

BIOMARIN PHARMACEUTICAL INC  
Form 10-K  
February 26, 2018

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, 2017

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 of other jurisdiction of incorporation or organization)	68-0397820 (I.R.S. Employer Identification No.)
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770 Lindaro Street San Rafael, California (Address of principal executive offices)	94901 (Zip Code)
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Registrant's telephone number, including area code: (415) 506-6700

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Securities registered pursuant to Section 12(b) of the Act:

Title of Each Class	Name of Each Exchange on Which Registered
Common Stock, \$.001 par value	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 and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted 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 and post such files). Yes No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§ 229.405 of this chapter) is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See 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
(Do not check if a smaller reporting company)	Emerging Growth company

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

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the 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, 2017 was \$8.5 billion, based on the closing price reported for such date on the Nasdaq Global Select Market.

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As of February 13, 2018, the registrant had 176,072,261 shares of common stock, par value \$0.001, outstanding.

The documents incorporated by reference are as follows: portions of the Registrant's Proxy Statement for its 2018 annual meeting of stockholders, are incorporated by reference into Part III.

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## BIOMARIN PHARMACEUTICAL INC.

## 2017 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®, Firdapse®, Kuvan®, Naglazyme® and Vimizim® are our registered trademarks. Kyndrisa™ is our trademark. 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 II, 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.

## 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 therapy portfolio consists of six commercial products and multiple clinical and pre-clinical product candidates. Our commercial products are Aldurazyme (laronidase) for Mucopolysaccharidosis I (MPS I), Brineura (cerliponase alfa) for the treatment of late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), Firdapse (amifampridine phosphate) for Lambert Eaton Myasthenic Syndrome (LEMS), Kuvan (sapropterin dihydrochloride) for phenylketonuria (PKU), Naglazyme (galsulfase) for Mucopolysaccharidosis VI (MPS VI) and Vimizim (elosulfase alpha) for Mucopolysaccharidosis IV Type A (MPS IV A).

We continue to invest in our clinical and pre-clinical product pipeline by committing significant resources to research and development programs and business development opportunities within our areas of scientific, manufacturing and technical expertise. We are conducting clinical trials on several product candidates for the treatment of various diseases. Our major product candidates in development include pegvaliase, an enzyme substitution therapy for the treatment of phenylketonuria (PKU); vosoritide, a peptide therapeutic for the treatment of achondroplasia, the most common form of disproportionate short stature in humans; valoctocogene roxaparvovec (formerly referred to as BMN 270), an AAV5 vector and factor VIII gene therapy drug development candidate, for the treatment of severe hemophilia A; BMN 250, an investigational enzyme replacement therapy using a novel fusion of recombinant human alpha-N-acetylglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo Syndrome Type B, or mucopolysaccharidosis type IIIB (MPS IIIB); and BMN 290, a selective chromatin modulation therapy, for the treatment of Friedreich's ataxia. We are conducting or planning to conduct preclinical development of several other product candidates for genetic and other metabolic diseases.

#### Recent Developments

##### FDA Regulatory Review of Pegvaliase

On December 22, 2017, we announced that the United States Food and Drug Administration (FDA) will require additional time to complete its review of our Biologics License Application (BLA) for pegvaliase, a PEGylated recombinant phenylalanine ammonia-lyase enzyme product to reduce blood phenylalanine (Phe) levels in adult patients with PKU who have uncontrolled blood Phe levels on existing management. The Prescription Drug User Fee Act (PDUFA) target action date for pegvaliase has been extended by three months to May 28, 2018, which was changed to May 25, 2018 due to the Memorial Day holiday.

##### Gene Therapy Product Candidate Valoctocogene Roxaparvovec for the Treatment of Hemophilia A

On December 19, 2017, we announced that we had dosed the first patient in the global GENER8-1 Phase 3 study with the 6e13 vg/kg dose for valoctocogene roxaparvovec, an investigational gene therapy for the treatment of patients with severe hemophilia A. This is the first of two Phase 3 studies in the global Phase 3 program to dose a patient. The global Phase 3 program includes two studies with valoctocogene roxaparvovec, one with the 6e13 vg/kg dose (GENER8-1) and one with the 4e13 vg/kg dose (GENER8-2). Both Phase 3 GENER8 studies will be open-label single-arm studies to evaluate the efficacy and safety of valoctocogene roxaparvovec. We plan to enroll the first

patient in GENEr8-2 in early 2018. The primary endpoint in both studies will be based on the factor VIII activity level achieved following treatment with valoctocogene roxaparvovec, and the secondary endpoints will measure annualized factor VIII replacement therapy use rate and annualized bleed rate. We also plan to conduct a Phase 1/2 Study with the 6e13kg/vg dose and with approximately 10 patients who are AAV5 positive. The first patient is expected to enroll in the first half of 2018.

On December 9, 2017, we announced an update to our previously reported results of an open-label Phase 1/2 study of valoctocogene roxaparvovec. The updated results were presented during an oral presentation at the

59th American Society of Hematology (ASH) Annual Meeting and Exposition by John Pasi, M.B. Ch.B., Ph.D. from Barts and The London School of Medicine and Dentistry and primary investigator for the Phase 1/2 study and included the presentation of data, which included sustained normal or near-normal factor VIII levels in severe hemophilia A for most patients with a maximum follow-up of 19 months. The data presented at ASH had a cut off of November 16, 2017 and included 78 weeks of data for the 6e13 vg/kg dose and 48 weeks of data for the 4e13 vg/kg dose.

In October 2017 we announced that valoctocogene roxaparvovec had been granted Breakthrough Therapy Designation from the FDA. The designation is intended to expedite the development and review of medicines to treat a serious disease and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies. Earlier in the year, the European Medicines Agency (EMA) granted access to its Priority Medicines (PRIME) regulatory initiative for valoctocogene roxaparvovec. To be accepted for PRIME, an investigational therapy has to show its potential to benefit patients with unmet medical needs based on early clinical data.

#### Sale of Priority Review Voucher

In December 2017, we sold a Rare Pediatric Disease Priority Review Voucher to Novartis Pharma AG for a lump sum payment of \$125.0 million. We received the voucher under an FDA program intended to encourage the development of treatments for rare pediatric diseases. We were awarded the voucher in April 2017 when we received approval from the FDA of Brineura for the treatment of CLN2.

#### Selection of BMN 290 for Friedreich's Ataxia

In October 2017 we announced that we had selected BMN 290, a selective chromatin modulation therapy intended for treatment of Friedreich's ataxia, a rare autosomal recessive disorder that results in disabling neurologic and cardiac progressive decline. We expect to submit to the FDA an Investigational New Drug (IND) application in the second half of 2018.

#### Product Candidate Vosoritide for the Treatment of Achondroplasia

In October 2017, we provided an update on the open-label Phase 2 study of vosoritide, an analog of C-type Natriuretic Peptide (CNP), in children with achondroplasia, the most common form of disproportionate short stature in humans. Vosoritide for achondroplasia has demonstrated sustained increase in average growth velocity over 30 months of treatment in 10 children, who completed 30 months of daily dosing at 15 µg/kg/day. Over this period of time, patients have experienced mean absolute growth increase of approximately 4 cm over what their baseline growth velocity would have predicted. The sustained increase in annualized growth velocity was accompanied by sustained improvements over time in height compared to age- and gender-matched unaffected children as measured by z-scores. In addition, treatment with vosoritide shows continued improvement over time in proportionality as measured by a ratio of the upper and lower body measurements, or U/L ratio.



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Our global Phase 3 randomized, placebo-controlled study of vosoritide in approximately 110 children with achondroplasia ages 5-14 for 52 weeks also continued in 2017. The study will be followed by a subsequent open-label extension. Children in this study will have completed a minimum six-month baseline study to determine their respective baseline growth velocity prior to entering the Phase 3 study. Vosoritide is being tested in children in the age range where their growth plates are still open. This is approximately 25% of people with achondroplasia. We expect to complete enrollment of the Phase 3 study in mid-2018 and provide top-line data in the second half of 2019.

Summary of Commercial Products and Development Programs

A summary of our commercial products and major development programs, including key metrics as of December 31, 2017, 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>	2017 Total Net Product Revenues (in millions)	2017 Research & Development Expense (in millions)
Aldurazyme	MPS I	Expired	Expired	Expired	\$ 90.0	<sup>(3)</sup> \$ 3.6
Brineura	CLN2	2024	2029	2027	\$ 8.6	\$ 52.0
Firdapse	LEMS	NA <sup>(4)</sup>	NA <sup>(4)</sup>	2019	\$ 18.8	\$ 2.8
Kuvan	PKU	Expired	NA	2020 <sup>(5)</sup>	\$ 407.5	\$ 28.4
Naglazyme	MPS VI	Expired	Expired	Expired	\$ 332.2	\$ 12.5
Vimizim	MPS IV A	2021	2026	2024	\$ 413.3	\$ 24.8

  

Major Product Candidates in Development	Target Indication	United States Orphan Designation	European Union Orphan Designation	Stage	2017 Research & Development Expense (in millions)
Pegvaliase	PKU	Yes	Yes	U.S. marketing authorization regulatory review <sup>(6)</sup>	\$ 122.1
Valoctocogene roxaparvovec	Hemophilia A	Yes	Yes	Clinical Phase 3	\$ 118.2
Vosoritide	Achondroplasia	Yes	Yes	Clinical Phase 3	\$ 55.1
BMN 250	MPS IIIB	Yes	Yes	Clinical Phase 1/2	\$ 56.0
BMN 290	Friedreich's Ataxia	NA	NA	Preclinical <sup>(7)</sup>	\$ 2.8

(1) See “Government Regulation—Orphan Drug Designation” below for further discussion.

(2) See “Government Regulation—Healthcare Reform” below for further discussion.

(3) The Aldurazyme total net product revenues noted above are the total net product revenues recognized by us in accordance with the terms of our agreement with Genzyme Corporation (Genzyme). See “Major Commercial Products—Aldurazyme” below for further discussion.

(4)

Firdapse has not received marketing approval in the United States. We have licensed the North American rights to develop and market Firdapse to a third party.

- (5) Kuvan has been granted orphan drug status in the European Union (EU), which together with pediatric exclusivity, confers 12 years of market exclusivity in the EU that expires in 2020.
- (6) We plan to submit a Marketing Authorization Application (MAA) to the EMA in the first quarter of 2018.
- (7) We plan to submit an IND for BMN 290 in the second half of 2018.

See “Patents and Proprietary Rights” below for additional information on our market protection.

## Major Commercial Products

### Aldurazyme

Aldurazyme is approved for marketing in the United States (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, a lysosomal enzyme normally required for the breakdown of glycosaminoglycans (GAGs). 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 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. We recognize a portion of this amount as product transfer revenue when the product is shipped and released to Genzyme because all of our performance obligations are fulfilled at that point and title to, and risk of loss for, the product has transferred to Genzyme. The product transfer revenue represents the fixed amount per unit of Aldurazyme that Genzyme is required to pay us if the product is unsold by Genzyme. The amount of product transfer revenue will eventually be deducted from the revenues earned by us calculated based on Genzyme's net sales recognized when the product is sold by Genzyme. Additionally, 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

Aldurazyme net product revenues for the years ended December 31, 2017, 2016 and 2015 totaled \$90.0 million, \$93.8 million and \$98.0 million, respectively. On January 1, 2018, we will adopt Accounting Standards Update No. 2014-09, Revenue from Contracts with Customers, as amended (commonly referred to as ASC Topic 606). ASC Topic 606 supersedes nearly all existing revenue recognition guidance under generally accepted accounting principles in the U.S. After adopting ASC Topic 606, we will recognize Aldurazyme revenues when the product is shipped to Genzyme and all required quality control certificates are complete, because all of our performance obligations are fulfilled at that point in time. We will record Aldurazyme net product revenues based on the estimated tiered payment that will be in effect when the product is sold through by Genzyme. We believe that any differences between estimated revenues from Genzyme and the actual payments will be insignificant. See Note 4 to our accompanying Consolidated Financial Statements for additional discussion.

### Brineura

Brineura is a recombinant human tripeptidyl peptidase 1 for the treatment of patients with CLN2, a form of Batten disease. CLN2 is an incurable, rapidly progressive disease that 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. We began shipping the product in the U.S. in June 2017.

On June 1, 2017, we announced that the European Commission (EC) granted marketing authorization for Brineura in the EU to treat children with CLN2 disease. Brineura is the first treatment approved in the EU for the treatment of CLN2 disease, and the marketing authorization for Brineura includes all 28 countries of the EU, Norway, Iceland and Liechtenstein. On April 21, 2017, the Committee for Medicinal Products for Human Use (CHMP), the scientific committee of the EMA adopted a positive opinion for our MAA for Brineura following an accelerated review procedure, reserved for medicinal products expected to be a major public health interest. Brineura is one of the first therapies to go through this process. We began shipping the product in the EU in July 2017.

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” below for additional information on combination products.

Brineura net product revenues for the year ended December 31, 2017 were \$8.6 million.

#### Kuvan

Kuvan is a proprietary synthetic oral form of 6R-BH<sub>4</sub>, 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 BH<sub>4</sub> deficiency, a different disorder than PKU. Kuvan net product revenues for the years ended December 31, 2017, 2016 and 2015 totaled \$407.5 million, \$348.0 million and \$239.3 million, respectively.

In the fourth quarter of 2015, we entered into the Termination and Transition Agreement with Ares Trading S.A. (Merck Serono), as amended and restated on December 23, 2015 (the A&R Kuvan Agreement) to terminate the Development, License and Commercialization Agreement, dated May 13, 2005, as amended (the License Agreement), including the license to Kuvan granted in the License Agreement from us to Merck Serono. Also in the fourth quarter of 2015, we and Merck Serono entered into a Termination Agreement (the Pegvaliase Agreement) to terminate the license to pegvaliase granted in the License Agreement from us to Merck Serono.

On January 1, 2016, pursuant to the A&R Kuvan Agreement and the Pegvaliase Agreement, we completed the acquisition from Merck Serono and its affiliates of certain rights and other assets, and the assumption from Merck Serono and its affiliates of certain liabilities, in each case with respect to Kuvan and pegvaliase. As a result, we acquired all global rights to Kuvan and pegvaliase from Merck Serono, with the exception of Kuvan in Japan. Previously, we had exclusive rights to Kuvan in the U.S. and Canada and pegvaliase in the U.S. and Japan.

Pursuant to the A&R Kuvan Agreement, we paid Merck Serono \$374.5 million, in cash, and are obligated to pay Merck Serono up to a maximum of €60.0 million, in cash, if future sales milestones are met. Pursuant to the Pegvaliase Agreement, we are obligated to pay Merck Serono up to a maximum of €125.0 million, in cash, if future development milestones are met.

Two companies previously filed paragraph IV certifications and submitted abbreviated new drug applications (ANDAs) to produce sapropterin dihydrochloride tablets and powder. We entered into settlement agreements regarding Kuvan with both companies. Please see “Government Regulation – Hatch-Waxman Act” below and “Legal Proceedings” in Part I, Item 3 of this Annual Report on Form 10-K for additional information. Please see “Risk Factors” included in Part I, Item 1A of this Annual Report on Form 10-K for a discussion of the risks posed by generic versions of Kuvan.

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 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. Naglazyme net product revenues for the years ended December 31, 2017, 2016 and 2015 were \$332.2 million, \$296.5 million and \$303.1 million, respectively.

#### Vimizim

Vimizim is an enzyme replacement therapy for the treatment of MPS IV A, a lysosomal storage disorder. MPS IV A 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 IV A and estimate that the total number of patients suffering from MPS IV A worldwide could be as many as 3,000.

Vimizim was granted marketing approval in the U.S. and the EU in February 2014 and April 2014, respectively, and subsequently in several other international markets. Vimizim net product revenues for the years ended December 31, 2017, 2016 and 2015 were \$413.3 million, \$354.1 million and \$228.1 million, respectively.

#### Major Product Candidates in Development

##### Pegvaliase

Pegvaliase is an investigational enzyme substitution therapy that we are developing as a subcutaneous injection for the treatment of PKU. In March 2016, we announced that our pivotal Phase 3 PRISM-2 study for pegvaliase met the primary endpoint of change in blood Phe compared with placebo ( $p < 0.0001$ ). This ongoing Phase 3 clinical trial includes an open-label study to evaluate safety and blood Phe levels in naïve patients and a randomized controlled study of the Phase 2 extension study patients and patients from the open-label trial to evaluate blood Phe levels and neurocognitive endpoints. Although we met the primary endpoint of the Phase 3 PRISM-2 study, we did not demonstrate a statistically significant improvement in inattention or mood scores, a key secondary clinical neurocognitive endpoint. The FDA has indicated that lowering Phe blood levels in adults could form the basis for an accelerated approval; however, a favorable outcome on prospectively-specified analyses of inattention in patients with baseline problems with attention may be required for full approval. Although the FDA accepted for Priority Review our BLA for pegvaliase in August 2017, there is no assurance that a reduction in blood Phe alone will be sufficient to support the FDA's full regulatory approval of pegvaliase. On September 14, 2017, we announced that the FDA provided us with the "Day-74" filing communication for our BLA for pegvaliase. In the letter, the FDA advised that it was not currently planning to hold an advisory committee meeting to discuss the application. When the FDA accepted our BLA and granted priority review status, we announced that the FDA had requested additional information on Chemistry, Manufacturing and Controls (CMC), which was likely to be classified as a major amendment to the BLA and result in a three month extension of the PDUFA date. As expected, the FDA designated the receipt of this additional information as a major amendment to the application thus extending the PDUFA target action date by three months to May 28, 2018, which was changed to May 25, 2018 due to the Memorial Day holiday. If we receive approval from the FDA for pegvaliase on the PDUFA target action date, we could potentially launch the sale of pegvaliase in the U.S. in the second half of 2018. We plan to submit a MAA to the EMA in the first quarter of 2018.

##### Valoctocogene Roxaparovec

Valoctocogene roxaparvovec is an 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.

In July 2016, we announced positive proof-of-concept data from a Phase 1/2 dose-escalation study for valoctocogene roxaparvec in patients with severe hemophilia A, and we subsequently provided positive updates to our interim results in January and July 2017. In December 2017, further updates on valoctocogene roxaparvec were presented during an oral presentation at the 59th ASH Annual Meeting and Exposition by John Pasi, M.B., Ch.B., Ph.D., from Barts and The London School of Medicine and Dentistry and primary investigator for this Phase 1/2 study. The data presented at ASH had a cut off of November 16, 2017 and included the following updates: For the 4e13 vg/kg dose, the three patients with the longest follow-up (at week 48) had factor VIII activity levels that were in or near the normal range with both median and mean values of 49%. Median annualized bleed and factor VIII use rates for the 4e13 vg/kg cohort were zero after week 4 and when their factor VIII activity rose above 5%. Mean annualized bleed and factor VIII use rates for the 4e13vg/kg cohort were 0.6 and 2.0, respectively. For the 6e13 vg/kg dose, at 78 weeks post infusion, the median and mean factor VIII levels for patients were 90 and 89%, respectively. Median annualized bleed and factor VIII use rates for the 6e13 vg/kg cohort were zero after week 4. Mean annualized bleed and factor VIII use rates for the 6e13 vg/kg cohort were 0.5 and 6.1, respectively. Patients in the Phase 1/2 study will be monitored for safety for five years.

On December 19, 2017, we announced that we had dosed the first patient in the global GENER8-1 Phase 3 study with the 6e13 vg/kg dose for valoctocogene roxaparvec. This is the first of two Phase 3 studies in the global Phase 3 program to dose a first patient. The global Phase 3 program includes two studies with valoctocogene roxaparvec, one with the 6e13 vg/kg dose (GENER8-1) and one with the 4e13 vg/kg dose (GENER8-2). Both Phase 3 GENER8 studies will be open-label single-arm studies to evaluate the efficacy and safety of valoctocogene roxaparvec. We plan to enroll the first patient in GENER8-2 in early 2018. The primary endpoint in both studies will be based on the factor VIII activity level achieved following valoctocogene roxaparvec, and the secondary endpoints will measure annualized factor VIII replacement therapy use rate and annualized bleed rate. Additionally, we plan to begin another Phase 1/2 Study with the 6e13kg/vg dose with approximately 10 patients who are AAV5 positive. The first patient is expected to enroll in the first half of 2018.

As further described above under “Recent Developments,” the FDA granted valoctocogene roxaparvec Breakthrough Therapy Designation, and the EMA granted access to its PRIME regulatory initiative for valoctocogene roxaparvec.

#### Vosoritide

Vosoritide is a peptide therapeutic in development for the treatment of achondroplasia, the most common form of disproportionate short stature in humans. In April 2016, we reported 12-month data for the patients in the 15 µg/kg/day cohort of the Phase 2 open-label, sequential cohort, dose-escalation study of vosoritide in children who are 5-14 years old, which showed a durable and consistent increase in mean annualized growth velocity of 46%-65% from baseline in the group. Vosoritide continued to be well tolerated with no treatment-related serious adverse events or adverse events leading to discontinuation. In October 2017, we provided an update on the Phase 2 study of vosoritide, which demonstrated sustained increase in average growth velocity over 30 months of treatment in 10 children that completed 30 months of daily dosing at 15 µg/kg/day. Over this period of time, patients experienced mean absolute growth increase of approximately 4 cm over what their baseline growth velocity would have predicted. The sustained increase in annualized growth velocity was accompanied by sustained improvements over time in height compared to age- and gender-matched unaffected children as measured by z-scores. In addition, treatment with vosoritide showed continued improvement over time in proportionality as measured by the U/L ratio.

Our global Phase 3 randomized, placebo-controlled study of vosoritide in approximately 110 children with achondroplasia ages 5-14 for 52 weeks also continued in 2017. The study will be followed by a subsequent open-label

extension. Children in this study will have completed a minimum six-month baseline study to determine their respective baseline growth velocity prior to entering the Phase 3 study. Vosoritide is being tested in children in the age range where their growth plates are still open. This is approximately 25% of people with achondroplasia. We expect to complete enrollment of the Phase 3 study in mid-2018 and provide top-line data in the second half of 2019. Additionally, we intend to begin an infant/toddler study in 2018 in children with achondroplasia ages 0-5. We have undertaken a natural history program to augment our clinical understanding of outcomes of untreated patients for comparison to patients treated with vosoritide.

## BMN 250

BMN 250 is an investigational enzyme replacement therapy using a novel fusion NAGLU with a peptide derived from IGF2 for the treatment of MPS IIIB. MPS IIIB is a rapidly progressive pediatric brain disease caused by NAGLU enzyme deficiency resulting in accumulation of heparan sulfate (HS) in the brain. The accumulation of HS leads to progressive cognitive decline, loss of developmental milestones, severe hyperactivity, sleep disorders, loss of mobility, and early death. BMN 250 is delivered directly into the central nervous system via an ICV access device into the cerebrospinal fluid, which allows for the drug to bypass the blood brain barrier and distribute directly within the brain. In January 2017, we announced positive, preliminary results from a multicenter, international Phase 1/2 clinical trial for BMN 250, which began enrolling patients in April 2016. The study demonstrated that BMN 250 reduced HS levels to normal range in cerebral spinal fluid of MPS IIIB patients. A complementary observational study was also initiated to study the progression of MPS IIIB over time.

In February 2018, we presented interim data from the Phase 1/2 trial for BMN 250 at WORLDSymposium 2018. In the completed dose escalation portion of the study (Part 1), which was designed to determine safety and pharmacodynamics activity of BMN 250, three patients received escalating weekly doses (30mg, 100mg, 300mg) of BMN 250 over nine to twelve months. In Part 2 of the study, patients roll over from Part 1 and from the 250-901 observational study; dosing is 300 mg weekly. This interim data cut included the three patients from the dose escalation study and three patients from Part 2. Eligible patients from the concurrently-running 250-901 observational study are expected to roll over into Part 2 on an ongoing basis. MPS IIIB patients are missing one of four enzymes required for HS degradation, resulting in HS elevations. Cerebrospinal fluid HS levels, which were markedly elevated at baseline, were reduced to the non-affected or normal range in all six patients at the time of the data cut. All patients in the 250-901 observational study have enlarged livers as assessed by abdominal MRI scans. All three BMN 250-treated Part 1 patients experienced decreases in liver size into the normal range for age; data for the first three rollover patients is pending. Untreated MPS IIIB patients in the 250-901 observational study show progressive declines over time in developmental quotient (DQ), a measure of cognitive function normalized to age. In contrast, preliminary data in all three Part 1 patients suggest DQ stabilization. Further data in these patients and in patients who roll into the treatment study is necessary to confirm this early observation. Interim trial data indicates that ICV-administered BMN 250 is well-tolerated by MPS IIIB patients, and that the adverse event profile for BMN 250 is generally consistent with that seen with other enzyme replacement therapies and the ICV mode of administration.

