	425	—	—
Net change in unrealized holding gain (loss), net of tax	324	5,482	1,391
Cash flow hedges:			
Unrealized holding gain (loss) arising during the period, net of tax impact of \$0 for all periods presented.	(23,462)	25,266	25,386
Less: reclassifications to net income (loss), net of tax impact of \$0 for all periods presented.	13,180	15,853	(2,047)
Net change in unrealized holding gain (loss), net of tax	(36,642)	9,413	27,433
Other	15	(2)	(6)
<b>OTHER COMPREHENSIVE INCOME (LOSS), NET OF TAX</b>	<b>(36,303)</b>	<b>14,893</b>	<b>28,818</b>
<b>COMPREHENSIVE INCOME (LOSS)</b>	<b>\$ 822,797</b>	<b>\$ (8,955)</b>	<b>\$ (48,393)</b>

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

**BIOMARIN PHARMACEUTICAL INC.**  
**CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY**  
**Years Ended December 31, 2020, 2019 and 2018**  
(In thousands of U.S. Dollars and share amounts in thousands)

	2020	2019	2018
Shares of Common Stock, beginning balances	179,838	178,253	175,844
Issuances under equity incentive plans	2,421	1,585	2,314
Repurchase of common stock	(518)	—	—
Conversion of convertible notes, net	—	—	95
Shares of Common Stock, ending balances	<u>181,741</u>	<u>179,838</u>	<u>178,253</u>
Total stockholders' equity, beginning balances	\$ 3,122,381	\$ 2,967,940	\$ 2,808,663
Common stock:			
Beginning balances	180	178	176
Issuances under equity incentive plans, net of tax	2	2	2
Ending balances	<u>182</u>	<u>180</u>	<u>178</u>
Additional paid-in capital:			
Beginning balances	4,832,707	4,669,926	4,483,220
Issuances under equity incentive plans, net of tax	27,275	(11,071)	31,583
Stock-based compensation	183,547	163,891	155,139
Repurchase of common stock	(50,000)	—	—
Conversion of convertible notes, net	—	—	(16)
Common stock held by the NQDC	(122)	(692)	—
Accounting impact of NQDC Plan change (See Note 17)	—	10,653	—
Ending balances	<u>4,993,407</u>	<u>4,832,707</u>	<u>4,669,926</u>
Treasury stock:			
Beginning balances	—	—	—
Purchase of treasury stock	(50,000)	—	—
Retirement of treasury stock	50,000	—	—
Ending balance	<u>—</u>	<u>—</u>	<u>—</u>
Company common stock held by the NQDC:			
Beginning balances	(9,961)	(13,301)	(14,224)
Common stock held by the NQDC	122	692	923
Accounting impact of NQDC Plan change (See Note 17)	—	2,648	—
Ending balances	<u>(9,839)</u>	<u>(9,961)</u>	<u>(13,301)</u>
Accumulated other comprehensive income (loss):			
Beginning balances	20,164	5,271	(22,961)
Impact of change in accounting principle	—	—	(586)
Other comprehensive income (loss)	(36,303)	14,893	28,818
Ending balances	<u>(16,139)</u>	<u>20,164</u>	<u>5,271</u>
Accumulated Deficit:			
Beginning balances	(1,720,709)	(1,694,134)	(1,637,548)
Impact of changes in accounting principles	—	(2,727)	20,625
Net income (loss)	859,100	(23,848)	(77,211)
Ending balances	<u>(861,609)</u>	<u>(1,720,709)</u>	<u>(1,694,134)</u>
Total stockholders' equity, ending balances	<u>\$ 4,106,002</u>	<u>\$ 3,122,381</u>	<u>\$ 2,967,940</u>

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

**BIOMARIN PHARMACEUTICAL INC.**  
**CONSOLIDATED STATEMENTS OF CASH FLOWS**  
**Years Ended December 31, 2020, 2019 and 2018**  
(In thousands of U.S. dollars)

	2020	2019	2018
<b>CASH FLOWS FROM OPERATING ACTIVITIES:</b>			
NET INCOME (LOSS)	\$ 859,100	\$ (23,848)	\$ (77,211)
Adjustments to reconcile net income (loss) to net cash used in operating activities:			
Depreciation and amortization	105,172	105,300	95,671
Non-cash interest expense	16,511	13,960	31,186
Amortization of premium on investments (accretion of discount)	567	(2,000)	358
Stock-based compensation expense	189,711	159,865	148,819
Gain on sale of nonfinancial assets	(59,495)	(25,000)	(50,000)
Inventory reserves, net of stock-based compensation	75,609	—	—
Deferred income taxes	(888,907)	(82,760)	(68,378)
Unrealized foreign exchange loss (gain)	8,011	1,025	(17,766)
Non-cash changes in the fair value of contingent consideration	4,500	5,205	9,296
Other	(997)	(2,393)	(2,347)
Changes in operating assets and liabilities:			
Accounts receivable, net	(59,035)	(37,852)	(54,274)
Inventory	(61,151)	(107,554)	(23,747)
Other current assets	18,312	(27,008)	(17,767)
Other assets	(28,647)	(8,895)	(935)
Accounts payable and accrued liabilities	(87,025)	77,089	38,389
Other long-term liabilities	(6,871)	3,128	8,914
Net cash provided by operating activities	85,365	48,262	20,208
<b>CASH FLOWS FROM INVESTING ACTIVITIES:</b>			
Purchases of property, plant and equipment	(114,312)	(145,026)	(144,620)
Maturities and sales of investments	555,834	740,211	993,734
Purchase of available-for-sale debt securities	(529,663)	(632,023)	(634,753)
Proceeds from sale of nonfinancial assets	67,159	25,000	50,000
Purchase of intangible assets	(23,207)	(18,380)	—
Investment in convertible note	(8,709)	—	—
Other	(723)	(808)	(10)
Net cash provided by (used in) investing activities	(53,621)	(31,026)	264,351
<b>CASH FLOWS FROM FINANCING ACTIVITIES:</b>			
Proceeds from exercises of awards under equity incentive plans	71,913	31,611	67,488
Taxes paid related to net share settlement of equity awards	(44,638)	(42,680)	(35,919)
Repurchase of common stock	(50,000)	—	—
Proceeds from convertible senior subordinated note offering, net	585,752	—	—
Repayments of convertible debt	(374,991)	—	(374,953)
Payment of contingent consideration	—	(58,518)	(44,623)
Principal repayments of financing leases	(6,918)	(5,087)	—
Net cash provided by (used in) financing activities	181,118	(74,674)	(388,007)
Effect of exchange rate changes on cash	(1,150)	902	(598)
<b>NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS</b>	<b>211,712</b>	<b>(56,536)</b>	<b>(104,046)</b>
Cash and cash equivalents:			
Beginning of period	437,446	493,982	598,028
End of period	\$ 649,158	\$ 437,446	\$ 493,982
<b>SUPPLEMENTAL CASH FLOW DISCLOSURES:</b>			
Cash paid for interest, net of interest capitalized into fixed assets	\$ 12,178	\$ 8,552	\$ 11,623
Cash paid for income taxes	\$ 8,977	\$ 9,726	\$ 16,676
<b>SUPPLEMENTAL CASH FLOW DISCLOSURES FOR NON-CASH INVESTING AND FINANCING ACTIVITIES:</b>			
Increase (decrease) in accounts payable and accrued liabilities related to fixed assets	\$ (5,184)	\$ 7,589	\$ (1,206)
Increase (decrease) in accounts payable and accrued liabilities related to intangible assets	(292)	4,247	—

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) NATURE OF OPERATIONS AND BUSINESS RISKS**

BioMarin Pharmaceutical Inc. (the Company or BioMarin) is a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare diseases and medical conditions. The Company selects product candidates for diseases and conditions that represent a significant unmet medical need, have well-understood biology and provide an opportunity to be first-to-market or offer a significant benefit over existing products. The Company's portfolio consists of several commercial products and multiple clinical and preclinical product candidates for the treatment of various diseases.

The Company expects to continue to finance future cash needs that exceed its operating activities primarily through its current cash, cash equivalents and investments and through proceeds from debt or equity offerings, commercial borrowing, or through collaborative agreements with corporate partners. If the Company elects to increase its spending on development programs significantly above current long-term plans or enters into potential licenses and other acquisitions of complementary technologies, products or companies, the Company may need additional capital.

The Company is subject to a number of risks, including: the continuing impact of the novel coronavirus disease (referred to as COVID-19) pandemic on the Company's business; the financial performance of its commercial products; the potential need for additional financing; the Company's ability to successfully commercialize its approved products; the uncertainty of the Company's research and development efforts resulting in future successful commercial products; the Company's ability to successfully obtain regulatory approval for new products; significant competition from larger organizations; reliance on the proprietary technology of others; dependence on key personnel; uncertain patent protection; dependence on corporate partners and collaborators; and possible restrictions on reimbursement from governmental agencies and healthcare organizations, as well as other changes in the healthcare industry.

**(2) BASIS OF PRESENTATION*****Basis of Presentation***

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

On January 1, 2020, the Company adopted Financial Accounting Standards Board (FASB) Accounting Standards Update (ASU) No. 2016-13, *Financial Instruments-Credit Losses: Measurement of Credit Losses on Financial Instruments* (ASU 2016-13), as amended, using a modified retrospective approach. The standard has amended the guidance for measuring and recording credit losses on financial assets measured at amortized cost by replacing the incurred-loss model with an expected-loss model. This new standard also requires that credit losses related to available-for-sale debt securities be recorded as an allowance through net income rather than by reducing the carrying amount under the current, other-than-temporary impairment model. Results for reporting periods beginning January 1, 2020 are presented under ASU 2016-13 and the adoption of this standard had no impact on the Company's Financial Statements. See Note 4 to these Consolidated Financial Statements for additional discussion of the adoption of ASU 2016-13 and required disclosures.

***Use of Estimates***

U.S. GAAP requires management to make estimates and assumptions that affect amounts reported in 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. The full extent to which the COVID-19 pandemic will directly or indirectly impact the Company's business, results of operations and financial condition, including revenues, expenses, reserves and allowances, manufacturing, clinical trials and research and development costs, will depend on future developments that are highly

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

uncertain at this time. As events continue to evolve and additional information becomes available, the Company's estimates may change materially in future periods.

**(3) SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES*****Cash and Cash Equivalents***

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

***Marketable and Non-Marketable Securities******Marketable Securities***

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

All marketable securities are classified as available-for-sale. Available-for-sale debt securities are measured and recorded at fair market value with unrealized gains and losses included in Accumulated Other Comprehensive Income (AOCI) on the Company's Consolidated Balance Sheets, with the exception of any declines in fair value below the cost basis that are a result of a credit loss, which, if any, are reported in Other Income, 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, Net. The Company regularly reviews its non-marketable equity securities for indicators of impairment.

***Inventory******Commercial Inventory***

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

***Inventory Produced Prior to Regulatory Approval***

When future commercialization for a product candidate is considered probable and management believes that material uncertainties related to the ultimate regulatory approval have been significantly reduced and the Company expects to realize economic benefit in the future, the Company capitalizes pre-launch or pre-qualification manufacturing costs prior to regulatory approval. For inventories that are capitalized in preparation of product launch, a number of factors are taken into consideration based on information available at the time, including the product candidate's current status in the drug development and regulatory approval process, results from the related pivotal clinical trial, results from meetings with the relevant regulatory authorities prior to the filing of regulatory applications, historical experience, as well as potential impediments to the approval process such as product safety or efficacy, as well as commercialization and market trends. If additional requirements are subsequently presented by the regulatory authorities, prior to their final decision thus extending anticipated regulatory approval timelines resulting in expiration of the product prior to revised demand forecasts, the pre-launch inventory costs are expensed to Cost of Sales. If the marketing

## BIOMARIN PHARMACEUTICAL INC.

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

application is ultimately rejected by the applicable regulators and the pre-launch inventory cannot be sold for commercial use, the pre-launch inventory costs are expensed to Research and Development (R&D).

**Property, Plant and Equipment**

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

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

**Leases**

Effective January 1, 2019, the Company adopted Accounting Standards Codification (ASC) Topic 842, *Leases*. The amended guidance required balance sheet recognition of lease right-of-use (ROU) assets and liabilities by lessees for leases classified as operating leases. The Company adopted ASC Topic 842 using the modified retrospective method for all lease arrangements at the beginning of the period of adoption through a cumulative adjustment to Accumulated Deficit.

The Company determines if an arrangement is a lease at contract inception. For leases where the Company is the lessee, ROU assets represent the Company's right to use the underlying asset for the term of the lease and the lease liabilities represent the lease payment obligation. ROU assets and lease liabilities are recognized at the lease commencement date based on the present value of the future lease payments over the lease term. The Company uses its incremental borrowing rate based on the information available at the commencement date of the underlying lease arrangement to determine the present value of lease payments. The ROU asset also includes any prepaid lease payments and any lease incentives received. The lease term to calculate the ROU asset and related lease liability includes options to extend or terminate the lease when it is reasonably certain that the Company will exercise the option. The Company's lease agreements generally do not contain any material variable lease payments, residual value guarantees or restrictive covenants.

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

**Goodwill and Intangible Assets**

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

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

**Impairment**

The Company assesses its 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. Impairment charges that are not material are recorded to Intangible Asset Amortization and Contingent Consideration in the Consolidated Statements of Operations.

**Long-lived Asset Impairment**

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

**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 arrangements that are within the scope of ASC Topic 606, 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 Genzyme (Genzyme). 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. Amounts collected from customers and remitted to governmental authorities, which primarily consist of value-added taxes related to product sales in foreign jurisdictions, are presented on a net basis on the Company's Consolidated Statements of Operations, in that taxes billed to customers are not included as a component of Net Product Revenues.

For Aldurazyme revenues, the Company receives a payment ranging from 39.5% to 50% on worldwide net Aldurazyme sales by Genzyme depending on sales volume, which is included in Net Product Revenues on the Company's Consolidated Statements of Operations. The Company recognizes its best estimate of the revenue it expects to earn when the product is released and control is transferred to Genzyme. The Company records Aldurazyme net product revenues based on the estimated variable consideration payable when the product is sold through by Genzyme. Actual amounts of consideration ultimately received may differ from the Company's estimates. Differences between the estimated variable consideration to be received from Genzyme

## BIOMARIN PHARMACEUTICAL INC.

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

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 rebates, 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, 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 Rebates:* The Company records reserves for rebates payable under Medicaid and other government programs as a reduction of revenue at the time product revenues are recorded. The Company's reserve calculations require estimates, including estimates of customer mix, 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 Brineura, Kuvan or Palynziq therapy. 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

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

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.

**Convertible Debt**

For non-conventional convertible debt that may be settled entirely or partially in cash, the Company separately accounts for the liability and equity components by allocating the proceeds from issuance between the liability component and the embedded conversion option, or equity component. The value of the equity component is calculated by first measuring the fair value of the liability component, using the interest rate of a similar liability that does not have a conversion feature, as of the issuance date. The difference between the proceeds from the convertible debt issuance and the amount measured as the liability component is recorded as the equity component with a corresponding discount recorded on the debt. The liability component is presented net of any discounts and issuance costs. For conventional convertible debt that may only be settled with common shares, the Company reports debt, net of any discounts or issuance costs, on the Consolidated Balance Sheets.

The Company recognizes discount accretion and debt issuance cost amortization using the effective interest method and is reported in Interest Expense on the Consolidated Statements of Operations.

**Net Income (Loss) Per Common Share**

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

**Stock-Based Compensation**

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

**Restricted Stock Units**

The fair value of restricted stock units (RSUs) with service-based vesting conditions and RSUs with performance conditions is determined to be the fair market value of the Company's underlying common stock on the date of grant. The stock-based compensation expense for RSUs with service-based vesting is recognized over the period during which the vesting restrictions lapse. Stock-based compensation expense for RSUs with performance conditions is recognized beginning in the period the Company determines it is probable that the performance condition will be achieved. Management expectations related to the achievement of performance goals associated with RSUs with performance conditions are assessed regularly to determine whether such grants are expected to vest. The fair value for RSUs with market conditions is estimated using the Monte Carlo valuation model. 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

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

options is based on observed historical exercise patterns. In estimating the life of stock options, the Company has identified two employee groups with distinctly different historical exercise patterns: executive and non-executive. The executive employee group has a history of holding stock options for longer periods than non-executive employees. The expected term of purchase rights for ESPP is based on each tranche of an offering period, which is four tranches in a twenty-four-month period.

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

***Income Taxes***

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

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

***Foreign Currency***

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

***Derivatives and Hedging Activities***

The Company uses forward foreign currency exchange contracts (forward contracts) to hedge certain operational exposures resulting from potential changes in foreign currency exchange rates. Such exposures result from portions of the Company's forecasted revenues and operating expenses being denominated in currencies other than the USD, primarily the Euro. The Company designates certain of these forward contracts as hedging instruments and also enters into forward contracts that are considered to be economic hedges that are not designated as hedging instruments. Whether designated or undesignated, these forward contracts protect against the reduction in value of forecasted foreign currency cash flows resulting from product revenues, royalty revenues, operating expenses and 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 rates and interest rates and takes into consideration the current creditworthiness of the counterparties or the Company, as applicable. For derivatives designated as hedging instruments, the entire change in the fair value of qualifying derivative instruments is recorded in AOCI and amounts deferred in AOCI are reclassified to earnings in the same line item in which the earnings effect of the hedged item is reported. Derivatives not designated as hedging instruments are adjusted to fair value through earnings in SG&A in the Consolidated Statements of Operations.

***Fair Value of Financial Instruments***

The Company applies fair value accounting for all financial assets and liabilities and non-financial assets and liabilities that are recognized or disclosed at fair value in the financial statements on a recurring basis. The Company defines fair value as the price that would be received from selling an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. When determining the fair value measurements for assets and liabilities that are required to be recorded at fair value, the Company considers the principal or most advantageous market in which the Company would transact and the



## BIOMARIN PHARMACEUTICAL INC.

**NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)**  
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market-based risk measurements or assumptions that market participants would use to price the asset or liability, such as risks inherent in valuation techniques, transfer restrictions and credit risk. When estimating fair value, depending on the nature and complexity of the asset or liability, the Company may use the following techniques:

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

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

- Quoted prices for identical assets or liabilities in active markets (Level 1 inputs)
- Quoted prices for similar assets or liabilities in active markets, or quoted prices for identical or similar assets or liabilities that are not active, or inputs other than quoted process that are directly or indirectly observable, or inputs that are derived principally from, or corroborated by, observable market data by correlation or other means (Level 2 inputs)
- Unobservable inputs that reflect estimates and assumptions (Level 3 inputs)

The Company's Level 2 instruments are valued using third-party pricing sources. The pricing services utilize industry standard valuation models, including both income and market-based approaches, for which all significant inputs are observable, either directly or indirectly, to estimate fair value. These inputs include reported trades of and broker/dealer quotes on the same or similar securities, issuer credit spreads, benchmark securities, prepayment/default projections based on historical data and other observable inputs. The Company validates the prices provided by its third-party pricing services by understanding the models used, obtaining market values from other pricing sources, analyzing pricing data in certain instances and confirming those securities traded in active markets.

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

See Notes 5, 10, 11, 13, 19 and 20 to these Consolidated Financial Statements for further information on the nature of these financial instruments.

**Segment Information**

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

**(4) RECENT ACCOUNTING PRONOUNCEMENTS**

Effective January 1, 2020, the Company adopted FASB ASU No. 2016-13, *Financial Instruments-Credit Losses: Measurement of Credit Losses on Financial Instruments* (ASU 2016-13), as amended, using a modified retrospective approach. The standard has amended the guidance for measuring and recording credit losses on financial assets measured at amortized cost by replacing the incurred-loss model with an expected-loss model. This new standard also requires that credit losses related to available-for-sale debt securities be recorded as an allowance through net income rather than by reducing the carrying amount

**BIOMARIN PHARMACEUTICAL INC.**
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under the previous other-than-temporary impairment model. The adoption of this standard had no impact on the Company's Financial Statements.

**(5) 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, 2020 and 2019, respectively:

December 31, 2020							
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Aggregate Fair Value	Cash and Cash Equivalents	Short-term Marketable Securities <sup>(1)</sup>	Long-term Marketable Securities <sup>(2)</sup>
<b>Level 1:</b>							
Cash	\$ 370,325	\$ —	\$ —	\$ 370,325	\$ 370,325	\$ —	\$ —
<b>Level 2:</b>							
Money market instruments	264,833	—	—	264,833	264,833	—	—
Corporate debt securities	413,137	3,261	(8)	416,390	—	220,551	195,839
U.S. government agency securities	265,298	1,555	(1)	266,852	14,000	192,488	60,364
Asset-backed securities	31,659	85	(2)	31,742	—	3,189	28,553
Foreign and other	549	168	—	717	—	—	717
Subtotal	975,476	5,069	(11)	980,534	278,833	416,228	285,473
Total	\$ 1,345,801	\$ 5,069	\$ (11)	\$ 1,350,859	\$ 649,158	\$ 416,228	\$ 285,473

December 31, 2019							
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Aggregate Fair Value	Cash and Cash Equivalents	Short-term Marketable Securities <sup>(1)</sup>	Long-term Marketable Securities <sup>(2)</sup>
<b>Level 1:</b>							
Cash	\$ 259,347	\$ —	\$ —	\$ 259,347	\$ 259,347	\$ —	\$ —
<b>Level 2:</b>							
Money market instruments	173,100	—	—	173,100	173,100	—	—
Corporate debt securities	518,523	3,575	(12)	522,086	—	233,294	288,792
U.S. government agency securities	209,633	993	(67)	210,559	4,999	83,067	122,493
Foreign and other	549	145	(1)	693	—	—	693
Subtotal	901,805	4,713	(80)	906,438	178,099	316,361	411,978
Total	\$ 1,161,152	\$ 4,713	\$ (80)	\$ 1,165,785	\$ 437,446	\$ 316,361	\$ 411,978

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

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

As of December 31, 2020, 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.

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**NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)**  
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The Company has certain investments in non-marketable equity securities, measured using unobservable valuation inputs remeasured on a nonrecurring basis, which are collectively considered strategic investments. As of December 31, 2020 and 2019, the fair value of the Company's strategic investments was \$10.5 million and \$6.2 million, respectively. These investments were recorded in Other Assets in the Company's Consolidated Balance Sheets.

See Note 3 to these Consolidated Financial Statements for additional discussion regarding the Company's fair value measurements.

**(6) GOODWILL AND INTANGIBLE ASSETS**

The change in the carrying value of Goodwill was as follows:

	December 31,	
	2020	2019
Beginning balance	\$ 197,039	\$ 197,039
Less: disposition	(840)	—
Ending carrying value	<u>\$ 196,199</u>	<u>\$ 197,039</u>

In January 2020, the Company completed the sale of worldwide rights to Firdapse, the Company's commercial product for the treatment of Lambert-Eaton myasthenic syndrome, to a third party in exchange for a one-time cash payment of \$67.2 million plus residual royalties. Under the terms of the agreement, the Company agreed to provide certain transition services to the third-party purchaser, such as customer sales and support, for up to 12 months after the closing of the transaction. During the first quarter of 2020, the Company recognized a before-tax net gain of \$59.5 million related to the sale of the Firdapse intellectual property (IP) and existing inventory. As a result of the sale of Firdapse, in the first quarter of 2020 the Company recognized a \$0.8 million reduction to Goodwill and disposed of \$32.2 million in intangible assets, including related accumulated amortization of \$31.6 million.

The Company performed its annual assessment for goodwill impairment for the year ended December 31, 2020 and no goodwill impairment charges were recorded.

Intangible Assets, Net consisted of the following:

	December 31,	
	2020	2019
Finite-lived intangible assets	\$ 644,087	\$ 652,734
Less: Accumulated amortization	(226,816)	(196,154)
Net carrying value	<u>\$ 417,271</u>	<u>\$ 456,580</u>

The following table summarizes the carrying value and estimated remaining life of the Company's finite-lived intangible assets as of December 31, 2020:

	Net Balance	Average Remaining Life
Acquired intellectual property	\$ 350,275	6.9 years
Repurchased royalty rights	19,687	2.9 years
Technology transfer	46,657	Not applicable (1)
Other	652	0.5 - 3.4 years
Total	<u>\$ 417,271</u>	

(1) The technology transfer intangible asset has not yet been placed into service.