## BMN 290

BMN 290 is a selective chromatin modulation therapy for the treatment of Friedreich's ataxia. Friedreich's ataxia is a rare autosomal recessive disorder, which results in disabling neurologic and cardiac progressive decline. Currently there are no approved disease modifying therapies for Friedreich's ataxia. In preclinical models, BMN 290 increases frataxin expression in affected tissues more than two-fold. BMN 290 is a second-generation compound derived from a compound we acquired from Repligen Corporation (Repligen) that had human clinical data demonstrating increases in frataxin in Friedreich's ataxia patients. We selected BMN 290 for its favorable penetration into the central nervous system and cardiac target tissues and its preservation of the selectivity of the original Repligen compound. We plan to submit an IND in the second half of 2018.

## Manufacturing

We manufacture the active pharmaceutical ingredients (API) for Aldurazyme, Brineura, Naglazyme, Vimizim, pegvaliase, and vosoritide in our production facilities located in Novato, California. 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 Aldurazyme, Naglazyme, Brineura, and Vimizim. Vialing and most packaging are performed by contract manufacturers. We believe that we have ample manufacturing capacity to support commercial demand for both Aldurazyme and Naglazyme for at least the next five years.

We currently manufacture the API for Brineura, Vimizim and BMN 250 in our manufacturing facility in Shanbally, Cork, Ireland. This facility has been approved by the FDA, Health Product Regulatory Authorities, EMA, EC, and health agencies in other countries for the testing, packaging, labeling, and release of Vimizim. In June 2017, the FDA approved the Shanbally facility as a bulk biologics manufacturing plant for Vimizim production, which enhances our business continuity and increases our operating capacity to support the anticipated commercial demand of Vimizim. In November and December 2017, the FDA and the EMA, respectively, approved the Shanbally facility as a formulated bulk drug substance manufacturing and quality control facility for Brineura, which further increases our operating capacity and supports the anticipated commercial demand for Brineura. Vialing and most packaging of Brineura, Vimizim and BMN 250 are performed

by contract manufacturers. We believe that with the Shanbally facility and our Novato, California facility, we have ample manufacturing capacity to support commercial demand for Brineura and Vimizim for at least the next five years.

Firdapse and Kuvan tablets and powder sachets are currently manufactured on a contract basis by third parties. In general, we expect to continue to contract with outside service providers for certain manufacturing services, including drug substance, API, final product vialing, tableting and sachet production 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 cGMPs 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. Although we have never experienced a disruption in supply from our contract manufacturers, we cannot provide assurance that we will not experience a disruption in the future.

In July 2017, we commissioned our commercial gene therapy manufacturing facility, located in Novato, California, and began cGMP production of valoctocogene roxaparvec to support clinical development activities and anticipated commercial demand. This facility is capable of supporting the manufacturing of product for approximately 2,000 patients per year, and the production process was 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 2018, the size of our sales force will be

appropriate to effectively reach our target audience in markets where our products are directly marketed. The launch of any future products, including pegvaliase, will likely require expansion of our commercial organization, including our sales force, in the U.S. and abroad, and we would need to commit significant additional funds, management's attention and other resources to such expansion.

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, Firdapse, Kuvan, Naglazyme, and Vimizim customers include a limited number of specialty pharmacies and end-users, such as hospitals and foreign government agencies. We also sell Brineura, Kuvan, Naglazyme 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. During 2017, 45% of our net Brineura, Firdapse, Kuvan, Naglazyme, and Vimizim product revenues were 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 candidate drugs and commercial drugs 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, 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, even 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.

Our products have no direct approved competition currently on the market, however, other companies are in the development phase with new and generic products. The following is a summary of some of the primary possible future competitors for our products, but the information below may not include all potential competition.

### Aldurazyme, Naglazyme, and Vimizim

In the mucopolysaccharidosis field, several companies are researching treatments using small molecules, gene therapy, and other novel technologies. Aldurazyme, for the treatment of MPS I, has potential competition from clinical stage product candidates from Sangamo Therapeutics, Inc. and ArmaGen, Inc. and earlier stage product candidates, including product candidates from RegenxBio Inc., Immusoft Corporation, and Eloxx Pharmaceuticals Ltd. 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. These companies, however, are likely a year or more away from commercial therapies.

### Kuvan and Pegvaliase

There are currently no other approved drugs 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 will allow these companies to market generic versions of sapropterin dihydrochloride. Please see “Government Regulation – Hatch-Waxman Act” below and “Legal Proceedings” in Part I, Item 3 of this Annual Report on Form 10-K for additional information.

#### Brineura

Brineura, for the treatment of CLN2, has potential competition from earlier stage products, including a preclinical gene therapy product candidate under development by Spark Therapeutics, Inc.

#### Product Candidates

Our product candidates have potential competition from product candidates either using similar technology to our programs or different treatment strategies. Valoctocogene roxaparvovec, for the treatment of hemophilia A, could have competition from marketed factor VIII replacement therapies, a novel bispecific antibody marketed



by Roche Holding Ltd, earlier stage programs, including a product candidates under development by Spark Therapeutics, Inc., Sangamo Therapeutics, Inc, and Shire Plc, and preclinical product candidates from other companies. In addition, Alnylam Pharmaceuticals, Inc. is developing a novel product candidate in the clinic for the treatment of hemophilia A. Vosoritide, for the treatment of achondroplasia, could have competition from early stage products under development by Ascendis Pharma A/S and Therachon AG. BMN 250, for the treatment of MPS IIIB, has potential competition from a clinical stage gene therapy program from Abeona Therapeutics Inc., preclinical gene therapy programs from Laboratorios del Dr. Esteve, S.A.U. and Orchard Therapeutics, Ltd. and other earlier stage product candidates, including a recombinant protein product candidate from ArmaGen. Inc. BMN 290, for the treatment of Friedreich's ataxia, has potential competition from clinical stage product candidates from Reata Pharmaceuticals, Inc. and Takeda Pharmaceutical Company and earlier stage product candidates from Agilis Biotherapeutics, Inc., Voyager Therapeutics, Inc., Pfizer Inc. and Chondrial Therapeutics 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.

As of January 19, 2018, the number of our worldwide issued patents now stands at 1,481, including 111 patents issued by the U.S. Patent and Trademark Office (the USPTO). Furthermore, our portfolio of pending patent applications totals 478 applications, including 79 pending U.S. applications.

With respect to Naglazyme, we have 53 issued patents, including three U.S. patents. Claims cover our ultrapure N-acetylgalactosamine-4-sulfatase compositions of Naglazyme, methods of treating deficiencies of N-acetylgalactosamine-4-sulfatase, including MPS VI, methods of producing and purifying such ultrapure N-acetylgalactosamine-4-sulfatase compositions and methods of detecting. These patents will expire between 2021 and 2028.

With respect to Kuvan, we own, co-own or have licensed a number of patents and pending patent applications that relate generally to formulations and forms of our drug substance, methods of use for various indications under development and dosing regimens. We have rights to 151 issued patents including 16 issued U.S. patents with claims to a stable tablet and oral solution formulation of 6R-BH4, methods of treating PKU using a once daily dosing regimen, methods of administration of Kuvan with food, crystalline forms of 6R-BH4, and methods of producing 6R-BH4. These patents will expire between 2024 and 2032.

We have rights to 33 issued patents, including six U.S. patents, related to Aldurazyme. These patents cover our ultra-pure alpha-L-iduronidase composition of Aldurazyme, methods of treating deficiencies of alpha-L-iduronidase by administering pharmaceutical compositions comprising such ultra-pure alpha-L-iduronidase, a method of purifying such ultra-pure alpha-L-iduronidase and the use of compositions of ultra-pure biologically active fragments of alpha-L-iduronidase. These patents will expire in 2019 and 2020. There are U.S. patents on alpha-L-iduronidase owned and controlled by a third party. We have examined such issued U.S. patents, the related U.S. and foreign applications and their file histories, the prior art and other information. Corresponding foreign applications were filed in Canada, Europe and Japan. The European application was rejected and abandoned and cannot be re-filed. The Japanese application has also lapsed and cannot be re-filed. Claims in the related Canadian application issued in 2007. We believe that such patents may not survive a challenge to patent validity but that it is unlikely that a court in any country would order us to stop marketing the only life-saving drug that is currently approved for this disease. However, the processes of patent law are uncertain and any patent proceeding is subject to multiple unanticipated outcomes. We believe that it is in the best interest of our joint venture with Genzyme to market Aldurazyme with

commercial diligence, in order to provide MPS I patients with the benefits of Aldurazyme. We believe that these patents and patent applications do not affect our ability to market Aldurazyme in Europe.

We have patent protection in the European Patent Organization countries for Firdapse for the treatment of LEMS. These patents will expire in 2022.

With respect to Vimizim, we own or have licensed a number of patents and pending patent applications that relate generally to compositions of matter, methods of use and methods of production. We have rights to

204 issued patents including 17 issued U.S. patents with claims to compositions of purified recombinant N-acetylgalactosamine-6-sulfate sulfatase (Vimizim) methods of treating Morquio Syndrome and sulfatase-modifying factor I (SUMF1) polypeptides and nucleic acids used in the manufacture of Vimizim. Issued U.S. patents cover SUMF1 compositions (set to expire in 2019), purified recombinant Vimizim compositions (set to expire in 2029) and methods of treating Morquio Syndrome (set to expire in 2029). We also have issued U.S. and European patents that cover methods of production (set to expire in 2024) and formulations (set to expire in 2031).

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 foreign 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 new drug applications, or NDAs, 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 clinical trial application (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. Satisfaction of FDA and EMA 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 deemed approved 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

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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 NDA or BLA is prepared and submitted to the FDA and an MAA is prepared and submitted to the EMA. FDA approval of the NDA or BLA is required before marketing of the product may begin in the U.S. and approval of the MAA by the EC is required before marketing of the product may begin in the EU. 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 FDA or the EMA may request additional information rather than accepting an NDA/BLA or MAA, respectively, 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, the FDA will re-initiate review. If it is satisfied that the deficiencies have been addressed, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included. It is not unusual, however, for the FDA to issue a complete response letter because it believes that the drug is not safe enough or effective enough or because it does not believe that the data submitted are reliable or conclusive.

For the EMA, an application designated as standard review typically lasts approximately eleven months depending on the length of time sponsors take to address EMA questions. The accelerated assessment procedure is applicable to marketing authorization applications for medicinal products that are expected to be of major public health interest. For applications that receive accelerated assessment designation and are able to remain on this timeline the review

typically lasts approximately seven months depending on the length of time sponsors take to address EMA questions. It is not unusual, however, for applications that receive accelerated assessment designation to revert to standard review, typically because the EMA has determined that the significance of the questions that the company needs to address would be more appropriate under the standard review timelines. At the end of the review period, EMA will issue an opinion either in support of marketing authorization (positive opinion) or recommending refusal of a marketing authorization (negative opinion). In the event of a negative opinion, the company may request a re-examination of the application. Within 60 days the company must provide the EMA detailed grounds for requesting re-examination. Within 60 days of providing this

information, the EMA will issue an opinion either in support of marketing authorization (positive opinion) or recommending refusal of a marketing authorization (negative opinion). In the event of a positive opinion, the EC will then grant marketing authorization in approximately 67 days. The EC follows the recommendation of the EMA in almost all cases.

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 risk evaluation and mitigation strategy (REMS), to help ensure that the benefits of the drug outweigh the potential risks. REMS can include medication guides, communication plans for healthcare professionals, and elements to assure safe use (ETASU). ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring and the use of patient registries. The requirement for REMS can materially affect the potential market and profitability of the drug. 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, or PMOA, 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 that generates the PMOA becomes the lead evaluator. If there are two independent modes of action, neither of which is subordinate to the other, the FDA makes a determination as to which Center to assign the product based on consistency with other combination products raising similar types of safety and effectiveness questions or to the Center with the most expertise in evaluating the most significant safety and effectiveness questions raised by the combination product. 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

evaluations 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.

The 21st Century Cures Act was signed into law on December 13, 2016. In Section 3038, the FDA is instructed that if a combination product has an approved constituent (e.g., an investigational drug delivered with devices already 510(k) cleared by the FDA), the FDA may only require the sponsor to submit data or information necessary to meet the standard for clearance or approval, taking into consideration incremental risks and benefits posed by the combination product, using a risk-based approach and taking into account any prior finding of safety and effectiveness or substantial equivalence for the approved constituent. It appears that the primary



purpose of this provision is to reduce the burden of proving the safety and effectiveness of the approved constituent by leveraging the FDA's prior clearance or approval. The FDA is instructed to focus on the new constituent plus the incremental risk created by a new use of the approved constituent. It is too soon to understand how the FDA will implement this provision.

#### 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 pre-clinical 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 pre-clinical 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.

### 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. and in the EU is defined as no more than five in 10,000 people in the EU, which is equivalent to around 250,000 people or less. Orphan drug designation must be requested before submitting a marketing application.

Orphan drug designation does not shorten the regulatory review and approval process, nor does it provide any advantage in 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. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the NDA/BLA application user fee. Although obtaining approval to market a product with orphan drug exclusivity may be advantageous, we cannot be certain:

- that we will be the first to obtain approval for any drug for which we obtain orphan drug designation;
- that orphan drug designation will result in any commercial advantage or reduce competition; or
- that the limited exceptions to this exclusivity will not be invoked by the relevant regulatory authority.

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.

### 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 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 Biologics Price Competition and Innovation Act of 2009 (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.

### Fast Track Designation

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.

### 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.

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, REMS, 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 access 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.

### Good Manufacturing Practices

The FDA, the EMA and other regulatory agencies regulate and inspect equipment, facilities and processes used in the manufacture of pharmaceutical and biologic products prior to approving a product. If, after receiving approval from regulatory agencies, a company makes a material change in manufacturing equipment, location or process, additional

regulatory review and approval may be required. All facilities and manufacturing techniques used for the manufacture of our products must comply with applicable regulations governing the production of pharmaceutical products known as cGMPs.

The FDA, the EMA and other regulatory agencies also conduct regular, periodic visits to re-inspect equipment, facilities and processes following initial approval of a product. If, as a result of these inspections, it is determined that our equipment, facilities or processes do not comply with applicable regulations and conditions

of product approval, regulatory agencies may require product recall, issue warning or similar letters or may seek civil, criminal, or administrative sanctions against us.

### Healthcare Reform

The U.S. and some foreign 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 Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 (as amended, 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 BPCIA, which was enacted as part of 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.

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 included a 50% 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. Substantial new provisions affecting compliance also have been added, which may require us to modify our business practices with healthcare practitioners. In addition, drug manufacturers are required to collect and report annually information on payments or transfers of value to physicians and teaching hospitals, as well as investment interests held by physicians and their immediate family members during the preceding calendar year. 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 judicial and Congressional challenges to certain aspects of the PPACA, and we expect that there will be additional challenges and amendments in the future, especially

with the recent change in administration.

Since January 2017, the U.S. President has signed two 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 has 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, two bills affecting the implementation of certain taxes under the PPACA have been signed into law.

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The Tax Cuts and Jobs Act of 2017 includes a provision repealing the “individual mandate,” the tax-based shared responsibility payment imposed by the PPACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year, effective January 1, 2019. Additionally, on January 23, 2018, the U.S. President signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain PPACA-mandated fees, including the so-called “Cadillac” tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. Congress also could consider subsequent legislation to repeal or replace elements of the PPACA.

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 2025 unless additional Congressional action is taken. 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, at the federal level, there have been several recent U.S. Congressional inquiries and proposed bills 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 U.S. Administration have each indicated that it will continue to seek new legislative and/or administrative measures to control 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.

#### 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 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 exemptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exemptions 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 exemption 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 its implementing regulations, also imposes obligations, including mandatory contractual terms, on certain types of individuals and entities, with respect to safeguarding the privacy, security and transmission of individually identifiable health information.

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

The majority of states also have statutes or regulations similar to the federal Anti-Kickback Statute and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer. Sanctions under these federal and state laws may include civil monetary penalties, damages, monetary fines, disgorgement, exclusion of a company's products from reimbursement under federal healthcare programs, criminal fines, contractual damages, reputational harm, diminished profits and future earnings, curtailment of operations and imprisonment. 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. Other states prohibit providing various other marketing-related activities. 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.

#### Approval Outside of the U.S./EU

For marketing outside the U.S. and the EU, we are subject to foreign 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 pre-clinical 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 foreign 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 United Kingdom, 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, such as government or private healthcare insurers, 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. 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 price levels sufficient to realize an appropriate return on 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. Beginning in 2017, non-innovator products are also subject to an additional rebate.

The statutory definition of AMP was amended in 2010. CMS has released the final rule pertaining to AMP and other aspects of the Medicaid drug rebate program, which was effective as of April 1, 2016.

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.

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.

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, by 2020 Medicare Part D beneficiaries will 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. Beginning in 2011, each manufacturer of drugs approved under NDAs or BLAs was required to enter into a Medicare Part D coverage gap discount agreement and provide a 50% 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 civil monetary penalties of \$100,000 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.

#### Employees

As of February 13, 2018, we had 2,581 full-time employees, 1,133 of whom were in operations, 654 of whom were in research and development, 378 of whom were in sales and marketing and 416 of whom were in administration.

We consider our employee relations to be good. Our employees are not covered by a collective bargaining agreement. We have not experienced employment related work stoppages.

#### Research and Development

For information regarding research and development expenses incurred during 2017, 2016 and 2015, see Item 7, Management's Discussion and Analysis of Financial Condition and Results of Operations—Research and Development.



## Segment and Geographic Area Financial Information

Our chief operating decision maker (i.e., our chief executive officer) reviews financial information on a consolidated basis, for the purposes of allocating resources and evaluating financial performance. Accordingly, we consider ourselves to have a single reporting segment and operating unit structure.

Net product revenues by geography are based on patients' locations for our commercial products, which are sold directly by us, and global sales of Aldurazyme, which is marketed by Genzyme. Genzyme is our sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third parties. Although Genzyme sells Aldurazyme worldwide, the revenues earned by us on Genzyme's net sales are not broken out by geographic region as the underlying revenue transactions are with Genzyme, whose headquarters are located in the U.S.

The table below summarizes consolidated net product revenue based on patient location for Brineura, Firdapse, Kuvan, Naglazyme, and Vimizim, which are sold directly by us, and global sales of Aldurazyme, which is marketed by Genzyme (in millions):

	Years Ended December 31,		
	2017	2016	2015
<b>Net product revenues:</b>			
United States	\$495.7	\$411.9	\$343.4
Europe	266.2	252.6	171.2
Latin America	182.4	147.5	142.3
Rest of the world	236.1	204.6	129.6
Total net product revenues marketed by BioMarin	1,180.4	1,016.6	786.5
<b>Aldurazyme net product revenues marketed by</b>			
Genzyme	90.0	93.8	98.0
Total net product revenue	\$1,270.4	1,110.4	\$884.5

Total revenues generated outside the U.S. was \$725.4 million, \$609.3 million and \$445.8 million, in the years ended December 31, 2017, 2016 and 2015, respectively.

The following table outlines non-monetary long-lived assets by geographic area (in millions):

	December 31,		
	2017	2016	2015
<b>Non-monetary long-lived assets:</b>			
United States	\$1,183.8	\$1,183.9	\$940.5
Europe	823.7	812.8	865.2
Rest of World	2.7	2.6	2.3
Total long-lived assets	\$2,010.2	\$1,999.3	\$1,808.0

The increase in non-monetary long-lived assets in 2017 compared to 2016 was primarily attributable to increased costs related to our in-process projects at our manufacturing facilities in the U.S. and Europe, partially offset by a net decrease in intangible assets due to normal amortization of the definite lived intangible assets. The increase in

non-monetary long-lived assets in 2016 compared to 2015 was primarily attributable to increased costs related to our in-process projects at our manufacturing facilities, partially offset by a net decrease in intangible assets resulting from the impairment of in-process research and development intangible assets due to program terminations in 2016.

See “Risk Factors” included in Part I, Item 1A of this Annual Report on Form 10-K for additional information regarding the risks we face related to our foreign operations.

#### 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 obtained by visiting the SEC's Public Reference Room at 100 F Street, NE, Washington, D.C. 20549 or by calling the SEC at 1-800-SEC-0330. Additionally, these reports are available at the SEC's website at <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.

### Risks Related to Our Business

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 and maintain regulatory approval to market and sell our product candidates. For example, in the U.S., we must obtain Food and Drug Administration (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, including pegvaliase, in any jurisdiction. For example, even though the pivotal Phase 3 PRISM-2 study of pegvaliase met the primary endpoint of change in blood Phe compared with placebo ( $p < 0.0001$ ), we did not demonstrate a statistically significant improvement in inattention or mood scores, a key secondary clinical neurocognitive endpoint. In August 2017, the FDA accepted for Priority Review our Biologics License Application (BLA) for pegvaliase, but there is no assurance that a reduction in blood Phe alone will be sufficient to support the FDA's full regulatory approval of pegvaliase.

Although the FDA and the EMA have programs to facilitate 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. 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 contract research organizations (CROs) to file some of our foreign 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.

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 is not a well-established area, 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 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, although we designed our Phase 3 study of vosoritide in a manner that we believe can demonstrate efficacy and safety of the product candidate for the target patient population, the FDA may ultimately disagree. On January 23, 2018, the FDA announced the following: “On Thursday, March 22, 2018, the Pediatric Advisory Committee (PAC) and the Endocrinologic and Metabolic Drugs Advisory Committee (EMDAC) will meet to discuss the major objectives of a phase 3 drug development program indicated for the treatment of children with achondroplasia (ACH) submitted by BioMarin Pharmaceutical Inc. The following elements of a phase 3 program should be considered for discussion: evidence required to establish dose-response, study design, e.g., placebo control, study duration, intended population, e.g., infants and toddlers and/or older children and adolescents, and endpoints that have a clinically meaningful impact on the patient’s functional or psychological well-being.” We cannot predict the outcome of this meeting, how the FDA will incorporate the advice from the meeting or whether or not the meeting will have any impact on our development of vosoritide. 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 Firdapse has received regulatory approval to be commercially marketed in the EU. Any product for which we have obtained regulatory approval, or for which we obtain regulatory approval in the future, along with the manufacturing processes and practices, post-approval clinical research, product labeling, advertising and promotional activities for such product, are subject to continual requirements of, and review by, the FDA, the 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, requirements regarding the distribution of samples to physicians, import and export requirements and recordkeeping.

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. Thus, we will not be able to promote any products we develop for indications or uses for which they are not approved.

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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, the value of our company 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.

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 granted to drugs intended to treat a rare disease or condition, defined as having a prevalence of no more than five in 10,000 people in the EU, which is equivalent to around 250,000 people or fewer. 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. Similar regulations are available in the EU with a ten-year period 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. 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 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. Historically, a biologic product approved under a BLA was not subject to the generic drug review and approval provisions of the FDC Act. However, 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. 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. Such biosimilars would reference biological products approved in the U.S. The BPCIA establishes a period of 12 years of exclusivity for reference products. Aldurazyme's exclusivity under the BPCIA expired in 2015, Brineura's exclusivity under the BPCIA expires in 2029, Naglazyme's exclusivity under the BPCIA expired in June 2017, and Vimizim's exclusivity under the BPCIA expires in 2026. Our products approved under BLAs, as well as products in development that may be approved under BLAs in the future, could be reference products for biosimilar marketing applications.

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.

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. We expect the number of preclinical studies and clinical trials that the regulatory authorities will require will vary 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. 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, including us with respect to Kyndrisa, 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 pre-clinical 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.

Our valoctocogene roxaparvec (formerly referred to as BMN 270) program is based on a gene therapy approach, which, as a novel technology, presents additional treatment, regulatory, manufacturing, and commercial 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 and commercializing more traditional pharmaceutical drugs, there are additional, unique 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 much or too little of the desired protein or RNA. There is also a risk that production of the desired protein or RNA will increase or decrease over time. Because the treatment is irreversible, there may be challenges in managing side effects, particularly those caused by overproduction. 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.

We may experience development problems related to our gene therapy program 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 and the FDA has approved several cell-based gene therapy treatments to date, the FDA has only approved one vector-based gene therapy product 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 our product candidate in any jurisdiction. Regulatory requirements governing gene and cell therapy products are still evolving and may continue to change in the future. 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. Delay or failure to obtain, or unexpected costs

in obtaining, the regulatory approval necessary to

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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.

Even if we obtain regulatory approval for valoctocogene roxaparvovec, we may experience delays, and increased costs, in developing a sustainable, reproducible and large-scale manufacturing process. Gene therapy products are novel, complex and difficult to manufacture, 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. Whether we produce valoctocogene roxaparvovec at a contract manufacturer or at our own gene therapy manufacturing facility, we will likely face technical and scientific challenges, considerable capital costs, and potential difficulty in recruiting and hiring experienced, qualified personnel. As a result, we could experience manufacturing delays that prevent us from completing our clinical studies or commercializing valoctocogene roxaparvovec in a timely, or on a profitable, basis, if at all.