As of December 31, 2020, the estimated future amortization expense associated with the Company's finite-lived intangible assets, exclusive of the technology transfer asset that has not been placed into service, was as follows:

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Fiscal Year	Amount
2021	\$ 61,963
2022	61,939
2023	61,311
2024	55,036
2025	35,759
Thereafter	94,606
	<u>\$ 370,614</u>

In 2019 and 2018, the Company received \$25.0 million and \$50.0 million, respectively, due to the achievement by a third party of development and regulatory milestones and commercial sales milestones related to a previously sold intangible asset, which the Company recorded as a Gain on Sale of Nonfinancial Assets in the Consolidated Statements of Operations.

**(7) PROPERTY, PLANT AND EQUIPMENT**

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

	December 31,	
	2020	2019
Building and improvements	\$ 761,560	\$ 725,906
Manufacturing and laboratory equipment	414,439	366,951
Computer hardware and software	189,740	167,554
Land	90,418	90,418
Leasehold improvements	55,134	51,324
Furniture and equipment	40,223	38,569
Land improvements	7,412	7,349
Construction-in-progress	109,140	111,897
	<u>1,668,066</u>	<u>1,559,968</u>
Accumulated depreciation	(635,595)	(549,100)
Total property, plant and equipment, net	<u>\$ 1,032,471</u>	<u>\$ 1,010,868</u>

The construction-in-progress balance primarily included costs related to significant in-progress projects at the Company's facilities in Marin County, California, and Shanbally, Ireland.

	Years Ended December 31,		
	2020	2019	2018
Depreciation expense	\$ 88,169	\$ 89,285	\$ 90,375
Depreciation capitalized into inventory	\$ 44,492	\$ 37,481	\$ 25,227

## (8) INVENTORY

Inventory consisted of the following:

	December 31,	
	2020	2019
Raw materials	\$ 76,673	\$ 74,442
Work-in-process	308,286	349,978
Finished goods	313,589	255,855
Total inventory	\$ 698,548	\$ 680,275

Valoctocogene roxaparvovec is an investigational gene therapy product candidate for the treatment of severe hemophilia A. The Company must receive marketing approval from the applicable regulators before the valoctocogene roxaparvovec inventory can be sold commercially. Starting in the second quarter of 2019, the Company believed that material uncertainties related to the ultimate regulatory approval of valoctocogene roxaparvovec for commercial sale had been significantly reduced and included the manufacturing-related costs for the commercial production of valoctocogene roxaparvovec in inventory. A number of factors were taken into consideration based on the information available at the time, including the status in the drug development process, pivotal clinical trial results for the underlying product candidate, results from meetings with the relevant regulatory authorities prior to the filing of regulatory applications, historical experience, as well as potential impediments to the approval process such as product safety or efficacy, as well as commercialization and marketplace trends. The Company had \$29.2 million of valoctocogene roxaparvovec pre-launch inventory as of December 31, 2019.

In the third quarter of 2020, the Company unexpectedly received a Complete Response Letter from the U.S. Food and Drug Administration (FDA) and a Joint Assessment Report from the European Medicines Agency (EMA) respectively, both indicating that the Company's regulatory applications for valoctocogene roxaparvovec could not be approved in their present form and requesting additional safety and efficacy data from the ongoing Phase 3 study. The Company evaluated the impact of the new requirement for Phase 3 data that is currently unknown and determined the value of the pre-launch inventory was no longer recoverable due to delays in anticipated regulatory approvals. As a result, the Company adjusted the pre-launch inventory to zero, its net realizable value, and recorded \$87.2 million to Cost of Sales during the year ended December 31, 2020. The Company had no pre-launch inventory balance as of December 31, 2020.

## BIOMARIN PHARMACEUTICAL INC.

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

**(9) SUPPLEMENTAL BALANCE SHEET INFORMATION**

Accounts Payable and Accrued Liabilities consisted of the following:

	December 31,	
	2020	2019
Accounts payable and accrued operating expenses	\$ 191,429	\$ 240,981
Accrued compensation expense	165,023	192,467
Accrued rebates payable	\$ 65,526	57,163
Forward foreign currency exchange contracts	17,798	10,448
Accrued royalties payable	17,155	30,797
Lease liability	11,754	10,700
Accrued income taxes	9,661	3,195
Value added taxes payable	9,562	8,395
Deferred revenue	152	13,037
Other	4,488	3,438
<b>Total accounts payable and accrued liabilities</b>	<b>\$ 492,548</b>	<b>\$ 570,621</b>

The roll forward of significant estimated accrued rebates and reserve for cash discounts for the years ended December 31, 2020, 2019 and 2018, were as follows:

	Balance at Beginning of Period	Provision for Current Period Sales	Payments	Balance at End of Period
<b>Year ended December 31, 2020:</b>				
Accrued rebates	\$ 57,163	\$ 113,165	\$ (104,802)	\$ 65,526
Reserve for cash discounts	\$ 1,889	\$ 17,191	\$ (17,364)	\$ 1,716
<b>Year ended December 31, 2019:</b>				
Accrued rebates	\$ 43,116	\$ 91,748	\$ (77,701)	\$ 57,163
Reserve for cash discounts	\$ 1,197	\$ 15,335	\$ (14,643)	\$ 1,889
<b>Year ended December 31, 2018:</b>				
Accrued rebates	\$ 36,472	\$ 67,843	\$ (61,199)	\$ 43,116
Reserve for cash discounts	\$ 1,055	\$ 12,474	\$ (12,332)	\$ 1,197

## BIOMARIN PHARMACEUTICAL INC.

**NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)**  
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**(10) FAIR VALUE MEASUREMENTS**

The Company measures certain financial assets and liabilities at fair value in accordance with the policy described in Note 3 – *Significant Accounting Policies* to these Consolidated Financial Statements.

The following tables present the classification within the fair value hierarchy of financial assets and liabilities not disclosed elsewhere in these Consolidated Financial Statements that are remeasured on a recurring basis as of December 31, 2020 and 2019. Other than the Company's fixed-rate convertible debt disclosed in Note 13 to these Consolidated Financial Statements, there were no financial assets or liabilities that were remeasured using a quoted price in active markets for identical assets (Level 1) as of December 31, 2020 and 2019.

	Fair Value Measurements as of December 31, 2020		
	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
<b>Assets:</b>			
Other current assets:			
NQDC Plan assets	\$ 2,415	\$ —	\$ 2,415
Other assets:			
NQDC Plan assets	19,962	—	19,962
Restricted investments <sup>(1)</sup>	4,487	—	4,487
Total other assets	24,449	—	24,449
Total assets	\$ 26,864	\$ —	\$ 26,864
<b>Liabilities:</b>			
Current liabilities:			
NQDC Plan liability	\$ 2,415	\$ —	\$ 2,415
Other long-term liabilities:			
NQDC Plan liability	19,962	—	19,962
Contingent consideration	—	60,130	60,130
Total other long-term liabilities	19,962	60,130	80,092
Total liabilities	\$ 22,377	\$ 60,130	\$ 82,507



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

	Fair Value Measurements as of December 31, 2019		
	Significant Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
<b>Assets:</b>			
Other current assets:			
NQDC Plan assets	\$ 1,177	\$ —	\$ 1,177
Other assets:			
NQDC Plan assets	16,288	—	16,288
Restricted investments <sup>(1)</sup>	3,168	—	3,168
Total other assets	19,456	—	19,456
Total assets	\$ 20,633	\$ —	\$ 20,633
<b>Liabilities:</b>			
Current Liabilities:			
NQDC Plan liability	\$ 1,177	\$ —	\$ 1,177
Other long-term liabilities:			
NQDC Plan liability	16,288	—	16,288
Contingent consideration	—	50,793	50,793
Total other long-term liabilities	16,288	50,793	67,081
Total liabilities	\$ 17,465	\$ 50,793	\$ 68,258

(1) The restricted investments as of December 31, 2020 and 2019 secure the Company's irrevocable standby letters of credit obtained in connection with certain commercial agreements.

There were no transfers between levels during the periods presented.

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

Contingent consideration as of December 31, 2019	\$ 50,793
Changes in the fair value of contingent consideration	4,500
Foreign exchange remeasurement of Euro denominated contingent consideration	4,837
Contingent consideration as of December 31, 2020	\$ 60,130

**(11) DERIVATIVE INSTRUMENTS AND HEDGING STRATEGIES**

The Company's forward foreign currency exchange contracts (forward contracts) designated as hedging instruments have maturities up to two years and three months. 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.

## BIOMARIN PHARMACEUTICAL INC.

**NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)**  
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Foreign Exchange Contracts	December 31, 2020	December 31, 2019
<b>Derivatives designated as hedging instruments:</b>		
Sell	\$ 782,327	\$ 820,546
Purchase	\$ 189,540	\$ 212,348
<b>Derivatives not designated as hedging instruments:</b>		
Sell	\$ 98,343	\$ 77,335
Purchase	\$ 12,277	\$ 30,818

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

Balance Sheet Location	December 31, 2020	December 31, 2019
<b>Derivatives designated as hedging instruments:</b>		
<b>Asset Derivatives - Level 2 <sup>(1)</sup></b>		
Other current assets	\$ 6,268	\$ 19,584
Other assets	3,148	13,539
Subtotal	\$ 9,416	\$ 33,123
<b>Liability Derivatives - Level 2 <sup>(1)</sup></b>		
Accounts payable and accrued liabilities	\$ 17,551	\$ 8,184
Other long-term liabilities	11,020	5,493
Subtotal	\$ 28,571	\$ 13,677
<b>Derivatives not designated as hedging instruments:</b>		
<b>Asset Derivatives - Level 2 <sup>(1)</sup></b>		
Other current assets	\$ 84	\$ 469
<b>Liability Derivatives - Level 2 <sup>(1)</sup></b>		
Accounts payable and accrued liabilities	\$ 247	\$ 2,264
<b>Total Derivatives Assets</b>	\$ 9,500	\$ 33,592
<b>Total Derivatives Liabilities</b>	\$ 28,818	\$ 15,941

(1) See Note 3 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 Operations for the periods presented.

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

	Years Ended December 31,			
	2020		2019	
		Cash Flow Hedging Gains (Losses) Reclassified into Earnings		Cash Flow Hedging Gains (Losses) Reclassified into Earnings
<b>Derivatives Designated as Cash Flow Hedging Instruments</b>				
Net product revenues as reported	\$ 1,805,861	\$ 18,122	\$ 1,661,043	\$ 17,672
Operating expenses as reported	\$ 1,897,220	\$ (4,942)	\$ 1,804,505	\$ (3,453)
		Gains (Losses) Recognized in Earnings		Gains (Losses) Recognized in Earnings
<b>Derivatives Not Designated as Hedging Instruments</b>				
Operating expenses		\$ 115		\$ (5,259)

As of December 31, 2020, the Company expects to reclassify unrealized losses of \$12.2 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 14 to these Consolidated Financial Statements.

**(12) LEASES**

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

Lease Classification	Classification	December 31,	
		2020	2019
<b>Assets:</b>			
Operating	Other assets	\$ 46,014	\$ 49,045
Financing	Other assets	11,095	10,389
Total ROU assets		\$ 57,109	\$ 59,434
<b>Liabilities:</b>			
<b>Current:</b>			
Operating	Accounts payable and accrued liabilities	\$ 8,889	\$ 7,451
Financing	Accounts payable and accrued liabilities	2,865	3,249
<b>Noncurrent:</b>			
Operating	Other long-term liabilities	40,483	44,092
Financing	Other long-term liabilities	4,006	6,708
Total lease liabilities		\$ 56,243	\$ 61,500

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**NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)**  
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Maturities of lease liabilities as of December 31, 2020 by fiscal year were as follows:

Maturity of Lease Liabilities	Operating	Financing	Total
2021	\$ 11,295	\$ 3,095	\$ 14,390
2022	10,127	2,383	12,510
2023	8,605	1,800	10,405
2024	6,570	50	6,620
2025	5,561	21	5,582
Thereafter	16,758	—	16,758
Total lease payments	58,916	7,349	66,265
Less: Interest	(9,544)	(478)	(10,022)
Present value of lease liabilities	<u>\$ 49,372</u>	<u>\$ 6,871</u>	<u>\$ 56,243</u>

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

Lease Cost	Classification	Years Ended December 31,	
		2020	2019
Operating <sup>(1)</sup>	Operating expenses	\$ 12,841	\$ 13,026
Financing:			
Amortization	Operating expenses	3,271	2,615
Interest expense	Operating expenses	448	606
Total lease costs		<u>\$ 16,560</u>	<u>\$ 16,247</u>

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

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:

Other Information	Years Ended December 31,	
	2020	2019
Weighted average remaining lease term (in years):		
Operating leases	6.8	7.9
Financing leases	2.6	3.3
Weighted average discount rate:		
Operating leases	4.9 %	5.2 %
Financing leases	5.2 %	5.4 %

As of December 31, 2020, no leases were expected to commence that would create significant rights and obligations for the Company.

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<b>Supplemental Cash Flow Information</b>	<b>Years Ended December 31,</b>	
	<b>2020</b>	<b>2019</b>
Cash paid for amounts included in the measurement of lease liabilities:		
Cash used in operating activities:		
Operating leases	\$ 10,536	\$ 8,183
Financing leases	\$ 450	\$ 628
Cash used in financing activities:		
Financing leases	\$ 6,918	\$ 5,087
ROU assets obtained in exchange for lease obligations:		
Operating leases	\$ 4,779	\$ 9,772
Financing leases	\$ 3,941	\$ 3,267

**(13) DEBT**
**Convertible Notes**

As of December 31, 2020, the Company had outstanding fixed-rate notes with varying maturities for an undiscounted aggregate principal amount of \$1.1 billion (collectively the Notes). The Notes are senior subordinated convertible obligations, and interest is payable in arrears, semi-annually. The following table summarizes information regarding the Company's convertible debt:

	<b>2020</b>	<b>2019</b>
1.25% senior subordinated convertible notes due in May 2027 (the 2027 Notes)	\$ 600,000	\$ —
Unamortized discount	(12,312)	—
Unamortized deferred offering costs	(683)	—
2027 Notes, net	587,005	—
0.599% senior subordinated convertible notes due in August 2024 (the 2024 Notes)	495,000	495,000
Unamortized discount	(5,116)	(6,533)
Unamortized deferred offering costs	(1,744)	(2,229)
2024 Notes, net	488,140	486,238
1.50% senior subordinated convertible notes matured in October 2020 (the 2020 Notes)	—	374,993
Unamortized discount	—	(12,078)
Unamortized deferred offering costs	—	(1,033)
2020 Notes, net <sup>(1)</sup>	—	361,882
<b>Total convertible debt, net</b>	<b>\$ 1,075,145</b>	<b>\$ 848,120</b>
<b>Fair value of fixed rate convertible debt <sup>(2)</sup>:</b>		
2027 Notes	\$ 627,090	\$ —
2024 Notes	530,714	521,839
2020 Notes	—	405,679
<b>Total fair value of fixed rate convertible debt</b>	<b>\$ 1,157,804</b>	<b>\$ 927,518</b>

(1) The 2020 Notes matured on October 15, 2020 and were settled in cash for approximately \$375.0 million. There were no shares issued in connection with the settlement as the Company's share price did not exceed the conversion price of

## BIOMARIN PHARMACEUTICAL INC.

**NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)**  
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\$94.15, as measured over a 25-day averaging period, and the capped call transaction entered into concurrently with the issuance of the 2020 Notes was not triggered. The Company incurred no gain or loss upon the extinguishment of the 2020 Notes.

- (2) The fair value of the Company's fixed-rate convertible debt is based on open market trades and is classified as Level 1 in the fair value hierarchy. See Note 3 to these Consolidated Financial Statements for additional information related to the Company's fair value measurements.

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

	Years Ended December 31,		
	2020	2019	2018
Coupon interest	\$ 12,350	\$ 4,907	\$ 12,452
Amortization of debt issuance costs	1,829	2,031	3,610
Accretion of discount on convertible notes	14,682	15,917	27,602
Total interest expense on convertible debt	\$ 28,861	\$ 22,855	\$ 43,664

**2027 Notes**

In May 2020, the Company issued \$600.0 million in aggregate principal amount of senior subordinated unsecured convertible notes with a maturity date of May 15, 2027. The 2027 Notes were issued to the public at par value and bear interest at the rate of 1.25% per annum. Interest is payable semi-annually in cash in arrears on May 15 and November 15 of each year, beginning November 15, 2020. The 2027 Notes are convertible, at the option of the holder into shares of the Company's common stock. The initial conversion rate for the 2027 Notes is 7.2743 shares per \$1,000 principal amount of the 2027 Notes, which represents a conversion price of approximately \$137.47 per share, subject to adjustment under certain conditions. Following certain corporate transactions, the Company will, in certain circumstances, increase the conversion rate for a holder that elects to convert its 2027 Notes in connection with such corporate transactions by a number of additional shares of the Company's common stock. A holder may convert fewer than all of such holder's 2027 Notes so long as the amount of the 2027 Notes converted is an integral multiple of \$1,000 principal amount. Net proceeds from the offering were \$585.8 million. In connection with the issuance of the 2027 Notes, the Company recorded a discount on the 2027 Notes of \$13.5 million, which will be accreted and recorded as additional interest expense over the life of the 2027 Notes. The Company also incurred \$0.7 million of issuance costs, which were deferred and are being amortized over the life of the 2027 Notes and recorded as additional interest expense.

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

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

**2024 Notes**

In August 2017, the Company issued \$495.0 million in aggregate principal amount of senior subordinated convertible notes with a maturity date of August 1, 2024. The 2024 Notes were issued to the public at 98% of face value and bear interest at the rate of 0.599% per annum. Interest is payable semi-annually in cash in arrears on February 1 and August 1 of each year, beginning February 1, 2018. The 2024 Notes are convertible, at the option of the holder into shares of the Company's common stock. The initial conversion rate for the 2024 Notes is 8.0212 shares per \$1,000 principal amount of the 2024 Notes, which represents a conversion price of approximately \$124.67 per share, subject to adjustment under certain conditions. Following certain corporate transactions, the Company will, in certain circumstances, increase the conversion rate for a holder that elects to convert its 2024 Notes in connection with such corporate transactions by a number of additional shares of the Company's common

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stock. A holder may convert fewer than all of such holder's 2024 Notes so long as the amount of the 2024 Notes converted is an integral multiple of \$1,000 principal amount. Net proceeds from the offering were \$481.7 million.

The 2024 Notes are senior subordinated, unsecured obligations, and rank (i) subordinated in right of payment to the prior payment in full of any of the Company's existing and future senior debt, (ii) equal in right of payment to any of the Company's existing and future senior subordinated debt, (iii) senior in right of payment to any of the Company's existing and future indebtedness that is expressly subordinated in right of payment to the 2024 Notes, and (iv) effectively subordinated to any of the Company's existing and future secured indebtedness, to the extent of the value of the collateral securing that indebtedness and structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables, and (to the extent the Company is not a holder thereof) preferred equity, if any, of the Company's subsidiaries. Upon the occurrence of a "fundamental change," as defined in the indenture governing the 2024 Notes, the holders may require the Company to repurchase all or a portion of such holder's 2024 Notes for cash at 100% of the principal amount of the 2024 Notes being purchased, plus any accrued and unpaid interest.

In connection with the issuance of the 2024 Notes, the Company recorded a discount on the 2024 Notes of \$9.9 million, which will be accreted and recorded as additional interest expense over the life of the 2024 Notes. In connection with the issuance of the 2024 Notes, the Company incurred \$3.4 million of issuance costs.

See Note 19 to these Consolidated Financial Statements for further discussion of the effect of conversion of the Company's convertible debt on net income (loss) per common share.

***Revolving Credit Facility***

In October 2018, the Company entered into an unsecured revolving credit facility of up to \$200.0 million (the 2018 Credit Facility). The 2018 Credit Facility includes a letter of credit subfacility and a swingline loan subfacility and is intended to finance ongoing working capital needs and for other general corporate purposes. Borrowings under the 2018 Credit Facility bear interest, at the Company's option, at a rate equal to either (a) the LIBOR rate (except that if LIBOR is less than zero it shall be deemed to be zero for purposes of the 2018 Credit Facility), or LIBOR successor rate, plus an applicable margin ranging from 1.00% to 1.95% per annum, based upon the Company's net leverage ratio and EBITDA for each of the two most recently ended four-quarter measurement periods, or (b) the Base Rate, generally the prime lending rate, plus an applicable margin ranging from 0.00% to 0.95%, based upon the Company's net leverage ratio and EBITDA for each of the two most recently ended four-quarter measurement periods. Commitment fees payable on the undrawn amount range from 0.15% to 0.35% per annum based upon the Company's net leverage ratio and EBITDA for each of the two most recently ended four-quarter measurement periods. The Company's obligations under the 2018 Credit Facility are guaranteed by its direct subsidiary, California Corporate Center Acquisition LLC, and such obligations may in the future be guaranteed from time to time by certain other material domestic subsidiaries. The 2018 Credit Facility matures on October 19, 2021 at which time all outstanding amounts become due and payable. The Company incurred approximately \$1.0 million of issuance costs, which are amortized to Interest Expense over the term of the 2018 Credit Facility. The 2018 Credit Facility contains financial covenants requiring the Company to maintain a minimum interest coverage ratio and a minimum liquidity requirement. As of December 31, 2020, there were no outstanding amounts due under the 2018 Credit Facility and the Company and certain of its subsidiaries that serve as guarantors were in compliance with all covenants.

**(14) ACCUMULATED OTHER COMPREHENSIVE INCOME (LOSS)**

The following table summarizes amounts reclassified out of AOCI and their effect on the Company's Consolidated Statements of Operations for the years ended December 31, 2020, 2019 and 2018.



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Details about AOCI Components	Consolidated Statements of Operations Classification	Years Ended December 31,		
		2020	2019	2018
Gains (losses) on cash flow hedges:				
Forward contracts	Net product revenues	\$ 18,122	\$ 17,672	\$ (6,005)
Forward contracts	Operating expenses	(4,942)	(1,819)	3,958
Total gain (loss) on cash flow hedges		13,180	15,853	(2,047)
Gain on sale of available-for-sale debt securities	Other income, net	552	—	—
Income tax effect of the above	Benefit from income taxes	(127)	—	—
Total gain on available-for-sale debt securities		425	—	—
		\$ 13,605	\$ 15,853	\$ (2,047)

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	Other	Total
AOCI balance at December 31, 2017	(20,232)	(2,722)	(7)	(22,961)
Impact of change in accounting principle <sup>(1)</sup>	—	(586)	—	(586)
AOCI balance as of January 1, 2018	(20,232)	(3,308)	(7)	(23,547)
Other comprehensive income (loss) before reclassifications	25,386	1,804	(6)	27,184
Less: gain (loss) reclassified from AOCI	(2,047)	—	—	(2,047)
Tax effect	—	(413)	—	(413)
Net current-period other comprehensive income (loss)	27,433	1,391	(6)	28,818
AOCI balance at December 31, 2018	\$ 7,201	\$ (1,917)	\$ (13)	\$ 5,271
Other comprehensive income (loss) before reclassifications	25,266	7,122	(2)	32,386
Less: gain (loss) reclassified from AOCI	15,853	—	—	15,853
Tax effect	—	(1,640)	—	(1,640)
Net current-period other comprehensive income (loss)	9,413	5,482	(2)	14,893
AOCI balance at December 31, 2019	16,614	3,565	(15)	20,164
Other comprehensive income (loss) before reclassifications	(23,462)	976	15	(22,471)
Less: gain (loss) reclassified from AOCI	13,180	552	—	13,732
Tax effect	—	(100)	—	(100)
Net current-period other comprehensive income (loss)	(36,642)	324	15	(36,303)
AOCI balance at December 31, 2020	(20,028)	3,889	—	(16,139)

(1) The amount represents the reclassification from AOCI to Accumulated Deficit due to the adoption of ASU 2018-02, *Income Statement-Reporting Comprehensive Income (Topic 220): Reclassification of Certain Tax Effects from Accumulated Other Comprehensive Income* (ASU 2018-02) in the first quarter of 2018.