Due to the relative novelty of gene therapy and the potential to provide extended duration therapeutic treatment with a one-time administration, we also face uncertainty with respect to the pricing, coverage and reimbursement of valoctocogene roxaparvovec, 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 roxaparvovec 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 roxaparvovec. Accordingly, sales of valoctocogene roxaparvovec, 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 a sufficient return on our investment.

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 roxaparvovec, the commercial success of valoctocogene roxaparvovec will 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. 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. Even if valoctocogene roxaparvovec displays a favorable efficacy and safety profile in clinical trials and is ultimately approved, market acceptance of valoctocogene roxaparvovec will not be fully known until after it is launched. Negative public opinion or more restrictive government regulations or could have a negative effect on our business and financial condition and may delay or impair the development and commercialization of, and demand for, valoctocogene roxaparvovec.

If we continue to incur operating losses and experience net cash outflows 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 and 2010. Based upon our current plan for investments in research and development for existing and new programs, as well as capital investments in our facilities and working capital needs, such as for inventory, we expect to operate at a net loss and experience net cash outflows for at least the next 12 months. 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 and cash flow positive 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.

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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, 2017, we had cash, cash equivalents and investments totaling \$1.8 billion and long-term debt obligations of \$1.2 billion (undiscounted). 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 pegvaliase, we made cash payments on this transaction totaling \$374.5 million, and may pay Merck Serono up to a maximum of €60 million, in cash, if future sales milestones are met with respect to Kuvan and up to a maximum of €125 million, in cash, if future development milestones are met with respect to pegvaliase. In October 2013, we completed an offering of senior subordinated convertible notes and received net proceeds of approximately \$696.4 million, after deducting commissions, estimated offering expenses payable by us and the purchase of the related capped calls. In August 2017, we completed an offering of senior subordinated convertible notes and received net proceeds of approximately \$481.7 million, after deducting commissions and estimated offering expenses payable by us, a majority of which we intend to use to repay, repurchase or settle in cash some or all of our 0.75% senior subordinated convertible notes due in 2018 (the 2018 Notes). We will need cash to not only repay the principal amount of the 2018 Notes, our 1.50% senior subordinated convertible notes due in 2020 (the 2020 Notes), and our 0.599% senior subordinated convertible notes due in 2024, (the 2024 Notes, and together with the 2018 Notes and the 2020 Notes, the Notes) but also the ongoing interest due on the Notes during their term.

We may require additional financing to fund the repayment of our 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 termination agreements with Merck Serono related to Kuvan and pegvaliase milestones;
- 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;
- Genzyme's ability to continue to successfully commercialize Aldurazyme; and
- whether our convertible debt is converted to common stock in the future.

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

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

We will need to raise additional funds from equity or debt securities, loans or collaborative agreements if we are unable to satisfy our liquidity requirements. The sale of additional 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, 2017, we had \$1.2 billion (undiscounted) principal amount of indebtedness, including \$375.0 million (undiscounted) principal amount of indebtedness under the 2018 Notes, \$375.0 million (undiscounted) principal amount of indebtedness under the 2020 Notes, and \$495.0 million (undiscounted) principal amount of indebtedness under the 2024 Notes. In November 2016, we also entered into a credit agreement (the Credit Agreement) with Bank of America, N.A., as the administrative agent, swing line lender and letter of credit issuer, providing for up to \$100.0 million in revolving loans. 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 Credit Agreement does, 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 Credit Agreement, the outstanding borrowings thereunder could become immediately due and payable, the Credit Agreement 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 our 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 Credit Agreement.

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 2018 Notes, 2020 Notes and 2024 Notes, which, if not converted, will be required to be repaid in cash at maturity in 2018, 2020 and 2024, respectively. In addition, in the event the conditional conversion feature of the 2018 Notes or 2020 Notes is triggered, holders of such Notes will be entitled to convert the 2018 Notes or 2020 Notes at any time during specified periods at their option. In addition, the 2018 Notes will be freely convertible on or after July 15, 2018 and the 2020 Notes will be freely convertible on or after July 15, 2020. We intend to use a majority of the net proceeds we received from the issuance of the 2024 Notes to repay, repurchase or settle in cash some or all of the 2018 Notes. We may also elect to settle conversions of the 2020 Notes in cash, which could further affect our liquidity. While we could seek





to obtain additional third-party financing to pay for any amounts due in cash upon such events, we cannot be sure that such third-party financing will be available on commercially reasonable terms, if at all.

The 2018 Notes mature in October 2018, and therefore we have reclassified the outstanding principal of such Notes as a current liability. We could also be required under applicable accounting rules to reclassify all or a portion of the outstanding principal of such the 2020 Notes as a current rather than long-term liability (for example, if there are 12 months or less remaining until maturity), which would result in a material reduction of our net working capital. While we could seek to obtain third-party financing to pay for any amounts due in cash upon such events, we cannot be sure that such third-party financing will be available on commercially reasonable terms, if at all. Furthermore, if we are required to share settle any conversions of Notes, due to lack of requisite liquidity or otherwise, we may cease to be eligible to account for the Notes using the treasury stock method, which may adversely impact our diluted earnings per share. In addition, although we had no outstanding balance under the Credit Agreement as of December 31, 2017, we also may borrow up to \$100.0 million in revolving loans under the Credit Agreement, which would be required to be repaid in cash at maturity in November 2018.

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, 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 products to produce sufficient quantities at acceptable costs, we may be unable to meet demand for our products and lose potential revenue, have reduced margins or be forced to terminate a program.

Due to the complexity of manufacturing our products, we may not be able to manufacture 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.

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 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.

Although we have entered into contractual relationships with third-party manufacturers to produce the active ingredient in Firdapse and Kuvan, if those manufacturers are unwilling or unable to fulfill their contractual obligations, we may be unable to meet demand for Firdapse and Kuvan or sell these products at all, we may lose potential revenue, and we may be forced to terminate a program. We have contracts for the production of final product for Firdapse and Kuvan. We also rely on third parties for portions of the manufacture of Aldurazyme, Brineura, Naglazyme and Vimizim. 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.

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Any interruption in the supply of finished products could hinder our ability to distribute finished products to meet commercial demand.

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.

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 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. 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 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

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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.

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 in the past undertaken and may in the future 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.

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. 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.

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 both in the U.S. and internationally. 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 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. 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 or threatened to impose revenue caps limiting the annual volume of sales of our products. To the extent that these caps are significantly below actual demand, 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. The U.S. 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 judicial and Congressional challenges to certain aspects of the PPACA, as well as recent efforts by the U.S. Presidential administration to repeal or replace certain aspects of the PPACA, and we expect there will be additional challenges and amendments to the PPACA in the future. Since January 2017, the U.S. President has signed two Executive Orders and other directives designed to delay, circumvent, or loosen certain requirements mandated by the PPACA. Concurrently, Congress has 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, the Tax Cuts and Jobs Act of 2017 includes a provision repealing the “individual mandate,” the tax-based shared responsibility payment imposed by the PPACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year, effective January 1, 2019. Additionally, on January 23, 2018, the U.S. President signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain PPACA-mandated fees, including the so-called “Cadillac” tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. Congress may consider other legislation to repeal or replace elements of the PPACA. It is uncertain the extent to which any such changes may impact our business or financial condition. 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, 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 over the manner in which manufacturers set prices for their marketed products. For example, there have been several recent U.S. Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the cost of drugs under Medicare, and reform government program reimbursement methodologies for drug products. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payers. In addition, individual states in the United States have also become increasingly active in passing legislation and implementing 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. Legally mandated price controls on payment amounts by third-party payers or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, 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. 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.

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.

If we are found in violation of federal or state healthcare laws, we may be required to pay a penalty or be suspended from participation in federal or state healthcare programs, which may adversely affect our business, financial condition and results of operations.

We are subject to various federal and state healthcare laws and regulations, including anti-kickback laws, false claims laws, data privacy and security laws, and laws related to ensuring compliance. 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 federal government regulations, certain arrangements, or safe harbors, are deemed not to violate the federal Anti-Kickback Statute. However, the exemptions 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 exemption 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 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 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.

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, security and transmission of individually identifiable health information. Many state and foreign 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.

Substantial new provisions affecting compliance have also been adopted, which may require us to modify our business practices with healthcare practitioners. The PPACA, through the Physician Payments Sunshine Act, requires drug manufacturers to collect and report to CMS information on payments or transfers of value to physicians and teaching hospitals, as well as investment and ownership interests held by physicians and their immediate family members during the preceding calendar year. Failure to submit required information may result in civil monetary penalties.

In addition, there has been a recent trend of increased state regulation of payments made to physicians. Certain states 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, and/or the tracking and reporting of gifts, compensation and other remuneration to physicians. The shifting compliance environment and the need to implement systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that a pharmaceutical manufacturer may violate one or more of the requirements.

Due to the breadth of these laws, the narrowness of available statutory and regulatory exceptions 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.

In addition, recent healthcare reform legislation has strengthened these laws. 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 False Claims Act. If we are found in violation of one of these laws, we may be subject to 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, debarment, suspension or exclusion from participation in federal or state healthcare programs, any of which could adversely affect our business, financial condition and results of 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, Kuvan, Naglazyme and Vimizim, and all of the sales of Firdapse are generated from countries other than the U.S. Similarly, we expect a significant portion of the sales of Brineura 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 foreign 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
- regulations relating to data security and the unauthorized use of, or access to, 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.



If we fail to comply with U.S. export control and economic sanctions, our business, financial condition and operating results may be adversely affected.

We rely on a general license from the U.S. Treasury Department's Office of Foreign Assets Control (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. Moreover, 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.

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 United States, the UK 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 foreign 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 foreign 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.

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 U.K. pound, the Canadian dollar, the Swiss franc, the Japanese yen and several other currencies, changes in those currencies relative to the U.S. dollar will impact our revenues and expenses. If the U.S. dollar 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 U.S. dollar 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 U.S. dollars, changes in currency exchange rates between the U.S. dollar 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 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.

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

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) and 3,4-DAP (the active ingredient in Firdapse) have 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.
- 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. We have limited experience with competitors interfering with or challenging the validity or enforceability of our patents or patent applications.
- Generic manufacturers may use litigation and regulatory means to obtain approval for generic versions of our products notwithstanding our filed patents or patent applications.
- 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 BioMarin's 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.

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 roxaparvec, 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.

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.

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 depends 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, Firdapse, 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.

If generic manufacturers are successful in their use of litigation or regulatory means to obtain approval for generic versions of Kuvan, our revenue and results of operations would be adversely affected.

The Drug Price Competition and Patent Term Restoration Act of 1984, known as the Hatch-Waxman Act, permits the FDA to approve 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.

We received a paragraph IV notice letter, dated December 23, 2016, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL), notifying us that DRL had filed an abbreviated new drug application (ANDA) seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral powder prior to the expiration of our patents listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). We filed a lawsuit alleging patent infringement against DRL. In August 2017, we entered into a settlement agreement with DRL (the DRL Powder Settlement Agreement) that resolved the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral powder. Under the terms of the DRL Powder Settlement Agreement, we granted DRL a non-exclusive license to our Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride in oral powder form in 100 mg and 500 mg packet formulations in the U.S. for the indications approved for Kuvan beginning on October 1, 2020, or earlier under certain circumstances.

We also received two separate paragraph IV notice letters, dated January 14, 2016 and January 22, 2015, from Par notifying us that Par had filed an ANDA seeking approval of proposed generic versions of Kuvan 100 mg oral powder and Kuvan 100 mg oral tablets, respectively, prior to the expiration of our patents listed in the FDA's Orange Book. We filed two lawsuits alleging patent infringement against Par (the lawsuit against Par pertaining to the proposed generic version of Kuvan 100 mg oral tablets was filed together with Merck & Cie), and the two Par cases were consolidated. In April 2017, we and Merck & Cie entered into a settlement agreement with Par (the Par Settlement Agreement) that resolved both cases against Par. Under the Par Settlement Agreement, we granted Par a non-exclusive

license to our Kuvan-related patents to allow Par to market a generic version of sapropterin dihydrochloride in 100 mg oral tablets and oral powder in 100 mg and 500 mg packet formulations in the U.S. for the indications approved for Kuvan beginning on: April 1, 2021 if Par is not entitled to the statutory 180-day first filer exclusivity period; October 1, 2020 if Par is entitled to the statutory 180-day first filer exclusivity period; or earlier under certain circumstances.

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We also received a paragraph IV notice letter, dated October 3, 2014, from DRL notifying us that DRL had filed an ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book. We, together with Merck & Cie, filed a lawsuit alleging patent infringement against DRL. In September 2015, we and Merck & Cie entered into a settlement agreement with DRL (the DRL Tablet Settlement Agreement) that resolved the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral tablets. Under the terms of the DRL Tablet Settlement Agreement, we granted DRL a non-exclusive license to our Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100 mg oral tablets in the U.S. for the indications approved for Kuvan beginning on October 1, 2020, or earlier under certain circumstances.

For more information regarding these matters, see "Legal Proceedings" in Part I, Item 3 of this Annual Report on Form 10-K.

The DRL Powder Settlement Agreement, the Par Settlement Agreement, and the DRL Tablet Settlement Agreement, as well as 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 DRL 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 from DRL and Par following the settlements described above could have a material adverse effect on our revenue and results of operations.

We also face potential generic competition for Kuvan in certain foreign countries, and 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.

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 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 roxaparvec, the commercial success of valoctocogene roxaparvec 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 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 and any failure, inadequacy, interruption or security lapse of that technology, including any cybersecurity incidents, could harm our ability to operate our business effectively.

We rely significantly on our information technology and manufacturing infrastructure to effectively manage and maintain our 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, could harm our ability to operate our business effectively. Our ability to manage and maintain our 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. Cybersecurity attacks in particular are evolving and include, but are not limited to, malicious software, attempts to

gain unauthorized access to data and other electronic security breaches that could lead to disruptions in systems, misappropriation of our confidential or otherwise protected information and corruption of data. 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 of these systems, may affect our ability to manage and maintain our inventory and internal reports, and result in delays in product fulfillment and reduced efficiency of our operations. A breach in security, unauthorized access resulting in misappropriation, theft, or sabotage with respect to our proprietary and confidential information, including research or clinical data, could require significant capital investments to remediate and could adversely affect our business, financial condition and results of operations.

If a natural disaster or terrorist or criminal activity caused significant damage to our facilities or the facilities 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.

We manufacture Aldurazyme, Brineura, Naglazyme and a portion of Vimizim in a manufacturing facility located near known earthquake fault zones, and 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 our ability to manufacture Aldurazyme, Brineura, Naglazyme and Vimizim or our third-party manufacturers' ability to manufacture Firdapse or Kuvan.

Our Galli Drive facility located in Novato, California is currently our only manufacturing facility for Aldurazyme and Naglazyme and is one of two manufacturing facilities for Brineura and Vimizim. It is located in the San Francisco Bay Area near known earthquake fault zones and is 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, power loss 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 Aldurazyme, Brineura, Naglazyme and Vimizim, or to have Firdapse or Kuvan manufactured, could be seriously, or potentially completely, impaired, and our commercialization efforts and revenue could be seriously impaired. 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.

The impact of the recently passed U.S. comprehensive tax reform bill on us is uncertain and could have a material adverse effect on our business and financial condition.

On December 22, 2017, the U.S. President signed into law the Tax Cuts & Jobs Act new legislation that significantly revises the Internal Revenue Code of 1986, as amended. The newly enacted federal income tax law, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the deduction for net operating losses to 80% of current year taxable income and elimination of net operating loss carrybacks, one time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, creation of a base erosion and anti-abuse tax and modification or repeal of many business deductions and credits (including reduction of tax credits under the Orphan Drug Act). Many aspects of the new federal tax law are unclear and may not be clarified for some time. We have estimated the impact of the Tax Cuts & Jobs Act by incorporating assumptions made based upon our current interpretation and analysis to date of the law. The actual impact of the Tax Cuts & Jobs Act may differ from our estimates due to, among other things, further refinement of our calculations, changes in interpretations and assumptions we have made, guidance that may be issued and actions we may take as a result of the new legislation. Notwithstanding the reduction in the corporate income tax rate, it is possible that the Tax Cuts & Jobs Act, or regulations or interpretations under it, could adversely affect our business and financial condition, and such effect could be material. In addition, it is uncertain if and to what extent various U.S. states will conform to the newly enacted federal tax law.

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.

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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.

#### 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 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 relating to our settlements with DRL and Par 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;
- 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 order for our products, in particular in Latin America, where governments place large periodic orders for Naglazyme and Vimizim;
- changes in assessments of us 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.

In the past, following periods of large price declines in the public market price of a company's securities, securities class action litigation has often been initiated against that company. Litigation of this type could result in substantial costs and diversion of management's attention and resources, which would hurt our business. Any adverse determination in litigation could also subject us to significant liabilities. 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 to the extent we deliver shares upon conversion of any of the Notes. The Notes may become in the future convertible at the option of their holders prior to their scheduled terms under certain circumstances. Any sales in the public market of the common stock issuable upon such conversion could adversely affect prevailing market prices of our common stock. In addition, the existence of the Notes may encourage short selling by market participants because the conversion of the Notes could be used to satisfy short positions, or anticipated conversion of the Notes into shares of our common stock could depress the price of our common stock.

The capped call transactions may affect the value of the Notes and our common stock.

In connection with the issuance of the 2018 Notes and 2020 Notes, we entered into capped call transactions with respect to 50% of the principal amount of the 2018 Notes and 50% of the principal amount of the 2020 Notes with certain hedge counterparties. The capped call transactions will cover, subject to customary anti-dilution adjustments, the aggregate number of shares of common stock underlying 50% of the principal amount of the relevant Notes and are expected generally to reduce potential dilution to the common stock upon conversion of the relevant Notes in excess of the principal amount of such converted Notes. In connection with establishing their initial hedges of the capped call transactions, the hedge counterparties (or their affiliates) entered into various derivative transactions with respect to the common stock concurrently with, and/or purchased the common stock shortly after, the pricing of the relevant notes. The hedge counterparties (or their affiliates) are likely to modify their hedge positions by entering into or unwinding various derivative transactions with respect to the common stock and/or by purchasing or selling the common stock or other securities of ours in secondary market transactions prior to the maturity of the relevant Notes (and are likely to do so during the settlement averaging period under the relevant capped call transactions, which precedes the maturity date of the relevant Notes, and on or around any earlier conversion date related to a conversion of the relevant Notes).

The effect, if any, of any of these transactions and activities on the market price of our common stock or the Notes will depend in part on market conditions and cannot be ascertained at this time, but any of these activities could adversely affect the value of our common stock, which could affect the value of the Notes and the value of our common stock, if any, that Note holders receive upon any conversion of the Notes.

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

We are incorporated in Delaware. Certain anti-takeover provisions of Delaware law and our charter documents as currently in effect may make a change in control of us more difficult, even if a change in control would be beneficial to the stockholders. Our anti-takeover provisions include provisions in our restated certificate of incorporation and amended and restated bylaws, as amended, 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, as amended, provide that the Court of Chancery of the State of Delaware will be the exclusive forum 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, as amended, provide that the Court of Chancery of the State of Delaware is the sole and exclusive forum for:

- any derivative action or proceeding brought on our behalf;
- any action asserting a claim of breach of a fiduciary duty owed by any director, officer or other employee of BioMarin to us or our stockholders;
- any action asserting a claim 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, as amended; and
- any action asserting a claim against us or any of our directors, officers or other employees that is governed by the internal affairs doctrine.

This exclusive-forum provision further provides 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 provision, including consent to the personal jurisdiction of the Court of Chancery of the State of Delaware related to any action covered by such provision.

This exclusive-forum provision 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 this exclusive-forum provision to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving the dispute in other jurisdictions, which could seriously harm our business.

#### Item 1B. Unresolved Staff Comments

None.

#### Item 2. Properties

The following table contains information about our current significant owned and leased properties as of December 31, 2017:

Location	Approximate Square Feet	Use	Lease Expiration Date
San Rafael facility, San Rafael, California	391,700	Corporate headquarters, laboratory and office	NA: owned property
Several leased locations in Novato, California	225,000	Office, laboratory and warehouse	2018-2021
Shanbally facility, Cork, Ireland	183,200	Manufacturing, laboratory and office	NA: owned

			property NA: owned
Galli Drive facility, Novato, California	98,200	Clinical and commercial manufacturing and laboratory	property NA: owned
Bel Marin Keys facility, Novato, California	83,000	Office and laboratory	property NA: owned
Digital Drive facility, Novato, California	47,000	Office and laboratory	property NA: owned
Leveroni Drive facility, Novato, California	38,300	Manufacturing	property
Leiden, The Netherlands	22,900	Office and laboratory	2018
London, England	22,600	Office	2025
Dublin, Ireland	17,500	Office	2024

In addition to the above, we also maintain small offices in a variety of locations around the world. We expect our facilities to be adequate for our operations for the foreseeable future. We believe that, to the extent required, we will be able to lease or buy additional facilities at commercially reasonable rates. We plan to use

contract manufacturing when appropriate to provide product for both clinical and commercial requirements until such time as we believe it prudent to develop additional in-house clinical and/or commercial manufacturing capacity.

### Item 3. Legal Proceedings

#### Paragraph IV Notices

We received a paragraph IV notice letter, dated December 23, 2016, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL), notifying us that DRL had filed an abbreviated new drug application (ANDA) seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral powder prior to the expiration of our patents listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). We filed a lawsuit alleging patent infringement against DRL. In August 2017, we entered into a settlement agreement with DRL (the DRL Powder Settlement Agreement) that resolved the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral powder. Under the terms of the DRL Powder Settlement Agreement, we granted DRL a non-exclusive license to our Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride in oral powder form in 100 mg and 500 mg packet formulations in the U.S. for the indications approved for Kuvan beginning on October 1, 2020, or earlier under certain circumstances.

We also received two separate paragraph IV notice letters, dated January 14, 2016 and January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying us that Par had filed an ANDA seeking approval of proposed generic versions of Kuvan 100 mg oral powder and Kuvan 100 mg oral tablets, respectively, prior to the expiration of our patents listed in the FDA's Orange Book. We filed two lawsuits alleging patent infringement against Par (the lawsuit against Par pertaining to the proposed generic version of Kuvan 100 mg oral tablets was filed together with Merck & Cie), and the two Par cases were consolidated. In April 2017, we and Merck & Cie entered into a settlement agreement with Par (the Par Settlement Agreement) that resolved both cases against Par. Under the Par Settlement Agreement, we granted Par a non-exclusive license to our Kuvan-related patents to allow Par to market a generic version of sapropterin dihydrochloride in 100 mg oral tablets and oral powder in 100 mg and 500 mg packet formulations in the U.S. for the indications approved for Kuvan beginning on: April 1, 2021 if Par is not entitled to the statutory 180-day first filer exclusivity period; October 1, 2020 if Par is entitled to the statutory 180-day first filer exclusivity period; or earlier under certain circumstances.

We also received a paragraph IV notice letter, dated October 3, 2014, from DRL notifying us that DRL had filed an ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book. We, together with Merck & Cie, filed a lawsuit alleging patent infringement against DRL. In September 2015, we and Merck & Cie entered into a settlement agreement with DRL (the DRL Tablet Settlement Agreement) that resolved the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral tablets. Under the terms of the DRL Tablet Settlement Agreement, we granted DRL a non-exclusive license to our Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100 mg oral tablets in the U.S. for the indications approved for Kuvan beginning on October 1, 2020, or earlier under certain circumstances.

### 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. The following table sets forth the range of high and low quarterly sales prices for our common stock for the periods noted, as reported by Nasdaq.

Year	Period	Prices	
		High	Low
2017	Fourth Quarter	\$96.05	\$80.10
	Third Quarter	\$95.45	\$80.29
	Second Quarter	\$100.51	\$85.45
	First Quarter	\$95.79	\$82.57
2016	Fourth Quarter	\$98.34	\$78.42
	Third Quarter	\$102.49	\$77.04
	Second Quarter	\$94.08	\$73.45
	First Quarter	\$105.61	\$62.12

On February 13, 2018, the last reported sale price on the Nasdaq Global Select Market for our common stock was \$81.57. 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 three years ended December 31, 2017.

## Issuer Purchases of Equity Securities

We did not make any purchases of our common stock during the year ended December 31, 2017.

## Holders

As of February 13, 2018, there were 46 holders of record of 176,072,261 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 (U.S.) and the Nasdaq Biotechnology Index, assuming the investment of \$100 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.

\*\$100 invested on December 31, 2012 in stock or index, including reinvestment of dividends.