**(15) REVENUE, CREDIT CONCENTRATIONS AND GEOGRAPHIC INFORMATION**

The Company operates in one business segment, which primarily focuses on the development and commercialization of innovative therapies for people with serious and life-threatening rare diseases and medical conditions. The Company considers

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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 following table disaggregates Total Revenues from external customers and collaborative partners by geographic region. Net product revenues by geographic region are based on patient location for the Company's commercial products, except for Aldurazyme, which is sold exclusively to Sanofi Genzyme (Genzyme) who markets and sells Aldurazyme world-wide. Aldurazyme revenues earned by the Company are included in the U.S. region as the transactions are with Genzyme, whose headquarters is located in the U.S.

	Years Ended December 31,		
	2020	2019	2018
Total revenues by geographic region:			
United States	\$ 912,375	\$ 778,440	\$ 696,793
Europe	518,465	509,188	436,434
Latin America	205,862	218,792	185,046
Rest of world	223,753	197,628	172,939
Total revenues	<u>\$ 1,860,455</u>	<u>\$ 1,704,048</u>	<u>\$ 1,491,212</u>

The following table disaggregates total Net Product Revenues by product.

	Years Ended December 31,		
	2020	2019	2018
Net product revenues by product:			
Vimizim	544,257	544,345	481,977
Kuvan	457,736	463,353	433,582
Naglazyme	391,298	374,334	345,851
Palynziq	170,983	86,857	12,173
Brineura	110,192	71,997	39,889
Firdapse	1,288	22,348	21,787
Total net product revenues marketed by the Company	<u>1,675,754</u>	<u>1,563,234</u>	<u>1,335,259</u>
Aldurazyme net product revenues marketed by Genzyme	<u>130,107</u>	<u>97,809</u>	<u>135,097</u>
Total net product revenues	<u>\$ 1,805,861</u>	<u>\$ 1,661,043</u>	<u>\$ 1,470,356</u>

The table below disaggregates total Net Product Revenues based on patient location for products sold directly by the Company, and global sales of Aldurazyme, which is marketed by Genzyme.

	Years Ended December 31,		
	2020	2019	2018
United States	\$ 756,863	\$ 669,171	\$ 560,030
Europe	498,725	485,596	424,357
Latin America	205,862	218,792	184,984
Rest of world	214,304	189,675	165,888
Total net product revenues marketed by the Company	<u>1,675,754</u>	<u>1,563,234</u>	<u>1,335,259</u>
Aldurazyme net product revenues marketed by Genzyme	<u>130,107</u>	<u>97,809</u>	<u>135,097</u>
Total net product revenue	<u>\$ 1,805,861</u>	<u>\$ 1,661,043</u>	<u>\$ 1,470,356</u>

## BIOMARIN PHARMACEUTICAL INC.

**NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)**  
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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,		
	2020	2019	2018
Customer A	16 %	17 %	18 %
Customer B	15 %	13 %	12 %
Customer C	12 %	11 %	10 %
Total	43 %	41 %	40 %

On a consolidated basis, two customers accounted for 24% and 22% of the Company's December 31, 2020 accounts receivable balance, respectively, compared to December 31, 2019 when two customers accounted for 24% and 16% of the accounts receivable balance, respectively. As of December 31, 2020 and 2019, the accounts receivable balance for Genzyme included \$72.1 million and \$60.2 million, respectively, of unbilled accounts receivable, which becomes payable to the Company when the product is sold through by Genzyme. 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 outbreak of COVID-19 continues to affect economies and business around the world. The Company's global revenue sources, mostly in the form of demand interruptions such as missed patient infusions and delayed treatment starts for new patients, and its business operations were impacted by COVID-19 during the year ended December 31, 2020 and the Company anticipates a continued impact due to COVID-19 on its financial results in fiscal year 2021. The extent and duration of such effects are highly uncertain and difficult to predict. The Company is actively monitoring and managing its response and assessing actual and potential impacts to its operating results and financial condition, as well as developments in its business, which could further impact developments, trends and expectations.

The Company is mindful that conditions in the current macroeconomic environment could affect the Company's ability to achieve its goals. 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 and effects of the impact of the ongoing COVID-19 pandemic may cause customers in those countries to delay payment or be unable to pay for the Company's products. The Company believes that the allowances for doubtful accounts related to these countries, if any, are adequate based on its analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries. The Company will continue to monitor these conditions and will attempt to adjust its business processes, as appropriate, to mitigate macroeconomic risks to its business.

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

	December 31,	
	2020	2019
Long-lived assets by geography:		
United States	\$ 771,286	\$ 773,991
Ireland	300,555	278,082
Rest of world	17,739	18,229
Total long-lived assets	\$ 1,089,580	\$ 1,070,302

**(16) EQUITY COMPENSATION PLANS AND STOCK-BASED COMPENSATION****Equity Compensation Plans***Shares Available Under Equity Compensation Plans*

As of December 31, 2020, an aggregate of approximately 36.7 million unissued shares was authorized for future issuance under the Company's stock plans, which primarily includes shares issuable under the 2017 Equity Incentive Plan and the ESPP.

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## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

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

Under the 2017 Equity Incentive Plan, shares issued under the Amended and Restated 2006 Share Incentive Plan (the 2006 Share Incentive Plan) and the 2017 Equity Incentive Plan that expire or are forfeited generally become available for future issuance under the 2017 Equity Incentive Plan. Shares formerly reserved for future issuance under the 2006 Share Incentive Plan were transferred to the 2017 Equity Incentive Plan and became available for issuance thereunder upon the effectiveness of the 2017 Equity Incentive Plan. No additional awards will be granted under the 2006 Share Incentive Plan; however, there are vested and unvested awards outstanding under the 2006 Share Incentive Plan. The Company's stock-based compensation plans are administered by the Company's Board of Directors (the Board), or designated Committee thereof, which selects persons to receive awards and determines the number of shares subject to each award and the terms, conditions, performance measures and other provisions of the awards. See Note 3 to these Consolidated Financial Statements for discussion regarding the valuation of equity awards.

*2017 Equity Incentive Plan*

The 2017 Equity Incentive Plan provides for awards of RSUs and stock options as well as other forms of equity compensation. Stock option awards granted to employees generally vest over a four-year period on a cliff basis twelve months after the grant date and then monthly thereafter. The contractual term of stock option awards is generally ten years from the grant date. RSUs granted to employees generally vest annually over a straight-line four-year period after the grant date. As of December 31, 2020, approximately 20.5 million shares were authorized and reserved for future issuance under the 2017 Equity Incentive Plan.

*Employee Stock Purchase Plan*

The ESPP was initially approved in June 2006, replacing the Company's previous plan, and was most recently amended in June 2019. Under BioMarin's ESPP, employees meeting specific employment qualifications are eligible to participate and can purchase shares on established dates (each purchase date) semi-annually through payroll deductions at the lower of 85% of the fair market value of the stock at the commencement of the offering period or each purchase date of the offering period. Each offering period will span up to two years. The ESPP permits eligible employees to purchase common stock through payroll deductions for up to 10% of qualified compensation, up to an annual limit of \$25,000. The ESPP is intended to qualify as an "employee stock purchase plan" under Section 423 of the Internal Revenue Code. During the year ended December 31, 2020, the Company issued 0.3 million shares under the ESPP. As of December 31, 2020, approximately 7.0 million shares were authorized and 3.4 million shares reserved for future issuance under the ESPP.

*Board of Director Grants*

On September 19, 2019, the Board of BioMarin approved revised compensation for the Independent Directors of the Company. 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 (\$375,000 prior to September 19, 2019), with the number of RSUs to be granted calculated based on the three-month trailing average closing price of the Company's common stock on the Nasdaq Global Select Market. The RSUs subject to the annual award vest in full on the one-year anniversary of the grant date, subject to each respective Director providing service to the Company through such vesting date. Upon election or appointment, a new Independent Director will receive an RSU grant on the same terms as the annual award, pro-rated for amount and vesting to the nearest quarter for the time such new Independent Director will serve prior to the Company's next annual meeting of stockholders.

*Stock-based Compensation*

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

	Years Ended December 31,		
	2020	2019	2018
Cost of sales	\$ 26,246	\$ 16,146	\$ 13,558
Research and development	61,942	56,649	57,557
Selling, general and administrative	101,523	87,070	77,704
Total stock-based compensation expense	\$ 189,711	\$ 159,865	\$ 148,819

Stock-based compensation of \$20.1 million, \$20.3 million and \$20.0 million was capitalized into inventory for the years ended December 31, 2020, 2019 and 2018, respectively. Capitalized stock-based compensation is recognized in Cost of Sales when the related product is sold.

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**NOTES TO CONSOLIDATED FINANCIAL STATEMENTS**  
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**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 under the plan for the year ended December 31, 2020:

	Shares	Weighted Average Grant Date Fair Value
Non-vested units as of December 31, 2019	3,585,384	\$ 88.54
Granted	2,190,380	\$ 77.13
Vested	(1,308,962)	\$ 87.57
Forfeited	(293,678)	\$ 85.17
Non-vested units as of December 31, 2020	<u>4,173,124</u>	<u>\$ 83.41</u>

The weighted-average grant date fair value per share of RSUs granted during the years ended December 31, 2020, 2019 and 2018, was \$77.13, \$91.28 and \$84.63, respectively. The total intrinsic value of restricted stock that vested and was released in the years ended December 31, 2020, 2019 and 2018, was \$109.9 million, \$101.0 million and \$84.5 million respectively.

As of December 31, 2020, total unrecognized compensation cost related to unvested RSUs with service-based vesting conditions of \$245.3 million was expected to be recognized over a weighted average period of 2.6 years.

*Restricted Stock Unit Awards with Performance-based Vesting Conditions*

The Compensation Committee of the Board (with respect to awards to certain executive officers other than the Chief Executive Officer) and the Board (with respect to awards to the Chief Executive Officer) may grant RSUs with performance-based vesting conditions (PRSUs) to certain executive officers.

*Revenue PRSUs:* Although no PRSUs with vesting conditions based on revenue performance were granted during the year ended December 31, 2020, awards granted during prior periods presented were contingent upon the achievement of an annual revenue target and the earned RSUs vest over a three-year service period. The number of shares that may be earned ranged between 50% and 200% of the base RSUs, depending on the percentage of the respective annual year's Net Product Revenues, excluding net revenues attributable to Aldurazyme, and determined using fixed foreign currency exchange rates achieved against the target, with a threshold achievement level of 75% of target and a ceiling achievement level of 125% of target.

Below is a summary of activity related to Revenue PRSUs under the Company's equity plan for the year ended December 31, 2020:

	Shares	Weighted Average Grant Date Fair Value
Non-vested units as of December 31, 2019	226,884	\$ 89.09
Adjustment for shares earned	2,272	\$ 73.82
Vested	(126,608)	\$ 88.09
Forfeited	(3,197)	\$ 94.53
Non-vested units as of December 31, 2020	<u>99,351</u>	<u>\$ 90.31</u>

The weighted-average grant date fair value of Revenue PRSUs was \$94.53 and \$83.57 for the years ended December 31, 2019 and 2018, respectively. As of December 31, 2020, total unrecognized compensation expense of \$3.5 million related to Revenue PRSUs was expected to be recognized over a weighted average period of approximately 1.0 year.

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## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

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*Other PRSUs:* Below is a summary of activity related to RSUs with vesting conditions based on other performance targets under the Company's equity plan for the year ended December 31, 2020:

	Shares		Weighted Average Grant Date Fair Value
Non-vested units as of December 31, 2019	44,260	\$	81.00
Granted	184,460	\$	84.17
Vested	(4,495)	\$	73.82
Forfeited	(3,560)	\$	78.72
Non-vested units as of December 31, 2020	220,665	\$	83.83

The weighted-average grant date fair value of Other PRSUs was \$84.17 and \$81.00 for the years ended December 31, 2020 and 2019, respectively.

Non-vested Other PRSUs include a grant with vesting contingent upon the achievement of a three-year Non-GAAP income target and a grant with vesting contingent upon achievement of a three-year strategic goal target. The awarded PRSUs, if any, vest ratably over a three-year service period. The Company evaluated the targets in the context of its current long-range financial plan, its product candidate development pipeline and planned regulatory activity and determined that attainment of each grant target was probable for accounting purposes commencing in the first quarter of 2020. The number of shares that may be earned range between 50% and 200% of the base RSUs.

Also included in non-vested Other PRSUs are grants that vest contingent upon achievement of certain regulatory milestones that, for accounting purposes, were deemed not yet probable of vesting as of December 31, 2020. Therefore, as of December 31, 2020, total unrecognized compensation expense of \$8.1 million related to awards deemed probable of vesting and \$7.9 million related to awards not yet deemed probable of vesting. The expected weighted average period over which expense is to be recognized for the awards that are considered probable of vesting as of December 31, 2020 was 2.2 years.

*Restricted Stock Unit Awards with Market-based Vesting Conditions*

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

Below is a summary of activity related to RSUs with market-based vesting conditions under the Company's equity plan for the year ended December 31, 2020:

	Shares		Weighted Average Grant Date Fair Value
Non-vested units as of December 31, 2019	99,010	\$	143.92
Granted	131,350	\$	115.85
Vested	—	\$	—
Forfeited	(9,370)	\$	143.92
Non-vested units as of December 31, 2020	220,990	\$	127.23

The Company utilized a Monte Carlo simulation model to determine the grant date fair value per base TSR-RSU of \$112.12 using the following assumptions for the grant in March 2020: an expected term of 2.8 years, discount rate of 0.4%, dividend yield of zero and expected volatility rate derived from historical volatilities for the Company and the members of the referenced peer group. In June 2020, the Compensation Committee of the Board approved an additional grant of RSUs with market-based vesting conditions with identical terms as those granted in March 2020. The third-party valuation per base TSR-RSU of \$217.65 used the following assumptions: an expected term of 2.5 years, discount rate of 0.2%, dividend yield of zero and expected volatility rate derived from historical volatilities for the Company and the members of the referenced peer group. As of

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December 31, 2020, total unrecognized compensation expense of \$14.1 million related to base TSR-RSUs was expected to be recognized over a weighted average period of 1.8 years.

**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, 2020. 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 <sup>(1)</sup>
Options outstanding as of December 31, 2019	7,264,235	\$ 67.99		\$ 146,700
Granted	891,610	\$ 76.02		
Exercised	(1,240,511)	\$ 43.87		
Expired and forfeited	(85,897)	\$ 86.07		
Options outstanding as of December 31, 2020	<u>6,829,437</u>	\$ 73.19	4.8	\$ 119,607
Options unvested as of December 31, 2020	1,438,573	\$ 81.56	8.6	\$ 11,635
Exercisable at December 31, 2020	5,390,864	\$ 70.95	3.8	\$ 107,972

- (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 \$87.69, the closing price of the Company's common stock on the Nasdaq Global Select Market on December 31, 2020.

The weighted-average fair value per stock option granted in the years ended December 31, 2020, 2019 and 2018, were \$27.47, \$36.84 and \$33.40, respectively. The total intrinsic value of options exercised during the years ended December 31, 2020, 2019 and 2018, was \$71.9 million, \$32.5 million and \$79.9 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,		
	2020	2019	2018
Expected volatility	36.5 – 42.2%	37.1 – 37.4%	36.8 – 38.4%
Dividend yield	0.00%	0.00%	0.00%
Expected term	4.6 – 5.9 years	4.6 – 5.8 years	4.6 – 5.7 years
Risk-free interest rate	0.3 – 1.7%	2.2 – 3.0%	2.3 – 2.8%

As of December 31, 2020, total unrecognized compensation cost related to unvested stock options of \$35.3 million was expected to be recognized over a weighted average period of 2.5 years. The net tax benefit from stock options exercised during the year ended December 31, 2020 was \$4.5 million.



## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(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,		
	2020	2019	2018
Expected volatility	30.6 – 69.2%	27.7 – 35.0%	29.7% – 35.0%
Dividend yield	0.00%	0.00%	0.00%
Expected term	0.5 – 2.0 years	0.5 – 2.0 years	0.5 – 2.0 years
Risk-free interest rate	0.1 – 2.8%	1.2 – 2.8%	1.2 – 2.8%

As of December 31, 2020, total unrecognized compensation cost related to unvested stock purchase rights under the ESPP of \$18.3 million was expected to be recognized over a weighted average period of 1.4 years.

**(17) OTHER EMPLOYEE BENEFITS*****Employment Agreements***

The Company has entered into employment agreements with certain officers. Generally, these agreements can be terminated without cause by the Company upon prior written notice and payment of specified severance, or by the officer upon four weeks' prior written notice to the Company.

***401(k) Plan***

The Company sponsors the BioMarin Retirement Savings Plan (the 401(k) Plan). Eligible employees may contribute to the 401(k) Plan up to the lesser of 60% of their current compensation or an amount up to a statutorily prescribed annual limit. 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 \$26.4 million, \$28.5 million and \$23.0 million for the years ended December 31, 2020, 2019 and 2018, respectively.

***Deferred Compensation Plan***

The Company amended the NQDC in the second quarter of 2019 to prohibit the diversification of deferrals of Company stock, which resulted in a change to the classification of the obligation associated with the Company's common stock held in the NQDC. 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 and, subsequent to the 2019 amendment, changes in the fair value of Company stock held in the NQDC are no longer recognized in earnings. Other contributions held in the NQDC are classified as trading securities and recorded at fair value with the corresponding deferred compensation obligation classified as a liability. Changes in the fair value of non-BioMarin investments are recognized in earnings in the period they occur.

See Note 10 to these Consolidated Financial Statements for additional discussion on the fair value and presentation of the NQDC assets and liabilities.

**(18) INCOME TAXES**

The benefit from income taxes was based on loss before income taxes as follows:

## BIOMARIN PHARMACEUTICAL INC.

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

	Years Ended December 31		
	2020	2019	2018
U.S. Source	\$ (162,939)	\$ (182,112)	\$ (128,700)
Non-U.S. Source	120,617	87,301	(14,005)
Loss before income taxes	<u>\$ (42,322)</u>	<u>\$ (94,811)</u>	<u>\$ (142,705)</u>

The U.S. and foreign components of the benefit from income taxes were as follows:

	Years Ended December 31,		
	2020	2019	2018
Provision for (benefit from) current income tax expense:			
Federal	\$ (14,758)	\$ 5,127	\$ (2,660)
State and local	1,201	1,331	588
Foreign	1,042	5,339	4,956
	<u>(12,515)</u>	<u>11,797</u>	<u>2,884</u>
Provision for (benefit from) deferred income taxes:			
Federal	(45,038)	(58,311)	(72,074)
State and local	(5,321)	(5,394)	(994)
Foreign	(838,548)	(19,055)	4,690
	<u>(888,907)</u>	<u>(82,760)</u>	<u>(68,378)</u>
Benefit from income taxes	<u>\$ (901,422)</u>	<u>\$ (70,963)</u>	<u>\$ (65,494)</u>

In the third quarter of 2020, the Company completed an intra-entity transfer of certain intellectual property rights to an Irish subsidiary where the Company's ex-U.S. regional headquarters are located and has significant manufacturing and commercial operations, to better align ownership of intellectual property rights with how the business operates. The transaction did not result in a taxable gain; however, the Company's Irish subsidiary recognized a deferred tax asset for the book and tax basis difference of the transferred intellectual property rights. As a result, the Company recognized a deferred tax asset of \$835.1 million and related tax benefit on its Consolidated Financial Statements based on the fair value of the transferred intellectual property rights. The tax deductions related to the amortization of these intangible assets will be recognized in the future and any amortization not deducted for tax purposes will be carried forward indefinitely under Irish tax laws. The Company expects to be able to realize the deferred tax asset resulting from this transaction and has not recorded a valuation allowance as of December 31, 2020.

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**NOTES TO CONSOLIDATED FINANCIAL STATEMENTS - (Continued)**  
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The following is a reconciliation of the statutory federal income tax (benefit) expense to the Company's effective tax rate:

	December 31,		
	2020	2019	2018
Federal statutory income tax benefit	\$ (8,888)	\$ (19,911)	\$ (29,968)
State and local taxes	(3,264)	(2,784)	(276)
Orphan Drug & General Business Credit	(44,114)	(43,124)	(66,451)
Stock compensation expense	(1,101)	239	(5,647)
Changes in the fair value of contingent consideration	—	(1,804)	(2,361)
Foreign Source Income Subject to U.S. Tax	6,266	(52)	6,543
Foreign tax rate differential <sup>(1)</sup>	(16,238)	(30,639)	12,583
Section 162(m) limitation	9,571	8,294	7,440
Tax Reserves	2,166	12,123	8,545
Intra-entity transfer of assets	(852,338)	—	—
CARES Act carryback claim	(2,201)	—	—
Other	1,843	(1,132)	(423)
Valuation allowance/deferred benefit	6,876	7,827	4,521
Effective income tax (benefit) expense	<u>\$ (901,422)</u>	<u>\$ (70,963)</u>	<u>\$ (65,494)</u>

- (1) For the year ended December 31, 2019, the foreign rate differential included foreign local tax expense which was at an effective rate lower than the U.S. statutory rate and was offset by the benefit of the valuation allowance release against the deferred tax assets of the Company's Dutch subsidiary of \$29.6 million.

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

	December 31,	
	2020	2019
<b>Net deferred tax assets:</b>		
Net operating loss carryforwards	\$ 30,718	\$ 32,181
Tax credit carryforwards	532,394	508,560
Accrued expenses, reserves, and prepaids	66,889	61,807
Intangible assets	873,575	29,413
Stock-based compensation	47,011	42,873
Lease liabilities	8,991	11,470
Inventory	32,012	6,290
Other	454	575
Valuation allowance	(93,075)	(86,197)
Total deferred tax assets	<u>1,498,969</u>	<u>606,972</u>
Joint venture basis difference	(1,164)	(993)
Acquired intangibles	(1,364)	(1,647)
Deferred revenue	(1,517)	(3,003)
Convertible notes discount	—	(2,364)
ROU assets	(8,280)	(10,258)
Property, plant and equipment	(54,682)	(46,642)
Total deferred tax liabilities	<u>(67,007)</u>	<u>(64,907)</u>
Net deferred tax assets	<u>\$ 1,431,962</u>	<u>\$ 542,065</u>

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. The valuation allowance increased in 2020 due primarily to the Company's expectation that state R&D credits will not be utilized.

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**NOTES TO CONSOLIDATED FINANCIAL STATEMENTS - (Continued)**  
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As of December 31, 2020, 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	\$ 4,753	2030 – 2033
Federal R&D and orphan drug credit carryforwards	\$ 566,887	2024 – 2040
State net operating loss carryforwards	\$ 182,961	2021 – 2040
Dutch net operating loss carryforwards	\$ 75,718	2022 – 2025

Not included in the table above are \$124.3 million of state research credit carryovers that will carry forward indefinitely.

The Company's net operating losses and credits could be subject to annual limitations due to ownership change limitations provided by IRC 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, 2020 and 2019, is as follows:

	December 31,	
	2020	2019
Balance at beginning of period	\$ 168,748	\$ 147,445
Additions based on tax positions related to the current year	16,481	19,287
(Deletions) Additions for tax positions of prior years	(2,527)	2,016
Lapse of statute of limitations	(138)	—
Balance at end of period	\$ 182,564	\$ 168,748

Included in the balance of unrecognized tax benefits as of December 31, 2020 were potential benefits of \$175.1 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, 2020. 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 2014 and earlier may still be adjusted upon examination by tax authorities.

U.S. income and foreign withholding taxes have not been recognized on the excess of the amount for financial reporting over the tax basis of investments in foreign subsidiaries that are essentially permanent in duration. This excess totaled approximately \$15.2 million as of December 31, 2020, which will be indefinitely reinvested; deferred income taxes have not been provided on such foreign earnings.

#### **(19) NET INCOME (LOSS) PER COMMON SHARE**

Potentially issuable shares of common stock include shares issuable upon the exercise of outstanding employee stock option awards, common stock issuable under the Company's ESPP, unvested RSUs, the Company's common stock held by the NQDC and contingent issuances of common stock related to the Company's convertible debt.