	Fiscal Year Ending December 31,					
	2012	2013	2014	2015	2016	2017
BioMarin Pharmaceutical Inc.	\$ 100.00	\$ 142.99	\$ 183.74	\$ 212.93	\$ 168.37	\$ 181.24
Nasdaq Composite	100.00	141.63	162.09	173.33	187.19	242.29
Nasdaq Biotechnology	100.00	174.05	230.33	244.29	194.95	228.29

## Item 6. Selected Consolidated Financial Data

We derived the selected consolidated statements of operations data for the years ended December 31, 2017, 2016 and 2015 and the selected consolidated balance sheet data as of December 31, 2017 and 2016 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, 2014 and 2013 and the selected consolidated balance sheet data as of December 31, 2015, 2014 and 2013 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)				
	2017 (1)	2016 (2)	2015	2014	2013
<b>Consolidated Statements of Operations data:</b>					
Total revenues <sup>(3)</sup>	\$1,313.6	\$1,116.9	\$889.9	\$749.3	\$548.5
Total costs and expenses <sup>(3)</sup>	\$1,328.3	\$1,920.3	\$1,000.6	\$842.2	\$704.5
Loss from operations	\$(14.7 )	\$(803.4 )	\$(110.7 )	\$(92.9 )	\$(156.0)
<b>Provision for (benefit from) income</b>					
taxes	\$81.2	\$(200.8 )	\$17.1	\$9.1	\$(0.2 )
Net loss	\$(117.0 )	\$(630.2 )	\$(171.8 )	\$(134.0)	\$(176.4)
Net loss per share, basic	\$(0.67 )	\$(3.80 )	\$(1.07 )	\$(0.92 )	\$(1.28 )
Net loss per share, diluted	\$(0.67 )	\$(3.81 )	\$(1.07 )	\$(0.92 )	\$(1.28 )
<b>Weighted average common shares</b>					
outstanding, basic	174.4	166.0	160.0	146.3	137.8
<b>Weighted average common shares</b>					
outstanding, diluted	174.4	166.2	160.0	146.3	137.8
	December 31,				
	(In millions)				
	2017	2016 (4)	2015	2014	2013
<b>Consolidated Balance Sheets data:</b>					
Cash, cash equivalents and investments <sup>(5) (6)</sup>	\$1,781.7	\$1,362.4	\$1,018.3	\$1,043.0	\$1,052.4
Total assets <sup>(1)</sup>	\$4,633.1	\$4,023.7	\$3,729.4	\$2,475.4	\$2,225.5
Other long-term obligations	\$194.4	\$157.3	\$220.8	\$68.8	\$64.2
Long-term convertible senior notes, net <sup>(6)</sup>	\$813.5	\$660.8	\$662.3	\$642.9	\$637.0
Total stockholders' equity	\$2,808.7	\$2,766.3	\$2,400.8	\$1,527.9	\$1,341.0

(1) Certain Consolidated Statement of Operations and Consolidated Balance Sheet data for the year ended December 31, 2017 included an incremental income tax expense of \$42.3 million driven by to the Tax Cuts and Jobs Act (the 2017 Tax Act), enacted in December 2017. See Note 15 to our accompanying Consolidated Financial Statements for additional information.



- (2) In January 2016, we adopted Accounting Standards Update No. 2016-09, Compensation-Stock Compensation (Topic 718) “Improvement to Employee Share-based Payment Accounting” (ASU 2016-09), which required us to record, among other items, excess tax benefits as a reduction of the provision for income taxes in the income statements. We were required to reflect any adoption adjustments as of January 1, 2016, the beginning of the annual period that includes the interim period of adoption. As such, certain Consolidated Statements of Operations data for the year ended December 31, 2016 included the impact of the ASU 2016-09 adoption.
- (3) See “Management's Discussion and Analysis of Financial Condition and Results of Operations” in Part II, Item 7 of this Annual Report on Form 10-K for a description of our results of operations for 2017.
- (4) Certain Consolidated Balance Sheets data as of December 31, 2016 include the impact of ASU 2016-09, which we early adopted in 2016. For instance, the net cumulative-effect adjustment of \$131.3 million decrease to Accumulated Deficit, which was recorded as of January 1, 2016, mostly related to the recognition of the previously unrecognized excess tax benefits using the modified retrospective method.
- (5) See “Management's Discussion and Analysis of Financial Condition and Results of Operations— Financial Position, Liquidity and Capital Resources” in Part II, Item 7 of this Annual Report on Form 10-K for additional discussion.

(6) During 2017 and 2013, we issued convertible senior notes in registered offerings with principal amounts of \$495.0 million and \$750.0 million, respectively.

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,
2017:				
Total revenues	\$303.7	\$317.4	\$ 334.1	\$ 358.3
Net loss <sup>(1)</sup>	\$(16.3)	\$(36.8)	\$ (12.5)	\$ (51.4)
Net loss per share, basic <sup>(1)</sup>	\$(0.09)	\$(0.21)	\$ (0.07)	\$ (0.29)
Net loss per share, diluted <sup>(1)</sup>	\$(0.09)	\$(0.21)	\$ (0.07)	\$ (0.30)
2016:				
Total revenues	\$236.7	\$300.1	\$ 279.9	\$ 300.1
Net loss <sup>(2)</sup>	\$(83.1)	\$(419.0)	\$ (37.4)	\$ (90.7)
Net loss per share, basic and diluted <sup>(2)</sup>	\$(0.51)	\$(2.58)	\$ (0.22)	\$ (0.53)

(1) The fourth quarter of 2017 included an incremental income tax expense of \$42.3 million related to the 2017 Tax Act. See Note 15 to our accompanying Consolidated Financial Statements for additional information.

(2) In the second quarter of 2016, we recorded an impairment charge of \$599.1 million related to the Kyndrisa and other exon and reveglucosidase alfa in-process research and development (IPR&D) assets based on the termination of the internal development of the respective programs. See “Management’s Discussion and Analysis of Financial Condition and Results of Operations— Financial Position, Liquidity and Capital Resources” in Part II, Item 7 of this Annual Report on Form 10-K for additional discussion.

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

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

### Overview

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 therapy portfolio consists of six commercial products and multiple clinical and pre-clinical product candidates. Our commercial products are Aldurazyme (laronidase) for Mucopolysaccharidosis I (MPS I), Brineura (cerliponase alfa) for the treatment of late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), Firdapse (amifampridine phosphate) for Lambert Eaton Myasthenic Syndrome (LEMS), Kuvan (sapropterin dihydrochloride) for phenylketonuria (PKU), Naglazyme (galsulfase) for Mucopolysaccharidosis VI (MPS VI) and Vimizim (elosulfase alpha) for Mucopolysaccharidosis IV Type A (MPS IV A).

### Business Developments

We continued to grow our commercial business and advance our product candidate pipeline during 2017. 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 in 2017:

#### Product Approvals

Brineura was approved to treat children with CLN2 disease by the U.S. Food and Drug Administration (FDA) in April 2017 and by the European Commission (EC) in June 2017. We began marketing Brineura in the U.S. and EU following approval in each of these markets.

#### Continued Emphasis on Research and Development

The FDA completed its review of the Investigational New Drug (IND) application for valoctocogene roxaparvovec (formerly referred to as BMN 270), an investigational gene therapy treatment for severe hemophilia A, concluded that we could proceed with its clinical development, and granted valoctocogene roxaparvovec Breakthrough Therapy Designation in October 2017. The European Medicines Agency (EMA) has granted its Priority Medicines (PRIME) designation to valoctocogene roxaparvovec. We initiated the first of two global Phase 3 program in the fourth quarter of 2017. In early 2018, we plan to enroll the first patient in the second global Phase 3 program and will begin another Phase 1/2 Study with the 6e13kg/vg dose.

•We selected our next product development candidate, BMN 290, a selective chromatin modulation therapy intended for treatment of Friedreich's ataxia, a rare autosomal recessive disorder that results in disabling neurologic and cardiac progressive decline. We expect to submit IND application in the second half of 2018.

•The FDA accepted for Priority Review the Biologics License Application (BLA) for pegvaliase, a PEGylated phenylalanine-metabolizing enzyme product, to reduce blood phenylalanine (Phe) levels in adult patients with PKU who have uncontrolled blood Phe levels on existing management. The Prescription Drug User Fee Act (PDUFA) target action date has been extended by three months to

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Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

May 28, 2018, which was changed to May 25, 2018 due to the Memorial Day holiday. We intend to submit a Marketing Authorization Application (MAA) to the EMA in the first quarter of 2018.

• We continued our ongoing trials for vosoritide for the treatment of children with achondroplasia, including a randomized, placebo-controlled Phase 3 study of vosoritide in approximately 110 children with achondroplasia 52 weeks and a long-term open-label Phase 2 study of approximately 23 children. In 2018, we expect to initiate an infant/toddler study and continue the natural history program to augment existing studies.

• We announced positive, preliminary results from a multicenter, international Phase 1/2 clinical trial for BMN 250, which began enrolling patients in April 2016. The study demonstrated that BMN 250 reduced heparan sulfate levels to normal range in cerebral spinal fluid of MPS IIIB patients and indicated that ICV-administered BMN 250 is well-tolerated by MPS IIIB patients. A complementary observational study was also initiated to study the progression of MPS IIIB over time.

Other 2017 Developments

• We executed a license agreement and a settlement agreement (the Sarepta Agreements) with Sarepta Therapeutics (Sarepta) that provide Sarepta with global exclusive rights to our Duchenne muscular dystrophy (DMD) patent estate for EXONDYS 51 and all future exon-skipping products. The Sarepta Agreements resolved the ongoing worldwide patent proceedings related to the use of EXONDYS 51 and all future exon-skipping products for the treatment of DMD. See Note 21 to our accompanying Consolidated Financial Statements for additional information.

• We commissioned our commercial gene therapy manufacturing facility, located in Novato, California, and began current Good Manufacturing Practices (GMP) production of valoctocogene roxaparvec to support clinical development activities and anticipated commercial demand.

• We entered into a settlement agreement with Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL) that resolves the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral powder. Please see "Legal Proceedings" included in Part I, Item 3 of this Annual Report on Form 10-K, for additional information.

• We entered into a settlement agreement with Par Pharmaceutical (Par) that resolves patent litigation in the U.S. with Par related to our Kuvan (sapropterin dihydrochloride) 100 mg oral tablets and oral powder in 100 mg and 500 mg packet formulations. Please see "Legal Proceedings" included in Part I, Item 3 of this Annual Report on Form 10-K, for additional information.

• We issued \$495.0 million in aggregate principal of 0.599% senior subordinated convertible notes due in 2024 (the 2024 Notes), which resulted in net proceeds of \$481.7 million.

Outlook 2018

In 2018, 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 pipeline of new product candidates. 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 Brineura and Vimizim and the international expansion of Kuvan.

We continue to monitor conditions in the macroeconomic environment that could affect our ability to achieve our goals, such as changes in the reimbursement and payer landscape, a worsening of economic conditions in certain key markets, particularly in Europe, patent expirations 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 2018 operating objectives.

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## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

## 2017 Financial Highlights

Key components of our results of operations include the following (in millions):

	Years Ended December 31,		
	2017	2016	2015
Net product revenues	\$1,270.4	\$1,110.4	\$884.5
Cost of sales	\$241.8	\$209.6	\$152.0
R&D expense	\$610.8	\$661.9	\$634.8
Selling, general and administrative (SG&A) expense	\$554.3	\$476.6	\$402.3
Intangible asset amortization and			
contingent consideration expense	\$46.5	\$(27.0 )	\$(17.7 )
Impairment of intangible assets	\$—	\$599.1	\$198.7
Net loss	\$(117.0 )	\$(630.2 )	\$(171.8)

See “Results of Operations” below for a discussion of the detailed components and analysis of the amounts noted above.

Total Revenues include net product revenues and royalty and other revenues. Net Product Revenues include revenues generated from our approved products. In the U.S., our commercial products are generally sold to specialty pharmacies or end-users, such as hospitals, which act as retailers. Outside the U.S., our commercial products are sold to our authorized distributors or directly to government purchasers or hospitals, which act as the end-users. Royalty and Other Revenues include royalties on net sales of products to licensees or sublicensees, collaborative agreement revenues and rental income associated with the tenants in our San Rafael, California facility.

Our cash, cash equivalents and investments totaled \$1.8 billion as of December 31, 2017, compared to \$1.4 billion as of December 31, 2016. 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 GAAP in the U.S. and pursuant to the rules and regulations promulgated by the Securities and Exchange Commission (the SEC), we make assumptions, judgments and estimates that can have a significant impact on our net income/loss and affect the reported amounts of certain assets, liabilities, revenue and expenses, and related disclosures. On an ongoing basis, we evaluate our estimates and discuss our critical accounting policies and estimates with the Audit Committee of our Board of Directors. We base our estimates on historical experience and various other assumptions that we believe to be reasonable under the circumstances. Actual results could differ materially from these estimates under different assumptions or conditions.

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 following critical accounting policies reflect the more significant judgments and estimates used in the preparation of our Consolidated Financial Statements:

• Revenue Recognition and Related Allowances;

• Inventory;

- Valuation of Goodwill and Acquired Intangible Assets;

• Valuation of Contingent Acquisition Consideration Payable; and

• Income Taxes.

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## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

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 persuasive evidence of an arrangement exists, delivery has occurred, the price to the buyer is fixed or determinable and collection from the customer is reasonably assured. 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. These are generally referred to as gross to net deductions and are recorded in the same period the related sales occur. Rebates, distributor fees and cash discounts represent the majority of our gross to net deduction and require complete and significant judgment by management. Estimates are assessed each period and updated to reflect current information.

**Revenue Related Allowances** – Our revenue related allowances include rebates, distributor fees and cash discounts. Rebates include amounts paid to Medicaid, other government programs, certain managed care providers, as well as foreign government rebates. Rebates, distributor fees and cash discounts are based on contractual arrangements or statutory requirements which may vary by product and payer.

Our allowance for government and other rebates is estimated based on products sold, customer mix, and program requirements. We evaluate our customer mix to estimate which sales will be subject to rebates 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 and record any necessary adjustments to our reserves. We record fees paid to distributors and cash discounts as a reduction of revenue based on contractual terms and product sold.

We believe the methodologies that we use to estimate allowances are reasonable and appropriate given the facts and circumstances. However, actual results may differ significantly from our estimates.

The following table summarizes the consolidated activities and ending balances in our revenue related allowances (in millions):

Accrued rebates, cash discounts and distributor fees	Balance at Beginning of Year	Decrease/(Increase) to Product Sales	Payments	Balance at End of Year
Year ended December 31, 2017:				
Activity related to 2017 sales	\$ —	\$ 112.3	\$(73.0 )	\$ 39.3
Activity related to sales prior to 2017	43.4	(3.8 )	(27.1 )	12.5
<b>Total</b>	<b>43.4</b>	<b>108.5</b>	<b>(100.1 )</b>	<b>51.8</b>
Year ended December 31, 2016:				
Activity related to 2016 sales	—	86.6	(58.4 )	28.2
Activity related to sales prior to 2016	39.2	(5.4 )	(18.6 )	15.2
<b>Total</b>	<b>\$ 39.2</b>	<b>\$ 81.2</b>	<b>\$(77.0 )</b>	<b>\$ 43.4</b>

## Inventory

When future commercialization for a product candidate is probable and management expects to realize economic benefit in the future, we capitalize pre-launch inventory costs prior to regulatory approval. For inventories that are capitalized in preparation of product launch, management considers a number of factors, including the product candidate's current status in the 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, commercialization and market trends.

Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

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.

#### 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 future cash flows from product sales;
- estimating the time and resources needed to complete the development and approval of product candidate;
- 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 perform a qualitative assessment of goodwill and indefinite lived assets to determine if it is more likely than not that the fair value of our single reporting unit or other indefinite lived assets is less than its carrying value. If it is determined that the fair value is more likely than not less than its carrying value, we will perform the two step impairment test. In the first step of the impairment review, we compare the carrying value of our single reporting unit or applicable asset to its fair value, which management estimates using a discounted cash flow analysis. If the carrying value of our single reporting unit or asset exceeds its fair value, we perform the second step, and determine the impairment loss, if any, as the excess of the carrying value of the goodwill or indefinite lived intangible asset over its fair value.

#### Valuation of Contingent Acquisition Consideration Payable

Significant estimates and judgements 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 acquisition consideration payments utilizing a probability-based income approach inclusive of an estimated discount rate. Each period we reassess the fair value of the contingent acquisition consideration payable 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 acquisition consideration payable 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 acquisition consideration payable.

We believe the fair value used to record contingent acquisition consideration payable incurred in connection with a 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

## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

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

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. We assess our ability to realize our deferred tax benefits at the end of each period. If we determine it is more likely than not that we will not realize the deferred tax benefits, valuation allowance may need to be established against all or a portion of our deferred tax assets. Changes in our valuation allowance will result in a change to tax expense.

We are subject to income taxes in the United States 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. Management is not aware of any potential changes that would have a material effect on our Consolidated Financial Statements, except for the Tax Cuts and Jobs Act (the 2017 Tax Act), which was enacted in December 2017. We have estimated the impact of the 2017 Tax Act by incorporating assumptions made based upon our current interpretation and analysis to date of the law. The actual impact of the 2017 Tax Act may differ from our estimates due to, among other things, further refinement of our calculations, changes in interpretations and assumptions we have made, guidance that may be issued and actions we may take as a result of the new legislation.

## 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.

## Results of Operations

## Net Loss

Our net loss for the year ended December 31, 2017 was \$117.0 million, compared to a net loss of \$630.2 million and \$171.8 million for the years ended December 31, 2016 and 2015, respectively. The changes in Net Loss were primarily a result of the following (in millions):

	Years Ended December 31,				
	2017	2016	2015	2017 vs. 2016	2016 vs. 2015
Total revenues	\$1,313.6	\$1,116.9	\$889.9	\$ 196.7	\$ 227.0
Cost of sales	241.8	209.6	152.0	32.2	57.6
R&D expense	610.8	661.9	634.8	(51.1 )	27.1

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SG&A expense	554.3	476.6	402.3	77.7	74.3
Intangible asset amortization and					
contingent consideration	46.5	(27.0 )	(17.7 )	73.5	(9.3 )
Impairment of intangible assets	—	599.1	198.7	(599.1 )	400.4
Gain on sale of intangible asset	(125.0 )	—	(369.5)	(125.0 )	369.5
Other, net	(21.0 )	(27.7 )	(44.0 )	6.7	16.3
Provision for (benefit from)					
income taxes	81.2	(200.8 )	17.1	282.0	(217.9 )
Net loss	\$(117.0 )	\$(630.2 )	\$(171.8)	\$ 513.2	\$ (458.4 )

The decrease in Net Loss for the year ended December 31, 2017 was primarily attributed to the following:

- an increase in Total Revenues driven by new patients initiating therapy and the \$31.5 million net upfront license payment from Sarepta pursuant to the Sarepta Agreements;

## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

the \$125.0 million gain on the sale of the Rare Pediatric Disease Priority Review Voucher (the PRV) that we received in connection with the approval from the FDA of Brineura for the treatment of CLN2; and the absence of the \$599.1 million intangible asset impairment charge recorded in 2016 when the Kyndrisa and reveglucosidase alfa programs were terminated; partially offset by the increase in the provision for income taxes and a \$5.8 million IPR&D asset impairment charge due to the termination of related development programs.

See below for additional information related to the Net Loss fluctuations presented above, including details of our operating expense fluctuations and the aforementioned impairment charge.

## Net Product Revenues

A summary of our various commercial products, including key metrics as of December 31, 2017, is provided below:

## U.S. Biologic

Commercial Products	Indication	U.S. Orphan Drug Exclusivity		
		Expiration	Expiration	Expiration
Aldurazyme	MPS I	Expired	Expired	Expired
Brineura	CLN2	2024	2029	2027
Firdapse	LEMS	NA <sup>(1)</sup>	NA <sup>(1)</sup>	2019
Kuvan	PKU	Expired	NA	2020 <sup>(2)</sup>
Naglazyme	MPS VI	Expired	Expired	Expired
Vimizim	MPS IVA	2021	2026	2024

(1) Firdapse has not received marketing approval in the U.S. We have licensed the North American rights to develop and market Firdapse to a third party.

(2) Kuvan has been granted orphan drug status in the EU, which together with pediatric exclusivity, confers 12 years of market exclusivity in the EU that expires in 2020. Furthermore, Ares Trading S.A. (Merck Serono) marketed Kuvan in the EU until January 1, 2016. See Note 5 to our accompanying Consolidated Financial Statements for further discussion.

Net Product Revenues consisted of the following (in millions):

	Years Ended December 31,				
	2017	2016	2015	2017 vs. 2016	2016 vs. 2015
Aldurazyme	\$90.0	\$93.8	\$98.0	\$ (3.8 )	\$ (4.2 )
Brineura	8.6	—	—	8.6	—
Firdapse	18.8	18.0	16.0	0.8	2.0
Kuvan	407.5	348.0	239.3	59.5	108.7
Naglazyme	332.2	296.5	303.1	35.7	(6.6 )
Vimizim	413.3	354.1	228.1	59.2	126.0
Total net product revenues	\$1,270.4	\$1,110.4	\$884.5	\$ 160.0	\$ 225.9





## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

Our Brineura, Firdapse, Kuvan, Naglazyme and Vimizim customers include a limited number of specialty pharmacies and end-users, such as hospitals and foreign government agencies. We also sell Brineura, Kuvan, Naglazyme 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 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 quarter to quarter variation in our revenues. Genzyme Corporation (Genzyme) is our sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third parties.

The following is additional discussion of our revenues by product:

• **Aldurazyme:** The decrease in 2017 compared to 2016 and the decrease in 2016 compared to 2015 were primarily attributable to the decreases in shipments to Genzyme, offset in part by the increase in Aldurazyme revenue reported by Genzyme. Aldurazyme revenues reported by Genzyme totaled \$233.8 million, \$223.3 million and \$217.8 million in 2017, 2016 and 2015, respectively. Although Genzyme sells Aldurazyme worldwide, the net product revenues earned by us on Genzyme's net sales are denominated in USD. We will adopt Accounting Standards Update No. 2014-09, Revenue from Contracts with Customers, as amended, (commonly referred to as ASC Topic 606) on January 1, 2018. After adopting ASC Topic 606, we will recognize Aldurazyme revenues when the product is shipped to Genzyme and all required quality control certificates are complete, because our performance obligations are fulfilled at that point in time. We will record Aldurazyme net product revenues based on the estimated tiered payment that will be in effect when the product is sold through by Genzyme. We believe any differences between the estimated revenue from Genzyme and actual payments will be insignificant. See Note 4 to our accompanying Consolidated Financial Statements for additional information on the impact of ASC Topic 606 on the first quarter of 2018.

• **Brineura:** The FDA and EC granted marketing approval for Brineura in April 2017 and June 2017, respectively. We began marketing the product following approval in each of these markets with the first commercial shipments in the U.S. and EU occurring in June 2017 and July 2017, respectively.

• **Kuvan:** The increase in 2017 compared to 2016 was primarily attributable to an increase in patients on Kuvan therapy in the U.S. and the completion of the transition of the ex-North American territories acquired in 2016. The increase in 2016 compared to 2015 was primarily attributable to the addition of international Kuvan product sales through the acquisition of certain rights and other assets with respect to Kuvan and pegvaliase from Merck Serono and its affiliates (the Merck PKU Business) in January 2016 and new patients initiating therapy in the U.S. Prior to our acquisition of the Merck PKU Business, we earned royalties of 4% on Merck Serono's net sales of Kuvan. Two companies previously filed paragraph IV certifications and submitted abbreviated new drug applications (ANDAs) to produce sapropterin dihydrochloride tablets and powder. We entered into settlement agreements regarding Kuvan with both companies. Please see "Government Regulation – Hatch-Waxman Act" below and "Legal Proceedings" in Part I, Item 3 of this Annual Report on Form 10-K for additional information. Please see "Risk Factors" included in Part I, Item 1A of this Annual Report on Form 10-K for a discussion of the risks posed by generic versions of Kuvan.

• **Naglazyme:** The increase in 2017 compared to 2016 was primarily attributable to new patients initiating therapy, the positive impact of foreign currency exchange rates and the timing of central government orders from Latin America and Europe. The decrease in Naglazyme net product revenues for 2016 compared to 2015 was primarily attributable to the timing of central government orders from Latin America and the negative impact of foreign currency exchange rates, partially offset by new patients initiating therapy in Europe and the Middle East.

• **Vimizim:** The increase in 2017 compared to 2016 and in 2016 compared to 2015 was primarily attributable to new patients initiating therapy.