**BIOMARIN PHARMACEUTICAL INC.**
**NOTES TO CONSOLIDATED FINANCIAL STATEMENTS - (Continued)**  
(In thousands of U.S. Dollars, except per share amounts or as otherwise disclosed)

The following table sets forth the computation of basic and diluted income (loss) per common share (common shares in thousands):

	Years Ended December 31,		
	2020	2019	2018
<b>Numerator:</b>			
Net income (loss), basic	\$ 859,100	\$ (23,848)	\$ (77,211)
Add: Interest on convertible notes	8,313	—	—
Less: gain on the Company's common stock held by the NQDC	—	—	(710)
Net income (loss), diluted	<u>\$ 867,413</u>	<u>\$ (23,848)</u>	<u>\$ (77,921)</u>
<b>Denominator:</b>			
Weighted-average common shares outstanding, basic	180,804	179,039	177,061
Effect of dilutive securities:			
Options to purchase common stock	1,543	—	—
Common stock issuable under the 2027 notes	2,874	—	—
Common stock issuable under the 2024 notes	3,970	—	—
Unvested RSUs	1,938	—	—
Common stock potentially issuable for ESPP purchases	353	—	—
The Company's common stock held by the NQDC	196	—	207
Weighted-average common shares outstanding, diluted	<u>191,678</u>	<u>179,039</u>	<u>177,268</u>
Net income (loss) per common share, basic	<u>\$ 4.75</u>	<u>\$ (0.13)</u>	<u>\$ (0.44)</u>
Net income (loss) per common share, diluted	<u>\$ 4.53</u>	<u>\$ (0.13)</u>	<u>\$ (0.44)</u>

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 basic and diluted income (loss) per common share as they were anti-dilutive (in thousands):

	Years Ended December 31,		
	2020	2019	2018
Options to purchase common stock	5,287	7,264	7,364
Common stock issuable under the 2020 Notes	—	3,983	3,983
Common stock issuable under the 2024 Notes	—	3,970	3,970
Unvested RSUs	2,235	3,956	3,404
Common stock potentially issuable for ESPP purchases	314	587	435
The Company's common stock held by the NQDC	—	205	—
Total number of potentially issuable shares	<u>7,836</u>	<u>19,965</u>	<u>19,156</u>

The potential effect of the capped call transactions with respect to the 2020 Notes was excluded from the diluted net income (loss) per share as the Company's closing stock price on December 31, 2019 and 2018 did not exceed the conversion price of \$94.15 per share for the 2020 Notes. There is no similar capped call transaction associated with the 2024 Notes or 2027 Notes. See Note 13 to these Consolidated Financial Statements for information on the Company's convertible debt.

**(20) LICENSE AND COLLABORATION AGREEMENTS**

In October 2019, the Company entered into a worldwide, exclusive licensing agreement with a third party for tralesenidase alfa (formerly referred to as BMN 250), an investigational enzyme replacement therapy to treat Sanfilippo Syndrome Type B. In consideration, the Company received an upfront payment of \$3.0 million, a minority 15% equity ownership interest in the licensee,

## BIOMARIN PHARMACEUTICAL INC.

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

and is entitled to receive royalties on net sales of tralesenidase alfa and milestone payments if certain development, regulatory and sales milestones are met by the licensee.

The Company evaluated the design and purpose of the third-party licensee and determined that it is a variable interest entity (VIE), as the equity-at-risk is insufficient to support the licensee's operations. The Company has concluded that it is not the primary beneficiary of the VIE as the Company does not have the power to direct the activities of the VIE that most significantly impact its performance. The Company is accounting for the minority equity investment at cost, less impairment, if any, adjusted for observable price changes, as it does not exercise significant influence over the operations of the licensee. Other than providing the licensee with specified transition services, the Company has no other involvement with the operations of the VIE as of December 31, 2020. As a result, the Company's loss exposure is limited to the value of the equity investment of \$8.1 million which is included in Other Assets on the Company's Consolidated Balance Sheets as of December 31, 2020.

In July 2017, the Company executed a license agreement and a settlement agreement with Sarepta Therapeutics (Sarepta) that provide Sarepta with global exclusive rights to the Company's Duchenne muscular dystrophy (DMD) patent estate for EXONDYS 51 and all future exon-skipping products. Under these agreements, Sarepta may pay certain additional regulatory and commercial milestone fees for exons 51, 45, 53 and possibly on future exon-skipping products to the Company if certain development and sales milestones are achieved. Additionally, the Company receives from Sarepta royalties based on 5% of net sales in the U.S. through the end of 2023 and 8% of net sales in the EU and in other countries, where certain of the Company's patents exist, through September 30, 2024. The Company retained the right to convert the license to a co-exclusive right in the event it decides to proceed with an exon-skipping therapy for DMD.

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 A&R Kuvan Agreement, if future sales milestones are met, the Company is obligated to pay Merck Serono up to a maximum of €60.0 million, in cash, which was an estimated fair value of \$73.6 million as of December 31, 2020. Pursuant to the Pegvaliase Agreement, the Company paid Merck Serono €125.0 million in cash when the Palynziq development milestones were achieved.

In October 2012, the Company licensed to Catalyst Pharmaceutical Partners, Inc. (Catalyst) the North American rights to develop and market Firdapse, the Company's commercial product for the treatment of Lambert-Eaton myasthenic syndrome. In exchange for the North American rights to Firdapse, commencing in the first quarter of 2019 the Company receives royalties of 7% to 10% on net product sales of Firdapse in North America. In January 2020, the Company completed the sale of worldwide rights to Firdapse to a third party. The Company retained the rights to receive the royalties from Catalyst. See Note 6 to these Consolidated Financial Statements for further information about the Firdapse sale.

In September 2007, the Company licensed to Asubio Pharma Co., Ltd. (a subsidiary of Daiichi Sankyo) exclusive rights to data and intellectual property contained in the Kuvan new drug application. The Company receives royalties on net sales of the product in Japan.

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.

**(21) 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.

***Contingent Payments***

As of December 31, 2020, the Company was subject to contingent payments totaling approximately \$662.8 million upon achievement of certain development and regulatory activities and commercial sales milestones if they occur before certain dates in

## BIOMARIN PHARMACEUTICAL INC.

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

the future. Of this amount, \$235.0 million related to an early stage development programs licensed from a third party in the second quarter of 2020, \$73.6 million related to the acquisition of certain rights and other assets with respect to Kuvan and Palynziq from Merck Serono and \$237.7 million related to programs that are no longer being developed.

As of December 31, 2020, the Company recorded \$60.1 million of contingent liabilities on its Consolidated Balance Sheet, all of which was long-term. See Note 3 and Note 10 to these Consolidated Financial Statements for further information regarding the fair value of the Company's contingent consideration.

***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 related to active pharmaceutical ingredients, certain inventory related items and certain third-party R&D services. As of December 31, 2020, such commitments and other minimum contractual obligations for clinical and post-marketing services were estimated at approximately \$142.8 million. The Company has also licensed technology, for which it is required to pay royalties upon future sales, subject to certain annual minimums.

Under certain of the Company's lease agreements, the Company is contractually obligated to return leased space to its original condition upon termination of the applicable lease agreement. The Company records interest expense to accrete the asset retirement obligation liability to full value and depreciates each retirement obligation asset, both over the term of the associated lease agreement. As of December 31, 2020 and 2019, the balance of the asset retirement obligation liability was \$3.7 million and \$3.0 million, respectively. See Note 3 to these Consolidated Financial Statements for further information on the Company's fair value measurements.

**PART-TIME EMPLOYMENT AGREEMENT**

THIS PART-TIME EMPLOYMENT AGREEMENT ("**Agreement**") is made effective as of January 1, 2021 ("**Effective Date**") by and between BioMarin Pharmaceutical Inc., a Delaware corporation (the "**Company**") and Robert A Baffi, Ph.D. ("**Employee**").

WHEREAS, Employee currently serves as Senior Advisor to the Company, a full-time position, pursuant to the Amended and Restated Employment Agreement by and between the Company and Employee, dated June 10, 2015 (the "**2015 Employment Agreement**");

WHEREAS, Employee desires to retire from his full-time position with the Company and terminate the 2015 Employment Agreement; and

WHEREAS, the Company desires to continue to employ Employee on a part-time basis as set forth below, and Employee desires to accept such part-time employment with the Company.

NOW THEREFORE, for good and valuable consideration (the receipt and adequacy of which are hereby acknowledged and agreed) the parties hereby covenant and agree as follows:

1. Termination of the 2015 Employment Agreement. As of the Effective Date, the 2015 Employment Agreement shall terminate pursuant to Employee's election under Section 6(a) of the 2015 Employment Agreement (At-Will Employment). Employee shall not be entitled to the Severance Benefits (as such term is defined in the 2015 Employment Agreement) or any other compensation or benefits under the 2015 Employment Agreement.

2. Title; Duties. The Company hereby employs the Employee on a part-time basis as Senior Advisor ("**Title**") to perform such duties consistent with his Title and position as may be determined and assigned to Employee by the Company's Chief Executive Officer ("**CEO**"), including, without limitation, the following duties:

(a) consult the Company's senior executives, including without limitation, the Company's CEO; Executive Vice President, Chief Technical Officer ("**CTO**"); President, Worldwide Research & Development ("**President, WWRD**"); and others on matters that you may be asked to provide strategic input;

(b) assist with specific internal projects at the request of the Company's CEO CTO, or President, WWRD;

(c) advise the Company regarding business development opportunities and transactions as directed by the CEO or Group Vice President, Business Development;

(d) at the request of the Company's CEO, CTO or the President, WWRD, participate in industry and regulatory groups and advisory committees; and

(e) prepare a written history of the Company's Technical Operations (TOPS) department and gene therapy manufacturing efforts for future publication by the Company.

The Employee may generally perform his duties remotely from his home office in Florida, provided that Employee acknowledges that his job duties may require occasional travel depending on the tasks involved.

3. Time and Effort. The Employee agrees to devote a reasonably appropriate amount of his professional employment time and effort to the performance of his duties as Title and to perform such other duties consistent with his Title and position as are reasonably assigned Employee from time to time by the CEO.

4. Term. The Company agrees to employ the Employee in accordance with the terms of this Agreement, which terms shall be effective commencing on the Effective Date and continuing thereafter until December 31, 2021 or earlier if terminated pursuant to **Section 6** hereof (the "**Term**").

5. Compensation; Benefits.



(a) Base Salary. For all the services to be rendered by the Employee in any capacity hereunder, the Company agrees to pay the Employee a base salary ("**Base Salary**") of two hundred thousand dollars (\$200,000.00) per annum. Base Salary shall be payable in approximately equal installments in accordance with the Company's customary payroll practices. The foregoing annual compensation amount may be, from time to time, adjusted above the Base Salary specified above by action of the CEO. In the event the Base Salary is adjusted upward by the CEO, such adjusted amount will be deemed to be the new Base Salary.

(b) Annual Bonus. The Employee shall not be entitled to participate in the Company's generally applicable employee bonus program.

(c) ESPP; Stock Options and Restricted Stock Units. Employee can continue to participate in the Company's Employee Stock Purchase Plan (ESPP) in accordance with the Company's customary practices. Employee was previously granted options to purchase certain shares of the Company's common stock and certain restricted stock units ("**RSUs**"). Nothing herein shall affect any stock options or RSUs previously granted to the Employee which shall continue to be governed by the applicable agreements and governing equity plans.

(d) Benefits Plans. During the Term, the Employee and his spouse shall continue to participate fully in all insurance benefit plans and programs (including, without limitation, medical, dental and vision insurance) pursuant to the terms of such plans or programs. The Company shall pay the premiums for such insurance benefit plans and programs on behalf of the Employee and his spouse. The Employee shall be responsible for any taxes on such insurance premium payments.

(e) Vacation. The Employee shall not be entitled to annual paid vacation time.

(f) Expenses. The Company shall reimburse the Employee for all reasonable and customary travel, business and entertainment expenses incurred in connection with the Employee's performance of his services hereunder in accordance with the policies and procedures established by the Company and paid promptly after the Employee makes a request therefore and no later than the end of the calendar year following the calendar year in which the expenses were incurred by the Employee.

(g) Withholding. The amounts payable pursuant to this Agreement (including insurance premium payments) shall be subject to withholding for appropriate taxes, assessments or withholdings as required by applicable law.

#### 6. At-Will Employment; Termination of Employment.

(a) At-Will Employment. Employee's employment with the Company under this Agreement is employment "at will." The Employee may terminate Employee's employment with Company at any time and for any reason whatsoever (or no reason) simply by notifying the Company. Likewise, the Company may terminate Employee's employment at any time, with or without Cause (as such term is defined in the 2015 Employment Agreement), and with or without advance notice.

(b) Employment Termination. In the event of a termination of Employee's employment at any time by the Employee for any reason whatsoever (or no reason) or by the Company for Cause, the Company shall be obligated only to pay the Employee the compensation due Employee up to the date of termination, all accrued, vested or earned benefits under any applicable benefit plan, and any other compensation to which the Employee is entitled under **Section 5** up to and ending on the date of the Employee's termination. If the Company terminates Employee's employment without Cause (including in connection with a Change in Control (as such term is defined in the 2015 Employment Agreement)), then the Company shall be obligated to pay the Employee any unpaid Base Salary and the amount of unpaid insurance premium payments that would have been paid had the Employee's employment not been terminated by the Company.

(c) Employee's Disability. The Company shall be entitled, by providing written notice to the Employee, to terminate the Employee's employment under this Agreement if the Employee shall become Disabled (as defined herein). Any delay or forbearance by the Company in exercising any such right to terminate this Agreement shall not constitute a waiver thereof. "**Disabled**" means such that Employee is unable to carry out his duties hereunder for four (4) consecutive calendar months or for a period aggregating one hundred twenty (120) days in any period of twelve (12) consecutive calendar months because of a physical or mental impairment.

(d) Employee's Death. The Employee's employment will immediately terminate upon the death of the Employee. The Employee's surviving designated beneficiary, or, if none, the Employee's estate, shall be entitled to receive the compensation due the Employee up to the date of the Employee's death, all accrued, vested or earned benefits under any applicable benefit plan and any other compensation to which the Employee is entitled under this Agreement up to and ending on the date of the Employee's death.

7. Compliance with Code Section 409A. If any amounts or benefits payable under this Agreement on account of Employee's termination of employment constitute deferred compensation subject to Section 409A of the Code, no payments or benefits shall be paid or provided until Employee incurs a separation from service within the meaning of Treas. Reg. § 1.409A-1(h) from the Company and any entity that would be considered a single employer with the Company under Code Sections 414(b) or 414(c) ("**Separation from Service**"). If, at the time of Employee's Separation from Service, the Employee is a "specified employee" (within the meaning of Code Section 409A and Treas. Reg. §1.409A-3(i)(2)), the Company will not pay or provide any "Specified Benefits" (as defined herein) during the six-month period (the "409A Suspension Period") beginning immediately after the Employee's Separation from Service. For purposes of this Agreement, "Specified Benefits" are any amounts or benefits that would be subject to Code Section 409A penalties if the Company were to pay them, pursuant to this Agreement, on account of the Employee's Separation from Service.

This Agreement is intended to comply with (or be exempt from) Code Section 409A, and the Company shall have complete discretion to interpret and construe this Agreement and any associated documents in any manner that establishes an exemption from (or otherwise conforms them to) the requirements of Code Section 409A. If, for any reason including imprecision in drafting, the Agreement does not accurately reflect its intended establishment of an exemption from (or compliance with) Code Section 409A, as demonstrated by consistent interpretations or other evidence of intent, the provision shall be considered ambiguous and shall be interpreted by the Company in a fashion consistent herewith, as determined in the sole and absolute discretion of the Company. The Company reserves the right to unilaterally amend this Agreement without the consent of the Employee in order to accurately reflect its correct interpretation and operation, as well as to maintain an exemption from or compliance with Code Section 409A. Nevertheless, and notwithstanding any other provision of this Agreement, neither the Company nor any of its employees, directors, or their agents shall have any obligation to mitigate, nor to hold the Employee harmless from, any or all taxes (including any imposed under Code Section 409A) arising under this Agreement.

8. Choice of Law; Venue. This Agreement shall be construed and performed in accordance to the laws (but not the conflicts of laws) of the State of California. Venue of any proceeding shall be exclusively in the County of Marin in the foregoing state, and both parties consent and agree to such exclusive venue.

9. Arbitration. To ensure rapid and economical resolution of any disputes which may arise under this Agreement, Employee and the Company agree that any and all claims, disputes or controversies of any nature whatsoever arising from or regarding the interpretation, performance, negotiation, execution, enforcement or breach of this Agreement, Employee's employment with the Company, or the termination of Employee's employment from the Company, including but not limited to statutory claims, shall be resolved by confidential, final and binding arbitration conducted before a single arbitrator with JAMS, Inc. ("**JAMS**") in San Francisco, California, in accordance with JAMS' then-applicable arbitration rules, which can be found at <http://www.jamsadr.com/rules-clauses/>, and which will be provided to Employee upon request. **The parties acknowledge that by agreeing to this arbitration procedure, they waive the right to resolve any such dispute through a trial by jury, judge or administrative proceeding.** Employee will have the right to be represented by legal counsel at any arbitration proceeding. The arbitrator shall: (a) have the authority to compel adequate discovery for the resolution of the dispute and to award such relief as would otherwise be available under applicable law in a court proceeding; and (b) issue a written statement signed by the arbitrator regarding the disposition of each claim and the relief, if any, awarded as to each claim, the reasons for the award, and the arbitrator's essential findings and conclusions on which the award is based. Nothing in this Agreement shall prevent either Employee or the Company from obtaining injunctive relief in court to prevent irreparable harm pending the conclusion of any such arbitration. The Company shall pay all filing fees in excess of those which would be required if the dispute were decided in a court of law, and shall pay the arbitrator's fees and any other fees or costs unique to arbitration. Any awards or orders in such arbitrations may be entered and enforced as judgments in the federal and state courts of any competent jurisdiction.

10. Notices. All notices provided for or permitted to be given pursuant to this Agreement must be in writing. All notices shall be given to the other party by personal delivery, overnight courier (with receipt signature), or facsimile transmission (with "answerback" confirmation of transmission), to the Company or the Employee at the Company's principal executive offices if to the Company or to the residential address of the Employee as contained in

Employee's personnel file if to Employee. Each such notice shall be deemed effective upon the date of actual receipt in the case of personal delivery, receipt signature in the case of overnight courier, or confirmation of transmission in the case of facsimile.

11. Entire Agreement; Amendment. This Agreement contains the sole and entire agreement of the parties and supersedes all prior agreements and understandings between the Employee and the Company and cannot be modified or changed by any oral or verbal promise or statement by whomsoever made; nor shall any written modification of it be binding upon the Company until such written modification shall have been approved in writing by the CEO.

12. Waiver; Consent. In the event any term or condition contained in this Agreement should be breached by any party and thereafter waived or consented to by the other party, which waiver or consent must be effectuated by a written instrument signed by the party against whom any waiver or consent is sought (and, in the case of the Company, approved by the CEO), such waiver or consent shall be limited to the particular breach so waived or consented to and shall not be deemed to waive or consent to any other breach occurring prior or subsequent to the breach so waived or consented to.

13. Severability. If any provisions of this Agreement or the application thereof to any person or circumstances shall be invalid or unenforceable to any extent, the remainder of this Agreement and the application of such provisions to other persons or circumstances shall not be affected thereby and shall be enforced to the extent permitted by law.

14. Survival. The provisions hereof which are to be performed or observed after the termination of this Agreement, and the representations, covenants and agreements of the parties contained herein with respect thereto shall survive the termination of this Agreement and be effective according to their terms.

15. Successors. All of the terms and provisions of this Agreement shall be binding upon and shall inure to the benefit of and be enforceable by and against the parties to this Agreement and the respective heirs, executors, and successors in interest; provided, however, that the duties of the Employee hereunder are personal in nature and may not be delegated without a written consent of the Company.

16. Assignment. This Agreement and the rights and benefits contained herein may not be assigned by either party hereto, except by the Company in connection with a merger, consolidation, share exchange, business combination or other reorganization of the Company or a sale of all or substantially all of the Company's business or assets.

17. Certain Representations, Covenants and Acknowledgements.

(a) The Employee represents that Employee is not subject to any employment, confidentiality, or other agreement or restriction that would prevent Employee from fully satisfying his duties under this Agreement or that would be violated if Employee did so.

(b) Without the Company's prior written approval, the Employee agrees not to: (i) disclose proprietary information belonging to a former employer or other entity without its written permission; (ii) contact any former employer's customers or employees to solicit their business or employment on behalf of the Company; or (iii) distribute announcements about or otherwise publicize his employment with the Company.

(c) The Employee acknowledges that Employee is free to seek advice from independent counsel with respect to this Agreement. The Employee is not relying on any representation or advice from the Company or any of its officers, directors, attorneys or other representatives regarding this Agreement, its content or effect.

18. Construction. The masculine pronoun, wherever used herein, shall be construed to include the feminine and the neuter, where appropriate. The singular form, wherever used herein, shall be construed to include the plural, where appropriate.

19. Drafting. The parties represent and acknowledge that they both have participated in the preparation and drafting of this Agreement and have each given their approval to all of the language contained in this

Agreement, and it is expressly agreed and acknowledged that if either party later claims that there is an ambiguity in the language of this Agreement, there shall be no presumption that such ambiguity be construed for or against either party hereto.

20. Counterparts. This Agreement may be executed and delivered (including by facsimile transmission) in one or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

IN WITNESS WHEREOF, the parties to this Agreement have executed this Agreement as of the date indicated above.

**BIOMARIN PHARMACEUTICAL INC.**

By: /s/ Jean-Jacques Bienaimé  
Name: Jean-Jacques Bienaimé

Its: Chief Executive Officer

**EMPLOYEE**

By: /s/ Robert A. Baffi, Ph.D.

Robert A. Baffi, Ph.D.

## Subsidiaries of BioMarin Pharmaceutical Inc. as of December 31, 2020

<b>Name</b>	<b>Direct Parent</b>	<b>Ownership</b>	<b>Jurisdiction of Incorporation</b>
BioMarin Commercial Ltd	BioMarin Pharmaceutical Inc.	100%	Ireland
BioMarin International Holdings Inc	BioMarin Pharmaceutical Inc.	100%	Delaware
BioMarin International Ltd	BioMarin Commercial Ltd.	100%	Ireland

**Consent of Independent Registered Public Accounting Firm**

The Board of Directors  
BioMarin Pharmaceutical Inc.:

We consent to the incorporation by reference in the registration statements (Nos. 333-136963, 333-168552, 333-181697, 333-188620, 333-197759, 333-201504, 333-206094, 333-218695, and 333-234231) on Forms S-8 and in the registration statement (No. 333-212974) on Form S-3 of BioMarin Pharmaceutical Inc. of our reports dated February 26, 2021, with respect to the consolidated balance sheets of BioMarin Pharmaceutical Inc. and subsidiaries as of December 31, 2020 and 2019, the related consolidated statements of operations, comprehensive income (loss), stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2020, and the related notes, and the effectiveness of internal control over financial reporting as of December 31, 2020, which reports appear in the December 31, 2020 annual report on Form 10-K of BioMarin Pharmaceutical Inc.

Our report refers to a change in accounting for leases as of January 1, 2019 due to the adoption of Financial Accounting Standards Board Accounting Standards Codification Topic 842, *Leases*.

/s/ KPMG LLP  
San Francisco, California  
February 26, 2021

## CERTIFICATION

I, Jean-Jacques Bienaimé, certify that:

1. I have reviewed this Annual Report on Form 10-K of BioMarin Pharmaceutical Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a) designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 26, 2021

/S/ JEAN-JACQUES BIENAIMÉ

Jean-Jacques Bienaimé  
Chief Executive Officer



## CERTIFICATION

I, Brian R. Mueller certify that:

1. I have reviewed this Annual Report on Form 10-K of BioMarin Pharmaceutical Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a) designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 26, 2021

/S/ BRIAN R. MUELLER

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Brian R. Mueller  
Executive Vice President, Chief Financial Officer

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

In connection with the Annual Report on Form 10-K of BioMarin Pharmaceutical Inc. (the Company) for the year ended December 31, 2020 as filed with the Securities and Exchange Commission on the date hereof (the Report), we, Jean-Jacques Bienaimé, and Brian R. Mueller, hereby certify, pursuant to 18 U.S.C. §1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002, that:

- (1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/S/ JEAN-JACQUES BIENAIMÉ

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Jean-Jacques Bienaimé  
Chief Executive Officer  
February 26, 2021

/S/ BRIAN R. MUELLER

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Brian R. Mueller  
Executive Vice President, Chief Financial Officer  
February 26, 2021

This certification accompanies the Form 10-K to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of BioMarin Pharmaceutical Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-K), irrespective of any general incorporation language contained in such filing.