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 (in millions):

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## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

	Years Ended December 31,		
	2017	2016	2015
Sales denominated in USD	\$748.5	\$643.2	\$580.7
Sales denominated in foreign currencies	521.9	467.2	303.8
Total net product revenues	\$1,270.4	\$1,110.4	\$884.5

The net impact of foreign currency exchange rates on product sales denominated in currencies other than USD during 2017 was positive by \$5.5 million, compared to a negative impact of \$3.6 million during 2016 and a negative impact of \$37.3 million during 2015. 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 and Other Revenues were \$43.2 million for the year ended December 31, 2017, compared to \$6.5 million and \$5.4 million for the years ended December 31, 2016 and 2015, respectively. The increase in 2017 as compared to 2016 and 2015 was primarily due to recognition of the \$31.5 million net upfront license revenue from Sarepta and \$3.8 million in royalty revenue earned on Sarepta net sales during 2017. We expect to continue earn royalties from Sarepta's net sales under the terms of the Sarepta Agreements in future quarters.

#### Cost of Sales and Product Gross Margin

Cost of Sales includes raw materials, personnel and facility and other costs associated primarily with manufacturing Aldurazyme, Brineura, Naglazyme and Vimizim at our production facilities. Cost of Sales also includes third-party manufacturing costs for the production of the active ingredient in Kuvan and Firdapse and third-party production costs related to final formulation and packaging services for all products and cost of royalties payable to third parties for all products

The following table summarizes our cost of goods sold and product gross margin (in millions, except percentages):

	Years Ended December 31,		
	2017	2016	2015
Total net product revenues	\$1,270.4	\$1,110.4	\$884.5
Cost of sales	241.8	209.6	152.0
Product gross margin	81	% 81	% 83

Our product gross margin for the year ended December 31, 2017 remained flat compared to 2016, which decreased from 2015 primarily due to increased Naglazyme and Vimizim manufacturing costs. We expect product gross margin to remain near 80 percent 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

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studies, personnel and raw materials costs associated with manufacturing product candidates, quality control and assurance, research and development, facilities and regulatory costs

A summary of our ongoing major development programs, including key metrics as of December 31, 2017, is provided below:

Major Product Candidates in Development	Target Indication	U.S. Orphan Designation	EU Orphan Designation	Stage
				U.S. marketing authorization
Pegvaliase	PKU	Yes	Yes	regulatory review
Valoctocogene roxaparvovec	Hemophilia A <sup>(1)</sup>	Yes	Yes	Clinical Phase 3
Vosoritide	Achondroplasia	Yes	Yes	Clinical Phase 3
BMN 250	MPS IIIB <sup>(2)</sup>	Yes	Yes	Clinical Phase 1/2
BMN 290	Friedreich's Ataxia	Not applicable	Not applicable	Preclinical

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## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

(1) Hemophilia A is also called factor VIII deficiency or classic hemophilia.

(2) Sanfilippo Syndrome Type B, or mucopolysaccharidosis type IIIB (MPS IIIB).

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.

R&D expense decreased to \$610.8 million for the year ended December 31, 2017, compared to \$661.9 million and \$634.8 million for the years ended December 31, 2016 and 2015, respectively. R&D expense consisted of the following (in millions):

	Years Ended December				
	2017	2016	2015	2017 vs. 2016	2016 vs. 2015
Pegvaliase	\$122.1	\$88.6	\$74.0	\$ 33.5	\$ 14.6
Valoctocogene roxaparvovec	118.2	58.9	32.7	59.3	26.2
Vosoritide	55.1	55.8	49.4	(0.7 )	6.4
BMN 250	56.0	46.1	33.6	9.9	12.5
BMN 290	2.8	2.1	2.9	0.7	(0.8 )
Brineura	52.0	77.2	39.9	(25.2 )	37.3
Other approved products	72.1	65.0	82.0	7.1	(17.0 )
Early stage programs	65.4	55.9	39.0	9.5	16.9
Other and non-allocated	67.1	212.3	281.3	(145.2 )	(69.0 )
Total	\$610.8	\$661.9	\$634.8	\$ (51.1 )	\$ 27.1

2017 compared to 2016

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

- a decrease in R&D expense for other and non-allocated programs primarily related to R&D spending in 2016 on the Kyndrisa, other exon-skipping, and reveglucosidase alfa development programs, all of which were terminated in 2016; and

- a decrease in R&D expense related to Brineura, which was approved for marketing in the U.S. and EU in June 2017 and July 2017, respectively. During the second quarter of 2016, we evaluated the facts and circumstances supporting recoverability of pre-launch manufacturing costs related to Brineura and concluded that recoverability was probable, resulting in the decrease in R&D costs as pre-launch manufacturing costs began to be capitalized during 2016. Prior to the second quarter of 2016, Brineura pre-launch manufacturing costs incurred were expensed to R&D expense as significant uncertainty existed over the recoverability of the costs at the time; partially offset by

- an increase in clinical trial activities related to BMN 250, pegvaliase and valoctocogene roxaparvovec product candidates; and

- an increase in pre-clinical activity for our early stage programs.

During 2018, we expect our R&D spending to increase over 2017 levels due to our BMN 250, BMN 290, pegvaliase, valoctocogene roxaparvovec, and vosoritide programs progressing in their development. We also expect increased

spending on pre-clinical activities for our early development stage programs. Additionally, we expect to continue incurring R&D expense for the foreseeable future due to long-term clinical activities related to post-approval regulatory commitments for our approved products. We continuously evaluate the recoverability of costs associated with pre-launch or pre-qualification manufacturing activities, and if it is determined that recoverability is highly likely and therefore future revenues are expected, the costs subsequently incurred related to pre-launch or pre-qualification manufacturing activities for purposes of commercial sales will likely be capitalized. 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.

## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

2016 compared to 2015

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

- an increase in R&D expense related to Brineura, pegvaliase, and vosoritide due to increased clinical trial activities related to these product candidates as they advanced to later stages of development;
- an increase in R&D expense related to BMN 250 and valoctocogene roxaparvovec due to increased pre-clinical and clinical activities related to these product candidates; and
- an increase in R&D expense related to vosoritide and valoctocogene roxaparvovec due to payments totaling \$12.0 million related to achievement of certain development milestones; partially offset by
  - a decrease in R&D expense for other and non-allocated programs primarily related to talazoparib due to the completion of the sale of the assets to Medivation, Inc. in the fourth quarter of 2015.

#### 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 expense increased to \$554.3 million for the year ended December 31, 2017, compared to \$476.6 million and \$402.3 million for the years ended December 31, 2016 and 2015, respectively. SG&A expenses consisted of the following (in millions):

	Years Ended December 31,				
	2017	2016	2015	2017 vs. 2016	2016 vs. 2015
S&M expense	\$291.5	\$252.9	\$202.9	\$ 38.6	\$ 50.0
G&A expense	262.8	223.7	199.4	39.1	24.3
Total SG&A expense	\$554.3	\$476.6	\$402.3	\$ 77.7	\$ 74.3

	Years Ended December 31,				
	2017	2016	2015	2017 vs. 2016	2016 vs. 2015
S&M expense by product:					
Brineura	\$31.6	\$15.4	\$—	\$16.2	\$ 15.4
Kuvan	87.7	65.2	40.7	22.5	24.5
Naglazyme	51.3	50.9	46.8	0.4	4.1
Vimizim	77.4	66.7	56.4	10.7	10.3
Other and non-allocated	43.5	54.7	59.0	(11.2)	(4.3)
Total S&M expense	\$291.5	\$252.9	\$202.9	\$38.6	\$ 50.0

2017 compared to 2016

The increase in S&M expense primarily comprised the following:

an increase in Kuvan and Vimizim S&M expense due to continued worldwide expansion of commercial activities; and

- an increase in Brineura S&M expense primarily due to marketing expense related to the commercial launch of Brineura in 2017; partially offset by
- a decrease in other and not allocated S&M expense primarily due to the decrease in S&M expenses related to Kyndrisa and other terminated programs.

The increase in G&A expense was primarily due to increased personnel-related costs mainly due to increased headcount.

We expect SG&A expense to increase in future periods as a result of the continued commercial launch of Brineura, pre-commercialization efforts related to product candidates, the continued international expansion of Kuvan and Vimizim, and the increase in administrative support required for our expanding operations.



## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

2016 compared to 2015

The increase in S&M expense primarily comprised the following:

- an increase in Kuvan S&M expense due to expansion of worldwide commercial activities as a result of acquiring the worldwide rights to Kuvan, except for Japan, from Merck Serono on January 1, 2016;
- an increase in Naglazyme and Vimizim S&M expense due to continued expansion of our worldwide commercial activities; and
- an increase in Brineura S&M expense due to an increase in pre-commercialization marketing expense for Brineura.

G&A expense increased in 2016 as compared to 2015, primarily due to increased headcount, partially offset by the impact of foreign currency fluctuations.

#### Intangible Asset Amortization and Contingent Consideration

Changes in the fair value of contingent acquisition consideration payable result from updates to the estimated probability of achievement or assumed timing of milestones and adjustments to the discount periods and rates. Intangible asset amortization and contingent consideration expense consisted of the following (in millions):

	Years Ended December 31,		
	2017	2016	2015
Increases (decreases) in the fair value of contingent acquisition			
consideration payable	\$10.3	\$(57.2)	\$(28.5)
Amortization of intangible assets	36.2	30.2	10.8
Total intangible asset amortization and			
contingent consideration	\$46.5	\$(27.0)	\$(17.7)

2017 compared to 2016

The changes in the fair value of the contingent acquisition consideration payable were primarily attributable to changes in the estimated probability of achieving development milestones based on the current status of the related development programs as well as the passage of time. The majority of the changes in fair value of contingent acquisition consideration payable for each period presented was attributed to the following:

- the continued progress of the PKU developmental program for pegvaliase; and
- the progress of the talazoparib program currently being developed by Pfizer, Inc.; partially offset by
- the reversal of the fair value of the Firdapse FDA approval milestone due to the reduction of the estimated probability of achieving the Firdapse FDA approval milestone prior to its expiration date; and
- the termination of the Kyndrisa and reveglucosidase alfa development programs in 2016 resulted in the reversal of the fair value of the remaining contingent consideration payable to the former Prosensa Holding N.V. and Zystor Therapeutics, Inc. shareholders, respectively.

The increase in amortization of intangible assets during 2017 was primarily attributable to the impairment of IPR&D assets we acquired from Zacharon Pharmaceuticals, Inc. as the related development programs were terminated. It is our policy to report impairment charges that are not material as a component of Intangible Asset Amortization and

Contingent Consideration on our Consolidated Statements of Operations.

2016 compared to 2015

The majority of the changes in fair value of contingent acquisition consideration payable for the year ended December 31, 2016 compared to the year ended December 31, 2015 was attributed to the discontinuance of the Kyndrisa and reveglucosidase alfa development programs, which resulted in the reversal of the fair value of the

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## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

remaining contingent consideration payable to the former Prosensa and ZyStor Therapeutics, Inc. shareholders, respectively, because the related sales milestones were no longer expected to be attained.

The increase in amortization of intangible assets during 2016 was primarily attributable to the amortization of the Kuvan intangible assets acquired from Merck Serono in January 2016.

#### Impairment of Intangible Asset

No material impairment charges were recorded in 2017. In 2016, we recorded an impairment charge of \$599.1 million related to the Kyndrisa and other exon and reveglucosidase alfa IPR&D assets based on the termination of the internal development of the respective programs. In 2015, we recorded an impairment charge of \$198.7 million related to the Kyndrisa IPR&D assets based on the then current status of our U.S. development efforts and the related discounted cash flows that no longer supported the full carrying-value of the Kyndrisa IPR&D assets. See Note 7 to our accompanying Consolidated Financial Statements for additional information regarding our Intangible Assets.

#### Gain on Sale of Intangible Asset

In December 2017, we sold the PRV that we received in connection with the FDA approval of Brineura for the treatment of CLN2. In exchange for the PRV, we received lump sum payment of \$125.0 million from Novartis Pharma AG. The proceeds from the sale of PRV were recognized as a gain on the sale of intangible asset. In 2015, we recognized a net gain of \$369.5 million for the sale of talazoparib to Medivation, Inc.

#### Interest Income

We invest our cash, short-term and long-term investments in U.S. government securities and other high credit quality debt securities in order to limit default and market risk. Interest income was \$14.9 million for the year ended December 31, 2017, compared to \$7.5 million and \$4.5 million for the years ended December 31, 2016 and 2015, respectively. The increase in interest income during 2017 compared to 2016 was primarily due to higher investment balances, which increased due to the investment of the net proceeds of \$481.7 million from our August 2017 issuance of the 2024 Notes and higher average interest rate on investments. Due to higher investment balances offset in part by planned spend, during 2018 we expect an increase in interest income from present levels.

#### Interest Expense

We incur interest expense on our convertible debt. Interest expense consisted of the following (in millions):

	Years Ended December 31,		
	2017	2016	2015
Coupon interest	\$10.4	\$9.6	\$9.8
Amortization of debt issuance costs	3.7	3.4	3.3
Accretion of discount on convertible notes	28.6	26.5	25.1
Total interest expense	\$42.7	\$39.5	\$38.2

The increased interest expense in 2017 compared to 2016 was primarily due to the August 2017 issuance of the 2024 Notes. The increased interest expense in 2016 compared to 2015 was attributable to an increase in the accretion of the discount on our 0.75% senior subordinated convertible notes due in 2018 (the 2018 Notes) and our 1.50% senior subordinated convertible notes due in 2020 (the 2020 Notes and, together with the 2018 Notes and the 2024 Notes, the Notes) which were issued in October 2013, using the effective interest rate method. We expect interest expense to increase over the next 12 months due to the coupon interest and amortization of issuance costs related to the 2024 Notes. See Note 13 to our accompanying Consolidated Financial Statements for additional information regarding our debt.

#### Other Expense

During the second quarter of 2015, we recorded write-offs of \$12.8 million for investments and advances related to a supplier of one of our multi-sourced materials due to a deterioration in its financial condition.

## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

## Provision for (Benefit from) Income Taxes

On December 22, 2017, the 2017 Tax Act was signed into law. The new law has resulted in significant changes to the U.S. corporate income tax system. These changes include a federal statutory rate reduction from 35% to 21% and the elimination or reduction of certain domestic deductions and credits, including a 50% reduction in the orphan drug credit benefit. The 2017 Tax Act changed U.S. international taxation from a worldwide basis to a modified territorial system that includes base erosion prevention measures on foreign earnings. This will result in our foreign subsidiaries being subject to U.S. taxation in the future. These changes are effective in 2018.

We recognized an income tax provision of \$81.2 million, an income tax benefit of \$200.8 million and an income tax provision of \$17.1 million in the years ended December 31, 2017, 2016 and 2015, respectively. Changes to tax laws and tax rates are required to be accounted for in the period of the enactment, therefore our 2017 tax provision included the impact of the 2017 Tax Act. Provision for (benefit from) income taxes for 2017, 2016 and 2015 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 provision for (benefit from) income taxes for the years ended December 31, 2017, 2016 and 2015 were further impacted by the following items:

• 2017 included a provisional expense of \$42.3 million related to the 2017 Tax Act primarily consisting of \$33.1 million for the re-measurement of the net deferred tax assets at the lower enacted corporate tax rate and \$9.2 million related to the new limitations on tax deductible compensation. Our deferred tax assets and liabilities are measured at the enacted tax rate expected to apply when these temporary differences are expected to be realized or settled.

• Additionally, we established a \$41.4 million valuation allowance on state tax credits as management assessed the impact of the 2017 Tax Act on our financial projections and concluded that it is more likely than not that these state tax credits will not be utilized in the foreseeable future because these credits do not expire and we project that we will be generating more credits than we will utilize on an annual basis. The 2017 Tax Act also includes a one-time mandatory deemed repatriation toll tax on accumulated earnings of our foreign subsidiaries that did not impact us due to a net deficit in these foreign subsidiaries;

• 2016 included a deferred tax benefit of \$143.5 million associated with the GAAP impairment of the Kyndrisa IPR&D; and

• 2015 included a deferred tax benefit of \$49.7 million associated with the GAAP impairment of the Kyndrisa IPR&D, which was partially offset by a \$29.7 million increase in the valuation allowance related to future contingent consideration on the sale of talazoparib that is reasonably uncertain of receipt.

The 2017 provisional charge is an estimate and the measurement of deferred tax assets is subject to further analysis and potential correlative adjustments as developing interpretations and guidance from the U.S. Treasury Department, the IRS, and other standard setting bodies provide additional clarifications of the provisions of the 2017 Tax Act. Updated guidance and regulations could result in changes to this estimate during 2018 when the analysis is complete. We have not yet elected an accounting method regarding whether to record deferred tax assets and liabilities for expected amounts of Global Intangible Low-Taxed Income (GILTI) inclusions or whether to treat such amounts as a period cost.

The consolidated GAAP net loss includes all of our foreign subsidiaries. In accordance with Accounting Standards Codification Topic 740, "Income Taxes," we calculate our provision for (benefit from) income taxes on an entity-by-entity and jurisdiction-by-jurisdiction basis as adjusted for differences between book-basis income and tax-basis income, which results in certain foreign entities being profitable and incurring foreign current income tax expense. Certain foreign entities incur significant amounts of R&D expense that results in significant losses that more than offset the income reported by the profitable foreign entities on a consolidated basis. The majority of these

material R&D losses are in foreign jurisdictions that do not have net operating loss carryforward provisions that result in deferred tax assets, which results in an effective tax rate of 0% on approximately \$53.5 million of foreign net losses during 2017. For the year ended December 31, 2017, our Dutch operations had GAAP income of \$20.7 million. For the year ended December 31, 2017, other foreign operations generated GAAP income of approximately \$16.3 million with an effective tax rate of approximately 22%.

#### Financial Position, Liquidity and Capital Resources

As of December 31, 2017, we had \$1.8 billion in cash, cash equivalents, and short-term and long-term investments. We expect to fund our operations with our net product revenues from our commercial products, cash, cash equivalents and investments, supplemented as may become necessary by proceeds from equity or debt financings and loans, or collaborative agreements with corporate partners. We may require additional financing to fund the repayment of our convertible debt, future milestone payments and our future operations,

## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

including the commercialization of our products and product candidates currently 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.

In managing our liquidity needs in the U.S., we do not rely on unrepatriated earnings as a source of funds and we have not provided for U.S. federal or state income taxes on these undistributed foreign earnings. We do not record U.S. tax expense on the undistributed earnings of our controlled foreign subsidiaries as these earnings are intended to be indefinitely reinvested offshore. As of December 31, 2017, the cumulative amount of these earnings was approximately \$10.7 million.

As of December 31, 2017, \$187.9 million of our \$1.8 billion balance of cash, cash equivalents and investments was held in foreign subsidiaries, a significant portion of which is required to fund the liquidity needs of these foreign subsidiaries. See Note 15 to our accompanying Consolidated Financial Statements for additional discussion.

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 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 were as follows (in millions):

	2017	2016	2015	2017 vs. 2016	2016 vs. 2015
Cash and cash equivalents	\$598.0	\$408.3	\$397.0	\$ 189.7	\$ 11.3
Short-term investments	797.9	381.3	195.6	416.6	185.7
Long-term investments	385.8	572.8	425.7	(187.0 )	147.1
Cash, cash equivalents and investments	\$1,781.7	\$1,362.4	\$1,018.3	\$ 419.3	\$ 344.1
Convertible debt, net	\$1,174.5	\$683.2	\$662.3	\$ 491.3	\$ 20.9

Our cash flows for each of the years ended December 31 are summarized as follows (in millions):

	2017	2016	2015	2017 vs. 2016	2016 vs. 2015
Cash & cash equivalents at the beginning of the period	\$408.3	\$397.0	\$875.5	\$ 11.3	\$ (478.5 )
Net cash used in operating activities	(8.8 )	(227.8 )	(219.5 )	219.0	(8.3 )
Net cash used in investing activities	(305.5 )	(484.0 )	(1,179.6)	178.5	695.6

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Net cash provided by financing activities	507.1	727.1	925.7	(220.0 )	(198.6 )
Foreign exchange impact	(3.1 )	(4.0 )	(5.1 )	0.9	1.1
Cash & cash equivalents at the end					
of the period	\$598.0	\$408.3	\$397.0	\$ 189.7	\$ 11.3
Short-term and long-term investments	1,183.7	954.1	621.3	229.6	332.8
Cash, cash equivalents and investments	\$1,781.7	\$1,362.4	\$1,018.3	\$ 419.3	\$ 344.1

Cash Used in Operating Activities

Cash used in operating activities for the year ended December 31, 2017 was \$8.8 million, compared to cash used in operating activities of \$227.8 million for the year ended December 31, 2016. Cash used in operating activities primarily consisted of net loss of \$117.0 million, adjusted for non-cash items such as \$140.3 million for stock-based compensation expenses, \$87.9 million for depreciation and amortization expense, \$44.5 million for a change in deferred income taxes and \$32.3 million of non-cash interest expense, partially offset by a \$125.0 million gain on the sale of an intangible asset. Changes in operating assets and liabilities resulted in a net



## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

cash outflow of \$94.0 million that consisted primarily of increased cash outflow for increased inventory spending for all commercial products to meet anticipated future sales demand.

Cash used in operating activities for the year ended December 31, 2016 was \$227.8 million, compared to cash used in operating activities of \$219.5 million for the year ended December 31, 2015. Cash used in operating activities primarily consisted of net loss of \$630.2 million, adjusted for non-cash items such as \$599.1 million of asset impairment charges, \$134.6 million for stock-based compensation expenses, \$96.9 million for depreciation and amortization expense, and \$29.9 million of non-cash interest expense, partially offset by \$228.1 million change in deferred income taxes and \$57.2 million related to the decrease in the fair value of contingent acquisition consideration payable. Changes in operating assets and liabilities resulted in a net cash outflow of \$160.3 million that consisted primarily of increased cash outflow for R&D expenses and increased inventory spending to meet anticipated future sales demand.

#### Cash Used in Investing Activities

Net cash used in investing activities for the year ended December 31, 2017 was \$305.5 million, compared to net cash used in investing activities of \$484.0 million for the year ended December 31, 2016. The decrease in net cash used in investing activities for the year ended December 31, 2017 compared to the prior year was primarily attributable to the \$125.0 million in proceeds from the sale of the PRV and an increase of \$102.7 million in net purchases of available-for-sale securities, partially offset by a \$50.8 million increase in the purchases of property, plant and equipment. We expect to continue to make significant capital investments in our manufacturing facilities and our corporate headquarters to accommodate anticipated headcount growth.

Net cash used in investing activities for the year ended December 31, 2016 was \$484.0 million, compared to net cash used in investing activities of \$1.2 billion for the year ended December 31, 2015. The decrease in net cash used in investing activities for the year ended December 31, 2016 compared to the prior year was primarily attributable to the absence of 2015 payments of \$538.4 million to acquire Prosensa and \$371.8 million deposit for the PKU rights from Merck Serono, a \$79.3 million decrease in the purchases of property, plant and equipment, and a decrease of \$116.3 million in net purchases of available-for-sale securities.

#### Cash Provided by Financing Activities

Net cash provided by financing activities for the year ended December 31, 2017 was \$507.1 million, compared to net cash provided by financing activities of \$727.1 million for the year ended December 31, 2016. The decrease in net cash provided by financing activities for the year ended December 31, 2017 compared to the prior year was primarily attributable a \$712.9 million decrease in net proceeds from public offerings of common stock, partially offset by \$481.7 million of net proceeds from the issuance of the 2024 Notes issued in August 2017.

Net cash provided by financing activities for the year ended December 31, 2016 was \$727.1 million, compared to net cash provided by financing activities of \$925.7 million for the year ended December 31, 2015. The decrease in net cash provided by financing activities for the year ended December 31, 2016 compared to the prior year was primarily attributable a \$175.3 million decrease in net proceeds from public offerings of common stock.

Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

Other Information

Our \$1.2 billion (undiscounted) of total convertible debt as of December 31, 2017 will impact our liquidity due to the semi-annual cash interest payments. Our indebtedness consists primarily of the Notes, which, if not converted, will be required to be repaid in cash at maturity in 2018, 2020 and 2024, respectively. Our senior subordinated convertible notes due in 2017 matured on April 23, 2017, with conversion of all principal amounts except for a final cash settlement of \$26,000. See Note 13 to our accompanying Consolidated Financial Statements for additional discussion.

In the event the conditional conversion feature of the 2018 Notes or 2020 Notes is triggered, holders of such Notes will be entitled to convert the 2018 Notes or 2020 Notes at any time during specified periods at their option. In addition, the 2018 Notes will be freely convertible on or after July 15, 2018 and the 2020 Notes will be freely convertible on or after July 15, 2020. We intend to use a majority of the net proceeds we received from the issuance of the 2024 Notes to repay, repurchase or settle in cash some or all of the 2018 Notes. We may also elect to settle conversions of the 2020 Notes in cash, which could further affect our liquidity. While we could seek to obtain additional third-party financing to pay for any amounts due in cash upon such events, we cannot be sure that such third-party financing will be available on commercially reasonable terms, if at all. The \$375.0 million principal amount of the 2018 Notes mature in October 2018 and therefore we have reclassified the net outstanding principal of the 2018 Notes as a current liability. Even if holders of the 2020 Notes do not elect to convert their 2020 Notes, we could be required under applicable accounting rules to reclassify all or a portion of the outstanding principal of such Notes as a current liability rather than long-term liability (for example, when there are 12 months or less remaining until maturity), which would result in a material reduction of our net working capital.

In August 2017, we completed an offering of \$495.0 million in aggregate principal amount of the 2024 Notes, which resulted in net proceeds of \$481.7 million, after deducting commissions and offering expenses.

In August 2016, we sold 7.5 million shares of our common stock at a price of \$96.00 per share in an underwritten public offering pursuant to an effective registration statement previously filed with the SEC. We received net proceeds of approximately \$712.9 million from this public offering after accounting for the underwriting discount and offering costs.

In November 2016 we entered into a credit agreement (the Credit Agreement) providing for up to \$100.0 million in revolving loans (the Revolving Credit Facility). We expect to use the proceeds of the Revolving Credit Facility to finance ongoing working capital needs (including timing differences resulting from the strategic management of short-term investments) and for other general corporate purposes. As of December 31, 2017, we had not drawn on the Revolving Credit Facility. Although quarterly interest payments will be due on any outstanding balance due, we anticipate any balance due to be short-term in nature. 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 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:

- 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;
- If we are unable to successfully develop and maintain manufacturing processes for our products to produce sufficient quantities at acceptable costs, we may be unable to meet demand for our products and lose potential revenue, have reduced margins or be forced to terminate a program;
- 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.

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## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

•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.

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 in the period since inception of our key programs were as follows (in millions):

	Since Program Inception
Brineura	\$ 240.2
Pegvaliase	522.9
Valoctocogene roxaparvovec	239.3
Vosoritide	229.7
BMN 250	154.6
BMN 290	7.8
Other approved products	979.8
Other and non-allocated	Not meaningful

We may elect to increase our spending above our current long-term plans and consequently we may be unable 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; pre-clinical studies and clinical trials for our other 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:

- 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 relating to our settlements with DRL and Par 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; and
- developments or disputes concerning patent or proprietary rights.

#### 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.

## Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

## 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, 2017 are presented in the table below (in millions).

	Payments Due within				Total
	1 Year or Less	>1 -3 Years	> 3 - 5 Years	More Than 5 Years	
2018 Notes and related interest	\$377.8	\$—	\$—	\$—	\$377.8
2020 Notes and related interest	5.6	386.2	—	—	391.8
2024 Notes and related interest	3.0	5.9	5.9	500.9	515.7
Operating leases	9.7	13.6	8.9	5.9	38.1
R&D and purchase commitments	52.1	0.7	—	—	52.8
Total	\$448.2	\$406.4	\$14.8	\$506.8	\$1,376.2

We are also subject to contingent payments related to certain development and regulatory activities and commercial sales and licensing milestones totaling approximately \$604.6 million as of December 31, 2017, which are due upon achievement of certain development and commercial milestones, and if they occur before certain dates in the future. Of this amount, \$222.0 million (USD equivalent of €185 million translated at 1.20 USD per Euro as of December 31, 2017) relates to the Merck PKU Business acquisition and \$53.4 million relates to programs that are no longer being developed.

As of December 31, 2017, we have recorded \$189.0 million of contingent acquisition consideration payable on its Consolidated Balance Sheets in Short-term and Long-term Contingent Acquisition Consideration Payable, of which \$53.6 million is expected to be paid by us in the next twelve months.

Any outstanding amounts due under the Revolving Credit Facility will be due in full in November 2018 with related interest due on a quarterly basis. As of December 31, 2017, there was no outstanding balance.

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 and sales activities in the U.S. as well as sales activities 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 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 and various foreign currencies, primarily the Euro. When the U.S. dollar strengthens against these currencies, the relative value of the sales made in the respective foreign currency decreases. Conversely, when the U.S. dollar 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 U.S. dollar and are adversely affected by a stronger U.S. dollar relative to those foreign currencies in which we transact significant business.

During 2017, approximately 41% of our net product sales were denominated in foreign currencies and 22% of our operating expenses 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. We also hedge certain monetary assets and liabilities, primarily those denominated in Euros, using forward foreign currency exchange 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 foreign currency exchange 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, 2017 and 2016, we had open forward foreign currency exchange contracts with notional amounts of \$276.1 million and \$223.5 million, respectively. A hypothetical 10% strengthening in foreign currency exchange rates compared with the U.S. dollar relative to exchange rates at December 31, 2017 would have resulted in a reduction in the value received over the remaining life of these contracts of approximately \$29.7 million on this date and, if realized, would negatively affect earnings during the remaining life of the contracts. The same hypothetical movement in foreign currency exchange rates with the U.S. dollar relative to exchange rates at December 31, 2016, would have resulted in a reduction of the value received over the remaining life of the contracts by approximately \$21.0 million on this date and, if realized, would have negatively affect earnings during the remaining life of these 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.

Based on our overall foreign currency exchange rate exposures at December 31, 2017, we believe that a near-term 10% fluctuation of the U.S. dollar exchange rate could result in a potential change in the fair value of our foreign currency sensitive assets, excluding our investments and open forward foreign currency exchange contracts by approximately \$4.7 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. 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.

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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 \$375.0 million of the 2018 Notes, \$375.0 million of the 2020 Notes and \$495.0 million of the 2024 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, 2017, the fair value of our convertible debt was \$1.3 billion.

In connection with the October 2013 offering of the 2018 Notes and the 2020 Notes, we paid \$29.8 million to purchase a capped call covering 3,982,988 shares of our common stock. If the per share price of our common stock remains below \$94.15, these capped call transactions would be not applicable and, therefore, would provide us no benefit in offsetting potential dilution from the 2018 Notes and the 2020 Notes. If the per share price of our common stock exceeds \$121.05, then, to the extent of the excess, these capped call transactions would result in additional dilution from conversion of the 2018 Notes and the 2020 Notes.

As of December 31, 2017, our investment portfolio did not include any investments with significant exposure to countries that face economic volatility and weakness. Based on our investment portfolio and interest rates at December 31, 2017, 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 \$9.6 million. Changes in interest rates may affect the fair value of our investment portfolio. However, we will not recognize such gains or losses in our Consolidated Statements of Operations unless the investments are sold or we determine that the decline in the investment's value is other-than-temporary.

The table below summarizes the expected maturities and average interest rates of our interest-generating investments at December 31, 2017 (in millions):

	Expected Maturity							Total
	2018	2019	2020	2021	2022	Thereafter		
Available-for-sale debt securities	\$840.3	\$353.4	\$32.2	\$ —	\$ —	0.2	\$1,226.1	
Average interest rate	1.7 %	2.1 %	2.1 %	—	—	5.1 %	1.8 %	

#### 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 and is set forth on pages F-1 to F-44.

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, 2017.

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### 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, 2017. 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, 2017 was effective.

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 during our most recently completed quarter that have materially affected or are reasonably likely to materially affect our internal control over financial reporting, as defined in Rule 13a-15(f) under the Exchange Act.

Effective January 1, 2018, we adopted Accounting Standards Update No. 2014-09, Revenue from Contracts with Customers, as amended (commonly referred to as ASC Topic 606). During the year ended 2017, we implemented internal controls to ensure that we have adequately evaluated our contracts and properly assessed the impact of ASC Topic 606 on our financial statements to facilitate the adoption on January 1, 2018. We do not expect significant changes to our internal control over financial reporting due to the adoption of ASC Topic 606.

### 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

As previously reported, on November 21, 2017, we and our wholly owned subsidiary, BioMarin Commercial Ltd., entered into a definitive agreement to sell a Rare Pediatric Disease Priority Review Voucher (PRV) for a lump sum payment of \$125.0 million. The PRV was sold to Novartis Pharma AG pursuant to an Asset Purchase Agreement, which contains customary representations, warranties and covenants. We received the PRV under a U.S. Food and Drug Administration (FDA) program intended to encourage the development of treatments for rare pediatric diseases. We were awarded the PRV in April 2017 when we received approval from the FDA of Brineura for the treatment of CLN2.

The transactions contemplated by the Asset Purchase Agreement closed on December 15, 2017.

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 2018 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 2018 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 2018 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 2018 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 “Transactions with Related Persons, Promoters and Certain Control Persons,” “Review, Approval and Ratification of Transactions with Related Parties” and “Director Independence” in the proxy statement for our 2018 annual meeting of stockholders.

Item 14. Principal Accounting 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 2018 annual meeting of stockholders.

Part IV

Item 15. Exhibits, Financial Statement Schedules

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- 2.1 Purchase Agreement, dated as of November 23, 2014, among BioMarin Falcons B.V., BioMarin Pharmaceutical Inc. and Prosensa Holding N.V., previously filed with the SEC on November 26, 2014 as Exhibit 2.01 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 2.2 Amended and Restated Termination and Transition Agreement, dated as of December 23, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on January 7, 2016 as Exhibit 2.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC.
- 2.3 Termination 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 2.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC.
- 2.4 Termination and Transition Agreement, dated as of October 1, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on January 7, 2016 as Exhibit 2.3 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC.
- 2.5 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.
- 2.6 Asset Purchase Agreement between BioMarin Pharmaceutical Inc. and Medivation, Inc., dated August 21, 2015, previously filed with the SEC on October 7, 2015 as Exhibit 2.1 to the Company's Current Report on Form 8-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.
- 3.1 Restated Certificate of Incorporation of BioMarin Pharmaceutical Inc., previously filed with the SEC on June 12, 2017 as Exhibit 3.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 3.2 Amended and Restated Bylaws of BioMarin Pharmaceutical Inc., previously filed with the SEC on October 31, 2017 as Exhibit 3.2 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference.
- 4.1 Indenture dated as of March 29, 2006, between BioMarin Pharmaceutical Inc. and Wilmington Trust Company, previously filed with the SEC on March 29, 2006 as Exhibit 4.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.



- 4.2 Second Supplemental Indenture, dated as of April 23, 2007, between BioMarin Pharmaceutical Inc. and Wilmington Trust Company, previously filed with the SEC on April 23, 2007 as Exhibit 4.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 4.3 Form of 1.875% Senior Subordinated Convertible Notes due 2017, previously filed with the SEC on April 23, 2007 as Exhibit 4.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.

- 4.4 Indenture, dated as of October 15, 2013, between BioMarin Pharmaceutical Inc. and Wilmington Trust, National Association, previously filed with the SEC on October 15, 2013 as Exhibit 4.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 4.5 First Supplemental Indenture, dated as of October 15, 2013, between BioMarin Pharmaceutical Inc. and Wilmington Trust, National Association (including the form of 0.75% Senior Subordinated Convertible Notes due 2018), previously filed with the SEC on October 15, 2013 as Exhibit 4.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 4.6 Second Supplemental Indenture, dated as of October 15, 2013, between BioMarin Pharmaceutical Inc. and Wilmington Trust, National Association (including the form of 1.50% Senior Subordinated Convertible Notes due 2020), previously filed with the SEC on October 15, 2013 as Exhibit 4.3 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 4.7 Base Indenture, dated August 11, 2017, between the Company and Wilmington Trust, National Association, as Trustee, previously filed with the SEC on August 11, 2017 as Exhibit 4.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 4.8 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.
- 10.1†Form of Indemnification Agreement for Directors and Officers, previously filed with the SEC on December 19, 2016 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.2†BioMarin Pharmaceutical Inc. Amended and Restated 2006 Employee Stock Purchase Plan, as adopted on June 21, 2006 and amended on March 5, 2014, previously filed with the SEC on June 10, 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.
- 10.3†BioMarin Pharmaceutical Inc. Amended and Restated 2006 Share Incentive Plan, as adopted on May 2, 2006 and as amended and restated on April 16, 2015, previously filed with the SEC on June 15, 2015 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.4†Form of Agreement Regarding Restricted Share Units for the BioMarin Pharmaceutical Inc. 2006 Share Incentive Plan, previously filed with the SEC on May 16, 2013 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.5†Form of Amendment to Agreement Regarding Restricted Share Units for the BioMarin Pharmaceutical Inc. 2006 Share Incentive Plan, previously filed with the SEC on December 9, 2016 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.6†Amended and Restated BioMarin Pharmaceutical Inc. Nonqualified Deferred Compensation Plan, as adopted on December 1, 2005 and as amended and restated on January 1, 2009 and further amended and restated on December 19, 2013 and October 7, 2014, previously filed with the SEC on October 14, 2014 as Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.7†

Summary of Bonus Plan, previously filed with the SEC on February 27, 2009 as Exhibit 10.33 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference.

- 10.8† Amended and Restated Employment Agreement with Jean-Jacques Bienaimé effective December 13, 2016 previously filed with the SEC on December 19, 2016 as Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.9 Grant Terms and Conditions Agreement between BioMarin Pharmaceutical Inc. and Harbor-UCLA Research and Education Institute dated April 1, 1997, as amended, previously filed with the SEC on July 21, 1999 as Exhibit 10.17 to the Company's Amendment No. 3 to Registration Statement on Form S-1 (File No. 333-77701), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.
- 10.10 License Agreement dated July 30, 2004, between BioMarin Pharmaceutical Inc. and Daiichi Suntory Pharma Co., Ltd., as amended by Amendment No. 1 to License Agreement dated November 19, 2004, previously filed with the SEC on March 16, 2005 as Exhibit 10.25 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.
- 10.11 Operating Agreement with Genzyme Corporation, previously filed with the SEC on July 6, 1999 as Exhibit 10.30 to the Company's Amendment No. 2 to Registration Statement on Form S-1 (File No. 333-77701), which is incorporated herein by reference.
- 10.12 Manufacturing, Marketing and Sales Agreement dated as of January 1, 2008, by and among BioMarin Pharmaceutical Inc., Genzyme Corporation and BioMarin/Genzyme LLC previously filed with the SEC on February 28, 2008 as Exhibit 10.30 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.
- 10.13 Amended and Restated Collaboration Agreement dated as of January 1, 2008, by and among BioMarin Pharmaceutical Inc., Genzyme Corporation and BioMarin/Genzyme LLC previously filed with the SEC on February 28, 2008 as Exhibit 10.31 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.
- 10.14 Members Agreement dated as of January 1, 2008 by and among BioMarin Pharmaceutical Inc., Genzyme Corporation, BioMarin Genetics Inc., and BioMarin/Genzyme LLC previously filed with the SEC on February 28, 2008 as Exhibit 10.32 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.
- 10.15† 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.
- 10.16† Form of Stock Options Agreement for the BioMarin Pharmaceutical Inc. 2006 Share Incentive Plan. (as Amended and Restated 2010), previously filed with the SEC on August 2, 2012 as Exhibit 10.11 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference.
- 10.17† 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.

10.18†Form of Agreement Regarding Restricted Stock Units for the BioMarin Pharmaceutical Inc. 2012 Inducement Plan, previously filed with the SEC on August 2, 2012 as Exhibit 10.14 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference.

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- 10.19 Capped Call Confirmation for the 2018 Notes, dated October 8, 2013, between BioMarin Pharmaceutical Inc. and Bank of America, N.A., previously filed with the SEC on October 11, 2013 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.20 Capped Call Confirmation for the 2020 Notes, dated October 8, 2013, between BioMarin Pharmaceutical Inc. and Bank of America, N.A., previously filed with the SEC on October 11, 2013 as Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.21 Capped Call Confirmation for the 2018 Notes, dated October 8, 2013, between BioMarin Pharmaceutical Inc. and Morgan Stanley & Co. LLC, previously filed with the SEC on October 11, 2013 as Exhibit 10.3 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.22 Capped Call Confirmation for the 2020 Notes, dated October 8, 2013, between BioMarin Pharmaceutical Inc. and Morgan Stanley & Co. LLC, previously filed with the SEC on October 11, 2013 as Exhibit 10.4 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.23 Capped Call Confirmation for the 2018 Notes, dated October 8, 2013, between BioMarin Pharmaceutical Inc. and Barclays Bank PLC, previously filed with the SEC on October 11, 2013 as Exhibit 10.5 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.24 Capped Call Confirmation for the 2020 Notes, dated October 8, 2013, between BioMarin Pharmaceutical Inc. and Barclays Bank PLC, previously filed with the SEC on October 11, 2013 as Exhibit 10.6 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.25 Additional Capped Call Confirmation for the 2018 Notes, dated October 9, 2013, between BioMarin Pharmaceutical Inc. and Bank of America, N.A., previously filed with the SEC on October 11, 2013 as Exhibit 10.7 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.26 Additional Capped Call Confirmation for the 2020 Notes, dated October 9, 2013, between BioMarin Pharmaceutical Inc. and Bank of America, N.A., previously filed with the SEC on October 11, 2013 as Exhibit 10.8 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.27 Additional Capped Call Confirmation for the 2018 Notes, dated October 9, 2013, between BioMarin Pharmaceutical Inc. and Morgan Stanley & Co. LLC, previously filed with the SEC on October 11, 2013 as Exhibit 10.9 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.28 Additional Capped Call Confirmation for the 2020 Notes, dated October 9, 2013, between BioMarin Pharmaceutical Inc. and Morgan Stanley & Co. LLC, previously filed with the SEC on October 11, 2013 as Exhibit 10.10 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.29 Additional Capped Call Confirmation for the 2018 Notes, dated October 9, 2013, between BioMarin Pharmaceutical Inc. and Barclays Bank PLC, previously filed with the SEC on October 11, 2013 as Exhibit 10.11 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.

10.30 Additional Capped Call Confirmation for the 2020 Notes, dated October 9, 2013, between BioMarin Pharmaceutical Inc. and Barclays Bank PLC, previously filed with the SEC on October 11, 2013 as Exhibit 10.12 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.

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- 10.31 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.
- 10.32 Convertible Promissory Note, dated as of November 26, 2014, between Prosensa Holding N.V. and BioMarin Falcons B.V., previously filed with the SEC on November 26, 2014 as Exhibit 10.3 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.33†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.
- 10.34 Form of Contingent Value Rights Agreement, dated as of January 14, 2015, by and between BioMarin Pharmaceutical Inc., BioMarin Falcons B.V. and American Stock Transfer & Trust Company, LLC, previously filed with the SEC on January 16, 2015 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.35†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.
- 10.36†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.
- 10.37†Form of Amended and Restated Employment Agreement for the Company's Executive Officers (other than the Company's Chief Executive Officer) previously filed with the SEC on June 15, 2015 as Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.38 Settlement and License Agreement among BioMarin Pharmaceutical Inc., Merck & Cie, Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd., dated September 14, 2015, previously filed with the SEC on November 2, 2015 as Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.
- 10.39 Credit Agreement by and among BioMarin Pharmaceutical Inc., as the Borrower, Bank of America, N.A., as Administrative Agent, Swing Line Lender, L/C Issuer and a Lender, and the Lenders party thereto, dated as of November 29, 2016, previously filed with the SEC on February 27, 2017 as Exhibit 10.49 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference.
- 10.40†Form of Agreement Regarding Performance Compensation 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 by reference herein.
- 10.41 Settlement and License Agreement among BioMarin Pharmaceutical Inc., Merck & Cie and Par Pharmaceutical, Inc., dated as of April 12, 2017, previously filed with the SEC on November 13, 2017 as



Exhibit 10.1 to the Company's Amendment No. 1 to Quarterly Report on Form 10-Q/A (File No. 000-26727), which is incorporated herein by reference. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.

- 10.42† BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on June 12, 2017 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.43† Form of Stock Options Agreement for the BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on June 12, 2017 as Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.44† Form of Agreement Regarding Restricted Stock Units for the BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on June 12, 2017 as Exhibit 10.3 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.45† Form of Agreement Regarding Performance Stock Award in the Form of Restricted Stock Units for the BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on June 12, 2017 as Exhibit 10.4 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.
- 10.46† 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.
- 10.47\* Asset Purchase Agreement by and between Novartis Pharma AG, BioMarin Pharmaceutical Inc. and BioMarin Commercial Ltd., dated as of November 21, 2017.
- 21.1\* Subsidiaries of BioMarin Pharmaceutical Inc.
- 23.1\* Consent of KPMG LLP, Independent Registered Public Accounting Firm for BioMarin Pharmaceutical Inc.
- 24.1\* Power of Attorney (Included in Signature Page to this Report)
- 31.1\* Certification of Chief Executive Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
- 31.2\* Certification of Chief Financial Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
- 32.1\* Certification of Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. This Certification accompanies this report and shall not, except to the extent required by the Sarbanes-Oxley Act of 2002, be deemed filed for purposes of §18 of the Securities Exchange Act of 1934, as amended.
  
- 101.INS XBRL Instance Document
- 101.SCH XBRL Taxonomy Extension Schema Document
- 101.CAL XBRL Taxonomy Extension Calculation Document
- 101.DEF XBRL Taxonomy Extension Definition Linkbase

101.LAB XBRL Taxonomy Extension Labels Linkbase Document

101.PRE XBRL Taxonomy Extension Presentation Link Document

\* Filed herewith

Management contract or compensatory plan or arrangement

Item 16. Form 10-K Summary

None.

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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, 2018 By: /S/ DANIEL SPIEGELMAN  
Daniel Spiegelman  
Executive Vice President and 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 Daniel Spiegelman, 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:

Signature	Title	Date
/S/ JEAN-JACQUES BIENAIMÉ Jean-Jacques Bienaimé	Chairman and Chief Executive Officer (Principal Executive Officer)	February 26, 2018
/S/ DANIEL SPIEGELMAN Daniel Spiegelman	Executive Vice President and Chief Financial Officer (Principal Financial Officer)	February 26, 2018
/S/ BRIAN R. MUELLER Brian R. Mueller	Senior Vice President, Finance and Chief Accounting Officer (Principal Accounting Officer)	February 26, 2018
/S/ WILLARD H. DERE, M.D. Willard H. Dere, M.D.	Director	February 26, 2018
/S/ MICHAEL G. GREY Michael G. Grey	Director	February 26, 2018
/S/ ELAINE J. HERON Elaine J. Heron	Director	February 26, 2018
/S/ ROBERT J. HOMBACH Robert J. Hombach	Director	February 26, 2018
/S/ V. BRYAN LAWLIS V. Bryan Lawlis	Director	February 26, 2018
/S/ ALAN J. LEWIS Alan J. Lewis	Director	February 26, 2018
/S/ RICHARD A. MEIER Richard A. Meier	Lead Independent Director	February 26, 2018

/S/ DAVID PYOTT  
David Pyott

Director

February 26,  
2018

/S/ DENNIS J. SLAMON  
Dennis J. Slamon

Director

February 26,  
2018

BIOMARIN PHARMACEUTICAL INC.

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Report of Independent Registered Public Accounting Firm

To the stockholders and the 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 as of December 31, 2017 and 2016, the related consolidated statements of operations, comprehensive loss, stockholders' equity, and cash flows for each of the years in the three year period ended December 31, 2017, 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, 2017 and 2016, and the results of its operations and its cash flows for each of the years in the three year period ended December 31, 2017, 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, 2017, 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, 2018 expressed an unqualified opinion on the effectiveness of the Company's internal control over financial reporting.

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.

/s/ KPMG LLP

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

San Francisco, California  
February 26, 2018





Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders

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, 2017, 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, 2017, 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, 2017 and 2016, the related consolidated statements of operations, comprehensive loss, stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2017, and the related notes (collectively, the "consolidated financial statements"), and our report dated February 26, 2018 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 in Item 9a. 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, 2018

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## BIOMARIN PHARMACEUTICAL INC.

## CONSOLIDATED BALANCE SHEETS

December 31, 2017 and 2016

(In thousands, except share and per share amounts)

	December 31, 2017	December 31, 2016
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 598,028	\$ 408,330
Short-term investments	797,940	381,347
Accounts receivable, net	261,365	215,280
Inventory	475,775	355,126
Other current assets	74,036	61,708
Total current assets	2,207,144	1,421,791
Noncurrent assets:		
Long-term investments	385,785	572,711
Property, plant and equipment, net	896,700	798,768
Intangible assets, net	517,510	553,780
Goodwill	197,039	197,039
Deferred tax assets	399,095	446,786
Other assets	29,852	32,815
Total assets	\$ 4,633,125	\$ 4,023,690
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable and accrued liabilities	\$ 401,921	\$ 370,505
Short-term convertible debt, net	360,949	22,478
Short-term contingent acquisition consideration payable	53,648	46,327
Total current liabilities	816,518	439,310
Noncurrent liabilities:		
Long-term convertible debt, net	813,521	660,761
Long-term contingent acquisition consideration payable	135,318	115,310
Other long-term liabilities	59,105	42,034
Total liabilities	1,824,462	1,257,415
Stockholders' equity:		
Common stock, \$0.001 par value: 500,000,000 and 250,000,000		
shares authorized, respectively; 175,843,749 and 172,647,588	shares	
issued and outstanding, respectively.	176	173
Additional paid-in capital	4,483,220	4,288,113
Company common stock held by Nonqualified Deferred Compensation		
Plan (the NQDC)	(14,224 )	(14,321 )

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Accumulated other comprehensive income (loss)	(22,961 )	12,816
Accumulated deficit	(1,637,548 )	(1,520,506 )
Total stockholders' equity	2,808,663	2,766,275
Total liabilities and stockholders' equity	\$ 4,633,125	\$ 4,023,690

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

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## BIOMARIN PHARMACEUTICAL INC.

## CONSOLIDATED STATEMENTS OF OPERATIONS

Years Ended December 31, 2017, 2016 and 2015

(In thousands, except per share amounts)

	2017	2016	2015
<b>REVENUES:</b>			
Net product revenues	\$ 1,270,445	\$ 1,110,381	\$ 884,522
Royalty and other revenues	43,201	6,473	5,373
Total revenues	1,313,646	1,116,854	889,895
<b>OPERATING EXPENSES:</b>			
Cost of sales	241,786	209,620	152,008
Research and development	610,753	661,905	634,806
Selling, general and administrative	554,336	476,593	402,271
Intangible asset amortization and contingent consideration	46,471	(26,953 )	(17,690 )
Impairment of intangible assets	—	599,118	198,700
Gain on sale of intangible asset	(125,000 )	—	(369,498 )
Total operating expenses	1,328,346	1,920,283	1,000,597
<b>LOSS FROM OPERATIONS</b>	<b>(14,700 )</b>	<b>(803,429 )</b>	<b>(110,702 )</b>
Equity in the loss of BioMarin/Genzyme LLC	(1,291 )	(538 )	(817 )
Interest income	14,853	7,487	4,501
Interest expense	(42,707 )	(39,499 )	(38,244 )
Other income (expense)	7,970	4,929	(9,462 )
<b>LOSS BEFORE INCOME TAXES</b>	<b>(35,875 )</b>	<b>(831,050 )</b>	<b>(154,724 )</b>
Provision for (benefit from) income taxes	81,167	(200,840 )	17,075
<b>NET LOSS</b>	<b>\$(117,042 )</b>	<b>\$(630,210 )</b>	<b>\$(171,799 )</b>
<b>NET LOSS PER SHARE, BASIC</b>	<b>\$(0.67 )</b>	<b>\$(3.80 )</b>	<b>\$(1.07 )</b>
<b>NET LOSS PER SHARE, DILUTED</b>	<b>\$(0.67 )</b>	<b>\$(3.81 )</b>	<b>\$(1.07 )</b>
Weighted average common shares outstanding, basic	174,427	165,985	160,025
Weighted average common shares outstanding, diluted	174,427	166,219	160,025

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

## BIOMARIN PHARMACEUTICAL INC.

## CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

Years Ended December 31, 2017, 2016 and 2015

(In thousands)

	2017	2016	2015
NET LOSS	\$(117,042)	\$(630,210)	\$(171,799)
OTHER COMPREHENSIVE INCOME (LOSS):			
Net foreign currency gain (loss)	5	(2 )	(59 )
Available-for-sale debt securities:			
Unrealized holding loss arising during the period, net			
of tax impact of \$272, \$4,412 and \$1,581, respectively.	(483 )	(7,692 )	(2,878 )
Less: reclassifications to net loss, net of tax impact of			
\$ (1,191), \$42 and \$(681), respectively.	2,061	(73 )	1,192
Net change in unrealized holding loss, net of tax	(2,544 )	(7,619 )	(4,070 )
Cash flow hedges:			
Unrealized holding gain (loss) arising during the			
period, net of tax impact of \$0.	(38,351 )	9,677	17,300
Less: reclassifications to net loss, net of tax impact of \$0.	(5,113 )	10,273	19,604
Net change in unrealized holding loss, net of tax	(33,238 )	(596 )	(2,304 )
OTHER COMPREHENSIVE LOSS, NET OF TAX	(35,777 )	(8,217 )	(6,433 )
COMPREHENSIVE LOSS	\$(152,819)	\$(638,427)	\$(178,232)

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

## BIOMARIN PHARMACEUTICAL INC.

## CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY

Years Ended December 31, 2017, 2016 and 2015

(In thousands of U.S. dollars and share amounts in thousands)

	Common stock		Additional Paid-in Capital	Company Stock Held by NQDC	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					
Balance at December 31, 2014	149,094	\$ 149	\$2,359,744	\$(9,695 )	\$ 27,466	\$(849,770 )	\$ 1,527,894
Net loss						(171,799 )	(171,799 )
Other comprehensive loss					(6,433 )		(6,433 )
Issuance of common stock, net							
of offering costs	9,775	10	888,247				888,257
Issuances under equity							
incentive plans, net of tax	2,208	2	40,054				40,056
Conversion of convertible notes, net	449	1	9,111				9,112
Company stock held by NQDC				(3,921 )			(3,921 )
Excess tax benefit from stock							
option exercises			2,190				2,190
Stock-based compensation			115,491				115,491
Balance at December 31, 2015	161,526	\$ 162	\$3,414,837	\$(13,616 )	\$ 21,033	\$(1,021,569 )	\$ 2,400,847
Net loss						(630,210 )	(630,210 )
Cumulative-effect adjustment							
of new share-based compensation guidance						131,273	131,273
Other comprehensive loss					(8,217 )		(8,217 )
Issuance of common stock, net	7,500	8	712,930				712,938



of offering costs							
Issuances under equity							
incentive plans, net of tax	3,184	3	14,755				14,758
Conversion of convertible							
notes, net	438		8,928				8,928
Company stock held by							
NQDC				(705 )			(705 )
Stock-based compensation			136,663				136,663
Balance at December 31, 2016	172,648	\$ 173	\$4,288,113	\$(14,321)	\$ 12,816	\$(1,520,506 )	\$2,766,275
Net loss						(117,042 )	(117,042 )
Other comprehensive loss					(35,777 )		(35,777 )
Issuances under equity							
incentive plans, net of tax	2,092	2	27,350				27,352
Conversion of convertible							
notes, net	1,104	1	22,476				22,477
Company stock held by							
NQDC				97			97
Stock-based compensation			145,281				145,281
Balance at December 31, 2017	175,844	\$ 176	\$4,483,220	\$(14,224)	\$(22,961 )	\$(1,637,548 )	\$2,808,663

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

## BIOMARIN PHARMACEUTICAL INC.

## CONSOLIDATED STATEMENTS OF CASH FLOWS

Years Ended December 31, 2017, 2016 and 2015

(In thousands of U.S. dollars)

	2017	2016	2015
<b>CASH FLOWS FROM OPERATING ACTIVITIES:</b>			
Net loss	\$(117,042)	\$(630,210)	\$(171,799 )
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization expense	87,861	96,912	47,187
Non-cash interest expense	32,300	29,930	28,493
Accretion of discount on investments	3,077	1,300	2,177
Stock-based compensation expense	140,263	134,641	111,525
Gain on sale of intangible asset	(125,000)	—	(369,498 )
(Gain) loss on sale of equity investment	(3,252 )	108	(3,022 )
Impairment of assets	—	599,118	211,502
Deferred income taxes	44,464	(228,054)	(76,827 )
Unrealized foreign exchange loss (gain)	6,258	(14,481 )	(19,575 )
Non-cash changes in the fair value of contingent acquisition			
consideration payable	10,342	(57,161 )	(28,457 )
Other	5,935	336	2,463
Changes in operating assets and liabilities:			
Accounts receivable, net	(25,256 )	(51,483 )	(16,367 )
Inventory	(96,890 )	(64,512 )	(50,989 )
Other current assets	(20,687 )	19,316	25,800
Other assets	(2,439 )	(4,979 )	(3,157 )
Accounts payable and accrued liabilities	45,517	(53,205 )	90,298
Other long-term liabilities	5,792	(5,413 )	747
Net cash used in operating activities	(8,757 )	(227,837)	(219,499 )
<b>CASH FLOWS FROM INVESTING ACTIVITIES:</b>			
Purchases of property, plant and equipment	(199,219)	(148,380)	(227,653 )
Deposit on purchase of PKU rights	—	—	(371,756 )
Maturities and sales of investments	425,960	367,569	424,713
Purchase of available-for-sale debt securities	(655,447)	(699,749)	(873,184 )
Proceeds from sale of intangible asset	125,000	—	410,000
Business acquisitions, net of cash acquired	—	(2,789 )	(538,392 )
Other	(1,753 )	(698 )	(3,326 )
Net cash used in investing activities	(305,459)	(484,047)	(1,179,598)
<b>CASH FLOWS FROM FINANCING ACTIVITIES:</b>			
Proceeds from exercises of awards under equity incentive plans	60,859	74,227	63,045
Taxes paid related to net share settlement of equity awards	(33,507 )	(59,469 )	(22,989 )
Proceeds from public offering of common stock, net	—	712,938	888,257
Proceeds from convertible senior subordinated note offering, net	481,713	—	—

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Payment of contingent acquisition consideration payable	(1,894 )	—	—
Other	(26 )	(588 )	(2,590 )
Net cash provided by financing activities	507,145	727,108	925,723
Effect of exchange rate changes on cash	(3,231 )	(3,934 )	(5,072 )
<b>NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS</b>	<b>189,698</b>	<b>11,290</b>	<b>(478,446 )</b>
Cash and cash equivalents:			
Beginning of period	408,330	397,040	875,486
End of period	\$598,028	\$408,330	\$397,040
<b>SUPPLEMENTAL CASH FLOW DISCLOSURES:</b>			
Cash paid for interest, net of interest capitalized into fixed assets	\$8,544	\$8,643	\$9,307
Cash paid for income taxes	\$23,895	\$95,857	\$16,084
Stock-based compensation capitalized into inventory	\$16,052	\$11,449	\$11,140
Depreciation capitalized into inventory	\$24,076	\$17,375	\$14,627
<b>SUPPLEMENTAL CASH FLOW DISCLOSURES FOR NON-CASH</b>			
<b>INVESTING AND FINANCING ACTIVITIES:</b>			
Increase (decrease) in accounts payable and accrued liabilities related to			
fixed assets	\$(25,786 )	\$20,158	\$(4,651 )
Conversion of convertible debt, net	\$22,477	\$8,928	\$9,112

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 therapy portfolio consists of six commercial products and multiple clinical and pre-clinical product candidates.

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 financial performance of its commercial products; the potential need for additional financings; the Company's ability to successfully commercialize its approved products; the uncertainty of the Company's research and development (R&D) 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. Please see "Risk Factors" included in Part I, Item 1A of this Annual Report on Form 10-K for a more detailed discussion of these risks.

(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 for Annual Reports on Form 10-K and include the accounts of BioMarin and its wholly owned subsidiaries. All significant 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 are no subsequent events or transactions that occurred subsequent to the balance sheet date and prior to the filing this Annual Report on Form 10-K that would require recognition or disclosure in the Consolidated Financial Statements.

## Use of Estimates

The preparation of financial statements in conformity with U.S. GAAP requires management to make judgments, estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities at the dates of the financial statements, and the reported amounts of revenues and expenses during the reporting period. 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.

## (3) SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

### Cash, Cash Equivalents and Investments

The Company treats liquid investments with original maturities of three months or less when purchased as cash and cash equivalents. Cash and cash equivalents primarily consist of cash on deposit with banks, investments in money market funds and debt securities with original maturities of three months or less when purchased.

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## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

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 balance sheet date. 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. Available-for-sale debt securities are recorded at fair market value, with unrealized gains and losses included in Accumulated Other Comprehensive Income (Loss) on the Company's Consolidated Balance Sheets, with the exception of unrealized losses believed to be other-than-temporary, if any, which are reported in Other Income (Expense) in the current period. Available-for-sale debt securities primarily consist of corporate securities, commercial paper, U.S. federal government agency securities and certificates of deposit.

#### 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 and adjusts inventory to its net realizable value, if required, for obsolete, or has a cost basis in excess of its expected net realizable value or for quantities in excess of expected requirements. These adjustments are recognized as Cost of Sales in the Company's Consolidated Statements of Operations.

When future commercialization is considered probable and the future economic benefit is expected to be realized, based on management's judgment, the Company capitalizes pre-launch inventory costs prior to regulatory approval. A number of factors are taken into consideration, including the current status in the regulatory approval 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, commercialization and marketplace trends. As of December 31, 2017, the amount of pre-launch inventory on the Company's Consolidated Balance Sheets was not significant.

#### Property, Plant and Equipment

Property, plant and equipment are stated at 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, while repairs and maintenance are charged to expense as incurred. Depreciation of property, plant and equipment are included in Cost of Sales, Selling, General and Administrative and Research and Development, as appropriate, in the Consolidated Statements of Operations. Property and equipment purchased for specific R&D projects with no alternative uses are expensed as incurred and recorded to Research and Development in the Consolidated Statements of Operations.

Leasehold improvements	Shorter of life of asset or lease term
Building and improvements	15 to 50 years
Manufacturing and laboratory equipment	5 to 18 years
Computer hardware and software	3 to 10 years
Office furniture and equipment	5 to 10 years

Vehicles	5 years
Land improvements	5 to 10 years
Land	Not applicable
Construction-in-progress	Not applicable

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BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

Leases

Certain of the Company's operating lease agreements include scheduled rent escalations over the lease term, as well as tenant improvement allowances. Scheduled increases in rent expense are recognized on a straight-line basis over the lease term. The difference between rent expense and rent paid is recorded as deferred rent and included in Other Liabilities in the Consolidated Balance Sheets. The tenant improvement allowances and free rent periods are recognized as a reduction of rent expense over the lease term on a straight-line basis. Rent expense is recorded to Cost of Sales, Research and Development and/or Selling, General and Administrative, as appropriate, in the Consolidated Statements of Operations.

Goodwill and Intangible Assets

The Company records goodwill in a business combination when the total consideration exceeds the fair value of the net tangible and identifiable intangible 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. The Company does not amortize goodwill and intangible assets with indefinite useful lives. 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.

Impairment

The Company reviews 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 which indicate that the carrying amount may not be recoverable. The Company qualitatively assesses goodwill and indefinite lived intangible assets to determine whether it is more likely than not that the fair value of the Company's single reporting unit or other indefinite lived intangible asset is less than its carrying amount. If it is determined that the fair value is more likely than not less than its carrying value, a two-step impairment test will be performed.

In the first step of the impairment test, the Company compares the carrying value of its single reporting unit or applicable asset to its fair value, which the Company estimates using a discounted cash flow analysis. If the carrying amount of its single reporting unit or asset exceeds its estimated fair value, the Company performs the second step, and determines the impairment loss, if any, as the excess of the carrying value of the goodwill or intangible asset over its fair value. Impairment charges that are not material are recorded to Intangible Asset Amortization and Contingent Consideration in the Consolidated Statements of Operations. See Note 7 to these Consolidated Financial Statements for the results of the Company's 2017 qualitative assessment.

Long-lived assets including property, plant and equipment and finite-lived intangible assets are reviewed for impairment whenever facts or circumstances whether internally or externally may suggest that the carrying value of an asset or asset group may not be recoverable. 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.

#### Revenue Recognition

The Company recognizes revenue when persuasive evidence of an arrangement exists, delivery has occurred, the price to the buyer is fixed or determinable and collection from the customer is reasonably assured.

Net Product Revenues—The Company recognizes revenues from product sales when title and risk of loss have passed to the customer, which typically occurs upon delivery. Product sales transactions are evidenced by customer purchase orders, customer contracts, invoices and/or the related shipping documents. Amounts collected from customers and remitted to governmental authorities, which primarily consists of value-added taxes related to product sales in foreign jurisdictions, are presented on a net basis in the Consolidated Statements of Operations, in that taxes billed to customers are not included as a component of net product revenues.

In the U.S., the Company's commercial products are generally sold to specialty pharmacies or end-users, such as hospitals, which act as retailers. Through December 31, 2015, the Company also sold Kuvan to Ares Trading S.A. (Merck Serono) at a price near its manufacturing cost, and Merck Serono resold the product to end users outside the U.S., Canada and Japan. The royalty earned from Kuvan product sold by Merck Serono in the

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

EU was included as a component of Net Product Revenues in the period earned. 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.

The Company receives a payment ranging from 39.5% to 50% on worldwide net Aldurazyme sales by Genzyme Corporation (Genzyme) depending on sales volume, which is included in Net Product Revenues in the Company's Consolidated Statements of Operations. The Company recognizes a portion of this amount as product transfer revenue when the product is shipped and released to Genzyme because all of the Company's performance obligations are fulfilled at that point and title to, and risk of loss for, the product has transferred to Genzyme. The product transfer revenue represents the fixed amount per unit of Aldurazyme that Genzyme is required to pay the Company if the product is unsold by Genzyme. The amount of product transfer revenue will eventually be deducted from the revenues earned by the Company calculated based on Genzyme's net sales recognized when the product is sold by Genzyme. The Company records the Aldurazyme revenues based on net sales information provided by Genzyme and record product transfer revenue based on the fulfillment of Genzyme purchase orders in accordance with the terms of the related agreements with Genzyme and when the title and risk of loss for the product is transferred to Genzyme. Although described as royalties in the Company's agreements with Genzyme, the revenues that the Company receives for Aldurazyme and, for the periods through 2015, for Kuvan, are similar to direct product sales because the Company manufactures the product and the revenue is highly dependent on substantial operational activities performed by the Company, including responsibility for global regulatory compliance. These responsibilities, and the operational risk that could reduce or eliminate the Company's receipt of these percentage of net sales amounts, are similar to many of the responsibilities and risks associated with the Company's direct sales of other commercial products. Due to the significant role the Company plays in the operations of Aldurazyme as well as the rights and responsibilities to deliver the products to Genzyme, the Company includes Aldurazyme revenues as a component of Net Product Revenues in the Company's Consolidated Statements of Operations. See Note 4 to these Consolidated Financial Statements for further discussion on the Company's adoption of Accounting Standards Update (ASU) No. 2014-09 (ASU 2014-09), Revenue from Contracts with Customers, as amended (commonly referred to as ASC Topic 606) on January 1, 2018.

The Company records an allowance for government and other rebates is estimated based on products sold, customer mix, and program requirements. The Company evaluates its customer mix to estimate which sales will be subject to rebates and consider changes to government program guidelines that would impact the actual rebates and/or estimates of which sales qualify for such rebates. The Company updates its estimates and assumptions on a quarterly basis based on actual historical experience and records any necessary adjustments to its reserves. The Company records fees paid to distributors and cash discounts as a reduction of revenue.

The Company records allowances for product returns, if appropriate, as a reduction of revenue at the time product sales are recorded. The Company relies on historical return rates to estimate returns. 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 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. Certain international customers, usually government entities, tend to purchase larger quantities of product less frequently. Although such buying patterns may result in revenue fluctuations from quarter to quarter, the Company has not experienced any increased product returns or risk of product returns related to such buying patterns. Genzyme's contractual return rights

for Aldurazyme are limited to defective product. Based on these factors and the fact that the Company has not experienced significant product returns to date, management has concluded that product returns will be minimal. In the future, if any of these factors and/or the history of product returns change, an allowance for product returns may be required.

Royalty and Other Revenues—includes royalties on net sales of products with which the Company has no direct involvement, collaborative agreement revenues and rental income.

Royalty revenue is recognized as earned in accordance with the contract terms at the time the royalty amount is fixed or determinable based on information received from the licensees and sublicensees and at the time collectibility is reasonably assured.

Revenue from non-refundable up-front license fees, such as under an obligation to supply product, is recognized as performance occurs and the Company's obligations are completed or recognized up-front upon agreement execution if there are no continuing involvement performance obligations. Revenue associated with substantive at-risk milestones is recognized based upon the achievement of the milestones set forth in the respective agreements. Advance payments received in excess of amounts earned are classified as deferred revenue on the Company's Consolidated Balance Sheets.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

Research and Development

R&D costs are generally expensed as incurred. These expenses include contract R&D provided by third parties, most product manufacturing prior to regulatory approval, materials and supplies, clinical and regulatory costs, and personnel costs including salaries, benefits and stock-based compensation. In instances where the Company enters into agreements with third parties for R&D activities, costs are expensed upon the earlier of when non-refundable amounts are due or as services are performed unless there is an alternative future use of the funds in other R&D projects. Amounts due under such arrangements may be either fixed fee or fee for service and may include upfront payments, monthly payments and payments upon the completion of milestones or receipt of deliverables.

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 accounts for the debt net of any discounts or issuance costs on the Consolidated Balance Sheet.

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

Net Loss Per Common Share

Basic net loss per share is calculated by dividing net loss by the weighted average shares of common stock outstanding during the period. Diluted net 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. See Note 14 to these Consolidated Financial Statements for further details.

Stock-Based Compensation

The Company has stock-based compensation plans, including an Employee Stock Purchase Plan (ESPP), under which various types of equity-based awards are granted or available to employees, including restricted stock units (RSUs) with both performance and service-based vesting conditions and stock options. Stock-based compensation expense is recognized on a straight-line basis over the requisite service period for each award and is classified as Cost of Sales, Research and Development or Selling, General and Administrative, as appropriate, in the Consolidated Statements of Operations. The Company accounts for forfeitures as they occur.

The fair value of each stock option award and the Company's ESPP awards are estimated on the date of grant using the Black-Scholes valuation model and the following assumptions: expected life of a stock option, 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 life of stock options is based on observed historical exercise patterns. Groups of employees that have similar historical exercise patterns were considered separately for valuation purposes. The Company has identified two groups, executive and non-executive employees, with distinctly different exercise patterns. The executive employee group has a history of holding stock options for longer periods than non-executive employees.

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.

The fair value of 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 for RSUs with service-based vesting is recognized ratably over the period during which the vesting restrictions lapse. Stock-based compensation for RSUs with performance conditions is recognized ratably over the service period beginning in the period the Company determines it is probable that the performance condition will be achieved.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

See Note 17 to these Consolidated Financial Statements for further information.

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. 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 Selling, General and Administrative in the Consolidated Statements of Operations.

Derivatives and Hedging Activities

The Company accounts for its derivative instruments as either assets or liabilities on the balance sheet and measures them at fair value. Derivatives that are not designated as hedging instruments are adjusted to fair value through earnings in Selling, General and Administrative in the Consolidated Statements of Operations.

For derivative instruments that hedge the exposure to variability in expected future cash flows that are designated as cash flow hedges, the effective portion of the gain or loss is reported as a component of Accumulated Other Comprehensive Income (Loss) in shareholders' equity and reclassified to earnings in the same period or periods during which the hedged transaction affects earnings. The ineffective portion of the gain or loss on the derivative instrument, if any, is recognized in earnings in the current period. 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.

See Note 11 to these Consolidated Financial Statements for further information.

## Fair Value of Financial Instruments

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

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BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

• 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)

See Note 12 to these Consolidated Financial Statements for further information.

Segment Information

The Company currently operates in one business 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 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, and does not have separately reportable segments.

Acquisitions

Acquisitions of businesses are accounted for using the acquisition method of accounting. The Company allocates the purchase price of acquired businesses to the tangible and intangible assets acquired and liabilities assumed based upon their estimated fair values on the acquisition date. The purchase price allocation process requires management to make significant estimates and assumptions, especially at the acquisition date with respect to intangible assets and IPR&D. In connection with the purchase price allocations for acquisitions, the Company estimates the fair value of contingent acquisition consideration payments utilizing a probability-based income approach inclusive of an estimated discount rate. Each reporting period thereafter, the Company revalues these obligations and records increases or decreases in their fair value as adjustments to Intangible Asset Amortization and Contingent Consideration in the Consolidated Statements of Operations. Changes in the fair value of the contingent acquisition consideration payable 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.

(4) RECENT ACCOUNTING PRONOUNCEMENTS

Accounting Pronouncements Not Yet Adopted

Effective January, 1, 2018, the Company will adopt Accounting Standards Codification, Topic 606, Revenue from Contracts with Customers (ASC Topic 606), which provides principles for recognizing revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the Company expects



to be entitled in exchange for those goods or services. The Company expects to adopt ASC Topic 606 on a modified retrospective basis through a cumulative adjustment to equity.

As the Company completes its analysis of the accounting for the Company's customer contracts under the new revenue standard, management is assessing the required changes to the Company's accounting policies, systems and internal control over financial reporting. Based on management's preliminary assessment of the Company's material contracts with customers, management does not anticipate that ASC Topic 606 will have a material impact on the timing of revenue recognition for the products that are marketed by the Company. Based on management's preliminary assessment of the Company's contract with Genzyme, the Company expects to recognize Aldurazyme revenues when the product is shipped to Genzyme and all the required quality control certifications are complete, because all of the Company's performance obligations are fulfilled at that point in time. The Company expects to record Aldurazyme net product revenues based on the estimated tiered payment that will be in effect when the product is sold through by Genzyme. Management believes any differences between estimated revenues from Genzyme and actual payments will be insignificant.

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BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

In the first quarter of 2018, the management expects the impact of adopting ASC Topic 606 to result in a \$20.0 million reduction in Accumulated Deficit, the cumulative effect adjustment under the modified retrospective approach, a \$26.0 million increase in Accounts Receivable and a \$6.0 million decrease in Deferred Tax Assets.

In February 2016, the Financial Accounting Standards Board (FASB) issued ASU No. 2016-02, Leases (ASU 2016-02). The amended guidance requires balance sheet recognition of lease right-of-use (ROU) assets and liabilities by lessees for leases classified as operating leases, with an option to not recognize lease ROU assets and lease liabilities for leases with a term of 12 months or less. The amendments also require new disclosures providing additional qualitative and quantitative information about the amounts recorded in the financial statements. Lessor accounting is largely unchanged. ASU 2016-02 is effective for the Company's fiscal year beginning January 1, 2019. Early adoption is permitted, but the Company has not made the election to do so. ASU 2016-02 will be effective for the Company's fiscal year beginning January 1, 2019. The amendments require a modified retrospective approach with optional practical expedients.

As of December 31, 2017, the Company's task force formed in connection with the adoption of ASU 2016-02 was in the process of analyzing the Company's lease contracts and the potential impact the standard may have on its Consolidated Financial Statements and related disclosures. After completing the analysis of the accounting for the Company's lease contracts under the standard, management will assess the required changes to the Company's accounting policies, systems and internal control over financial reporting. Based on management's preliminary analysis, the Company anticipates the standard may have a material impact on the Company's Consolidated Balance Sheets due to the requirement to recognize lease ROU assets and corresponding liabilities related to leases on the Company's Consolidated Balance Sheets, but it is not anticipated to have a material impact on the Company's other Consolidated Financial Statements. See Note 23 to these Consolidated Financial Statements for additional information regarding the Company's lease obligations as of December 31, 2017.

In January 2017, the FASB issued ASU No. 2017-01, Clarifying the Definition of a Business (ASU 2017-01), which is intended to clarify the definition of a business. ASU 2017-01 is effective for the Company's fiscal year beginning January 1, 2018. The new guidance is required to be applied on a prospective basis. The Company anticipates that the adoption of ASU 2017-01 will result in more acquisitions being accounted for as asset acquisitions.

In August 2017, the FASB issued ASU No. 2017-12, Derivatives and Hedging (Topic 815): Targeted Improvements to Accounting for Hedging Activities (ASU 2017-12). The standard changes the recognition and presentation requirements of hedge accounting, including eliminating the requirement to separately measure and report hedge ineffectiveness and presenting all items that affect earnings in the same income statement line as the hedged item. ASU 2017-12 is effective for the Company's fiscal year beginning January 1, 2019 and early adoption is permitted. Although the Company is currently evaluating the impact that the standard will have on its Consolidated Financial Statements, adoption of the standard is not expected to have a material impact due to the nature of the Company's hedging activity. As of December 31, 2017, the Company has not elected to early adopt ASU 2017-12.

#### Accounting Pronouncements Adopted

In January 2017, the FASB issued ASU No. 2017-04, Goodwill and Other - Simplifying the Test for Goodwill Impairment (ASU 2017-04), which eliminated the requirement to determine the fair value of individual assets and

liabilities of a reporting unit to measure goodwill impairment. Under the amendments, goodwill impairment testing shall be performed by comparing the fair value of the reporting unit with its carrying amount and recognizing an impairment charge for the amount by which the carrying amount exceeds the reporting unit's fair value. The new standard should be applied on a prospective basis. Early adoption was permitted for annual or interim goodwill impairment testing performed after January 1, 2017. The Company elected to early adopt ASU 2017-04 in the first quarter of 2017, which had no effect on the Company's Consolidated Financial Statements as no goodwill impairments were identified in fiscal year 2017.

In May 2017, the FASB issued ASU No. 2017-09, Compensation - Stock Compensation (Topic 718): Scope of Modification Accounting (ASU 2017-09). The amendment provided clarification about which changes to the terms or conditions of a share-based payment award require an entity to apply modification accounting in Topic 718. As early adoption was permitted, the Company elected to early adopt ASU 2017-09 in the second quarter of 2017, which did not have a material impact on the Company's Consolidated Financial Statements because the Company's policies had already been in compliance.

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BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

In January 2016, the FASB issued ASU No. 2016-01, Recognition and Measurement of Financial Assets and Financial Liabilities (ASU 2016-01). ASU 2016-01 changed accounting for equity investments, financial liabilities under the fair value option and the presentation and disclosure requirements for financial instruments. In addition, the update clarifies guidance related to the valuation allowance assessment when recognizing deferred tax assets resulting from unrealized losses on available-for-sale debt securities. The guidance shall be adopted using a modified retrospective approach, with certain exceptions. As early adoption was permitted for certain provisions, the Company elected to early adopt ASU 2016-01 in 2017, which did not have a material impact on the Company's Consolidated Financial Statements because the Company's policies had already been in compliance.

(5) ACQUISITIONS

The Merck PKU Business

On October 1, 2015, the Company entered into a Termination and Transition Agreement with Ares Trading S.A. (Merck Serono), as amended and restated on December 23, 2015 (the A&R Kuvan Agreement), to terminate the Development, License and Commercialization Agreement, dated May 13, 2005, as amended (the License Agreement), between the Company and Merck Serono, including the license to Kuvan the Company had granted to Merck Serono under the License Agreement. Also on October 1, 2015, the Company and Merck Serono entered into a Termination Agreement (the Pegvaliase Agreement) to terminate the license to pegvaliase the Company had granted to Merck Serono under the License Agreement. On January 1, 2016, pursuant to the A&R Kuvan Agreement and the Pegvaliase Agreement, the Company completed the acquisition from Merck Serono and its affiliates of certain rights and other assets with respect to Kuvan and pegvaliase (the Merck PKU Business). As a result, the Company acquired all global rights to Kuvan and pegvaliase 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 pegvaliase in the U.S. and Japan. In connection with the acquisition of the Merck PKU Business, the Company recognized transaction costs of \$0.6 million, of which \$0.3 million was recognized in each of the years ended December 31, 2016 and 2015.

Pursuant to the A&R Kuvan Agreement, the Company paid Merck Serono \$374.5 million in cash and is obligated to pay Merck Serono up to a maximum of €60.0 million, in cash, if future sales milestones are met. Pursuant to the Pegvaliase Agreement, the Company is obligated to pay Merck Serono up to a maximum of €125.0 million, in cash, if future development milestones are met. Merck Serono transferred certain inventory, regulatory materials and approvals, and intellectual property rights to the Company and performed certain transition services for the Company. As of December 31, 2016, the inventory acquired from Merck Serono has been sold through to customers. The Company and Merck Serono have no further rights or obligations under the License Agreement with respect to Kuvan or pegvaliase.

Kuvan is a commercialized product for the treatment of patients with phenylketonuria (PKU) and/or for primary BH4 deficiency in certain countries. At the time of the acquisition, pegvaliase was in pivotal studies as a potential therapeutic option for adult patients with PKU. In March 2016, the Company announced that its pivotal Phase 3

PRISM-2 study of pegvaliase met the primary endpoint of change in blood Phe compared with placebo ( $p < 0.0001$ ). The Company submitted a marketing application for pegvaliase in the U.S. in June 2017. The Company plans to submit a Marketing Authorization Application for pegvaliase to the European Medicines Agency (EMA) in the first quarter of 2018. In a notice received from the U.S. Food and Drug Administration (FDA), the Prescription Drug User Fee Act (PDUFA) target action date for pegvaliase has been extended by three months to May 28, 2018, which was changed to May 25, 2018 due to the Memorial Day holiday. Kuvan has Orphan Drug exclusivity in the EU until 2020, and pegvaliase has Orphan Drug designation in the U.S. and the EU.

The acquisition date fair value of the contingent acquisition consideration payments, Kuvan global marketing rights, with the exception of Japan, and pegvaliase IPR&D acquired was estimated by applying a probability-based income approach utilizing an appropriate discount rate. See Note 12 to these Consolidated Financial Statements for additional discussion regarding fair value measurements of the contingent acquisition consideration payable included on the Company's Consolidated Balance Sheet.

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## 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 presents the final allocation of the purchase consideration for the Merck PKU Business acquisition, including the contingent acquisition consideration payable based on the acquisition date fair value. The allocation of the purchase price below reflects an inventory adjustment in the second quarter of 2016.

Cash payments	\$374,545
Estimated fair value of contingent acquisition consideration payable	138,974
Total consideration	\$513,519

Kuvan intangible assets	\$172,961
Pegvaliase IPR&D	326,359
Inventory	14,199
Total identifiable assets acquired	\$513,519

The amount allocated to the Kuvan intangible assets is considered to be finite-lived and is being amortized on a straight-line basis over its estimated useful life through 2024.

The amount allocated to pegvaliase acquired IPR&D is considered to be indefinite-lived as R&D efforts are ongoing and, as such, is not subject to amortization. See Note 7 to these Consolidated Financial Statements for further discussion of indefinite-lived intangible assets.

## (6) INVESTMENTS

The amortized cost, gross unrealized holding gains or losses, and fair value of the Company's available-for-sale debt securities by major security type at December 31, 2017 and 2016 are summarized in the tables below:

	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Aggregate Fair Value at December 31, 2017
Corporate debt securities	\$707,652	\$ 150	\$ (2,553 )	\$ 705,249
Commercial paper	24,566	—	—	24,566
U.S. government agency securities	472,593	—	(1,975 )	470,618
Foreign and other	25,540	150	(64 )	25,626
Total	\$1,230,351	\$ 300	\$ (4,592 )	\$ 1,226,059

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		Gross Unrealized	Gross Unrealized	Aggregate Fair Value at December 31, 2016
	Amortized Cost	Holding Gains	Holding Losses	
Certificates of deposit	\$2,800	\$ —	\$ —	\$ 2,800
Corporate debt securities	641,670	329	(2,282 )	639,717
Commercial paper	16,075	—	—	16,075
U.S. government agency securities	310,635	37	(747 )	309,925
Foreign and other	48	86	—	134
Total	\$971,228	\$ 452	\$ (3,029 )	\$ 968,651

The fair values of available-for-sale securities by contractual maturity were as follows:

	December 31,	
	2017	2016
Maturing in one year or less	\$840,274	\$395,940
Maturing after one year through five years	385,785	572,711
Total	\$1,226,059	\$968,651

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

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 other-than-temporary and, if it is other-than-temporary, an impairment loss is recognized in earnings equal to the difference between the investment's amortized cost and fair value at such date. As of December 31, 2017, some of the Company's investments were in an unrealized loss position. However, the Company has the ability and intent to hold all investments that have been in a continuous loss position until maturity or recovery, thus no other-than-temporary impairment was deemed to have occurred.

See Note 12 to these Consolidated Financial Statements for additional discussion regarding the fair value of the Company's available-for-sale securities.

## (7) INTANGIBLE ASSETS

Intangible assets consisted of the following:

	December 31,	
	2017	2016
Intangible assets:		
Finite-lived intangible assets	\$ 303,298	\$ 305,122
Indefinite-lived intangible assets	326,359	332,199
Gross intangible assets:	629,657	637,321
Less: Accumulated amortization	(112,147)	(83,541)
Net carrying value	\$ 517,510	\$ 553,780

## Finite-Lived Intangible Assets

The following table summarizes the net-book-value and estimated remaining life of the Company's finite-lived intangible assets as of December 31, 2017:

	Net Balance at	
	December 31,	
	2017	Average Remaining Life
Repurchased royalty rights	\$ 39,938	5.9 years
Acquired intellectual property	149,229	7.2 years
License payments for marketing approvals	1,357	4.4 years
In-place and above market tenant leases	627	0.7 - 5.6 years



Total	\$ 191,151
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As of December 31, 2017, the estimated future amortization expense associated with the Company's finite-lived intangible assets for each of the five succeeding fiscal years was as follows:

Fiscal Year	Amount
2018	\$ 30,400
2019	30,086
2020	27,605
2021	26,681
2022	26,657
Thereafter	49,722
	\$ 191,151

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

## Indefinite-Lived Intangible Assets

Indefinite-lived intangible assets consisted of the following:

	December 31,	
	2017	2016
<b>In-Process Research and Development:</b>		
Pegvaliase	\$ 326,359	\$ 326,359
Other acquired pre-clinical compounds	—	5,840
Net carrying value	\$ 326,359	\$ 332,199

Intangible assets related to IPR&D assets are considered to be indefinite-lived until the completion or abandonment of the associated R&D efforts.

The Company recognized an impairment charge of \$5.8 million related to acquired IPR&D assets during the fourth quarter of 2017, which was included in Intangible Asset Amortization and Contingent Consideration in the Consolidated Statements of Operations.

In 2016 and 2015, the Company recorded impairment charges of \$574.1 million and \$198.7 million, respectively, related to the Kyndrisa and other exon IPR&D assets based on the status of development efforts. These impairments reduced the remaining book value to zero due to the termination of the programs. In 2016, the Company also recognized an impairment charge of \$25.0 million related to the reveglucosidase alfa IPR&D assets due to the decision to terminate that development program.

In December 2017, the Company sold the Rare Pediatric Disease Priority Review Voucher (PRV) it received from the FDA in connection with the U.S. approval of Brineura. In exchange for the voucher the Company received \$125.0 million from Novartis Pharma AG. The proceeds from the sale of the PRV were recognized as a gain on the sale of intangible asset as the PRV did not have a carrying value on the Company's Consolidated Balance Sheet at the time of sale.

## (8) PROPERTY, PLANT AND EQUIPMENT

Property, plant and equipment, net, consisted of the following:

	December 31,	
	2017	2016

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Building and improvements	\$663,347	\$510,805
Manufacturing and laboratory equipment	294,521	242,899
Computer hardware and software	144,268	129,506
Leasehold improvements	42,572	44,184
Furniture and equipment	31,515	27,229
Land improvements	5,331	4,881
Land	62,369	55,412
Construction-in-progress	59,511	126,446
	1,303,434	1,141,362
Less: Accumulated depreciation	\$(406,734 )	(342,594 )
Total property, plant and equipment, net	\$896,700	\$798,768

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

Depreciation for the years ended December 31, 2017, 2016 and 2015 was \$75.8 million, \$73.2 million and \$50.1 million, respectively, of which \$24.1 million, \$17.4 million and \$14.6 million was capitalized into inventory, respectively. Capitalized interest related to the Company's property, plant and equipment purchases for each of the three years ended December 31, 2017 was insignificant.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

## (9) INVENTORY

Inventory consisted of the following:

	December 31,	
	2017	2016
Raw materials	\$49,877	\$51,250
Work-in-process	234,674	167,788
Finished goods	191,224	136,088
Total inventory	\$475,775	\$355,126

## (10) SUPPLEMENTAL BALANCE SHEET INFORMATION

Accounts payable and accrued liabilities consisted of the following:

	December 31,	
	2017	2016
Accounts payable and accrued operating expenses	\$166,616	\$191,353
Accrued compensation expense	140,781	109,038
Accrued rebates payable	36,472	34,737
Accrued royalties payable	18,820	15,151
Value added taxes payable	9,740	7,848
Forward foreign currency exchange contracts	14,464	5,201
Accrued income taxes	5,528	—
Other	9,500	7,177
Total accounts payable and accrued liabilities	\$401,921	\$370,505

The roll forward of significant estimated accrued rebates and reserve for cash discounts for the years ended December 31, 2017, 2016 and 2015 were as follows:

Balance at	Provision	Provision/	Actual Charges	Actual Charges	Balance at
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	Beginning of Period	for Current Period Sales	(Reversals) for Prior Period Sales	Related to Current Period Sales	Related to Prior Period Sales	End of Period
<b>Year ended December 31, 2017:</b>						
Accrued rebates	\$ 34,737	\$ 56,282	\$ (3,686 )	\$ (31,714 )	\$ (19,147 )	\$ 36,472
Reserve for cash discounts	888	10,730	(58 )	(9,601 )	(904 )	1,055
<b>Year ended December 31, 2016:</b>						
Accrued rebates	\$ 32,553	\$ 44,347	\$ (5,205 )	\$ (23,879 )	\$ (13,079 )	\$ 34,737
Reserve for cash discounts	831	8,889	(22 )	(8,160 )	(650 )	888
<b>Year ended December 31, 2015:</b>						
Accrued rebates	\$ 14,859	\$ 45,356	\$ (1,245 )	\$ (18,421 )	\$ (7,996 )	\$ 32,553
Reserve for cash discounts	688	7,402	—	(6,722 )	(537 )	831

(11) DERIVATIVE INSTRUMENTS AND HEDGING STRATEGIES

Foreign Currency Exchange Rate Exposure

The Company uses forward foreign currency exchange 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 U.S. dollar, primarily the Euro.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

The Company designates certain of these forward foreign currency exchange contracts as hedging instruments and enters into some forward foreign currency exchange contracts that are considered to be economic hedges that are not designated as hedging instruments. Whether designated or undesignated, these forward foreign currency exchange 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 U.S. dollar. The fair values of forward foreign currency exchange contracts are estimated using current exchange rates and interest rates, and take into consideration the current creditworthiness of the counterparties or the Company, as applicable. Information regarding the specific instruments used by the Company to hedge its exposure to foreign currency exchange rate fluctuations is provided below. See Note 12 to these Consolidated Financial Statements for additional discussion regarding the fair value of forward foreign currency exchange contracts.

The Company enters into forward foreign currency exchange contracts in order to protect against the fluctuations in revenue and operating expenses associated with foreign currency-denominated cash flows. The Company has formally designated these forward foreign currency exchange contracts as cash flow hedges and expects them to be highly effective in offsetting fluctuations in operating expenses denominated in Euros and revenues denominated in currencies other than the U.S. dollar related to changes in foreign currency exchange rates.

The following table summarizes the Company's designated forward foreign currency exchange contracts outstanding as of December 31, 2017 (notional amounts in millions):

	Number of Contracts	Aggregate Notional Amount in Foreign Currency	Maturity
Foreign Exchange Contracts	Contracts	Currency	Maturity
Brazilian Reais – Sell	6	160.0	May 2018
Canadian Dollars – Sell	24	27.2	Jan. 2018 - Dec. 2018
Colombian Pesos – Sell	12	93,000.0	Jan. 2018 - Dec. 2018
Euros – Purchase	80	122.2	Jan. 2018 - Dec. 2020
Euros – Sell	289	373.5	Jan. 2018 - Dec. 2020
Total	411		

The maximum length of time over which the Company is hedging its exposure to the reduction in value of forecasted foreign currency revenues through forward foreign currency exchange contracts is through December 2020. Over the next twelve months, the Company expects to reclassify \$12.1 million from Accumulated Other Comprehensive Income (Loss) to earnings as the forecasted revenue and operating expense transactions occur.

The following table summarizes the Company's non-designated forward foreign currency exchange contracts outstanding as of December 31, 2017 (notional amounts in millions):

	Number of Contracts	Aggregate Notional Amount in Foreign Currency	Maturity
Foreign Exchange Contracts			
British Pounds – Sell	1	5.0	January 2018
Euros – Purchase	4	93.5	January 2018
Total	5		

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## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

The fair value carrying amounts of the Company's derivative instruments were as follows:

	Asset Derivatives December 31, 2017 Balance Sheet		Liability Derivatives December 31, 2017 Balance Sheet	
	Location	Fair Value	Location	Fair Value
Derivatives designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	\$ 4,015	Accounts payable & accrued liabilities	\$ 14,420
Forward foreign currency exchange contracts	Other assets	4,973	Other long- term liabilities	12,686
Total		\$ 8,988		\$ 27,106
Derivatives not designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	\$ 675	Accounts payable & accrued liabilities	\$ 44
Total		675		44
Total value of derivative contracts		\$ 9,663		\$ 27,150

	Asset Derivatives December 31, 2016 Balance Sheet		Liability Derivatives December 31, 2016 Balance Sheet	
	Location	Fair Value	Location	Fair Value
Derivatives designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	\$ 13,048	Accounts payable & accrued liabilities	\$ 5,176
Forward foreign currency exchange contracts	Other assets	8,194	Other long- term liabilities	2,342



contracts			
Total		\$ 21,242	\$ 7,518
Derivatives not designated as			
hedging instruments:			
Forward foreign currency exchange			Accounts payable &
contracts	Other current assets	\$ 964	accrued liabilities
Total		964	25
Total value of derivative contracts		\$ 22,206	\$ 7,543

The effect of the Company's derivative instruments on the Consolidated Financial Statements for the years ended December 31, 2017, 2016 and 2015 was as follows:

	Years Ended December 31,		
	2017	2016	2015
<b>Derivatives Designated as Hedging Instruments:</b>			
Net gain (loss) recognized in accumulated other			
comprehensive loss <sup>(1)</sup>	\$(38,351)	\$9,677	\$17,300
Net gain (loss) reclassified from accumulated			
other comprehensive income (loss) into earnings <sup>(2)</sup>	(5,113 )	6,529	19,604
Net gain (loss) recognized in net loss <sup>(3)</sup>	2,576	5,070	(727 )
<b>Derivatives Not Designated as Hedging Instruments:</b>			
Net gain (loss) recognized in net loss <sup>(4)</sup>	\$8,255	\$(8,687)	\$4,493

(1) Net change in the fair value of the effective portion classified as accumulated other comprehensive income (loss).

(2) Effective portion classified as Net Product Revenues and SG&A expense.

(3) Ineffective portion and amount excluded from effectiveness testing classified as SG&A expense.

(4) Classified as SG&A expense.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

The Company is exposed to counterparty credit risk on all of its derivative financial instruments. The Company has established and maintains strict counterparty credit guidelines and enters into hedges only with financial institutions that are investment grade or better to minimize the Company's exposure to potential defaults. The Company does not require collateral to be pledged under these agreements.

## (12) FAIR VALUE MEASUREMENTS

The Company measures certain financial assets and liabilities at fair value on a recurring basis, including available-for-sale fixed income securities and foreign currency derivatives.

The tables below present the fair value of these financial assets and liabilities determined using the following input levels.

	Fair Value Measurements at December 31, 2017			
	Quoted Price in			
	Active Markets			
	For Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Cash equivalents (including available-for-sale debt securities):				
Money market instruments	—	215,441	—	215,441
Corporate debt securities	—	3,096	—	3,096
Commercial paper	—	2,751	—	2,751
U.S. government agency securities	—	35,497	—	35,497
Foreign and other	—	990	—	990
Total cash and cash equivalents	—	257,775	—	257,775
Available-for-sale debt securities:				
Short-term:				

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Corporate debt securities	—	406,188	—	406,188
Commercial paper	—	21,815	—	21,815
U.S. government agency securities	—	345,501	—	345,501
Foreign and other	—	24,436	—	24,436
Long-term:				
Corporate debt securities	—	295,965	—	295,965
U.S. government agency securities	—	89,620	—	89,620
Foreign and other	—	200	—	200
Total available-for-sale debt securities	—	1,183,725	—	1,183,725
Other Current Assets:				
NQDC Plan assets	—	967	—	967
Forward foreign currency exchange contract <sup>(1)</sup>	—	4,690	—	4,690
Restricted investments <sup>(2)</sup>	—	15,647	—	15,647
Total other current assets	—	21,304	—	21,304
Other Assets:				
NQDC Plan assets	—	11,859	—	11,859
Forward foreign currency exchange contract <sup>(1)</sup>	—	4,973	—	4,973
Total other assets	—	16,832	—	16,832
Total assets	\$—	\$ 1,479,636	\$ —	\$ 1,479,636
Liabilities:				
Current Liabilities:				
NQDC Plan liability	\$ 1,356	\$ 967	\$ —	\$ 2,323
Forward foreign currency exchange contract <sup>(1)</sup>	—	14,464	—	14,464
Contingent acquisition consideration payable	—	—	53,648	53,648
Total current liabilities	1,356	15,431	53,648	70,435
Other long-term liabilities:				
NQDC Plan liability	18,272	11,859	—	30,131
Forward foreign currency exchange contract <sup>(1)</sup>	—	12,686	—	12,686
Contingent acquisition consideration payable	—	—	135,318	135,318
Total other long-term liabilities	18,272	24,545	135,318	178,135
Total liabilities	\$ 19,628	\$ 39,976	\$ 188,966	\$ 248,570

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## 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 at December 31, 2016			Total
	Quoted Price in			
	Active Markets	Significant Other	Significant	
	For Identical Assets (Level 1)	Observable Inputs (Level 2)	Unobservable Inputs (Level 3)	
<b>Assets:</b>				
Cash equivalents (including available-for-sale debt securities):				
Money market instruments	—	235,571	—	235,571
Corporate debt securities	—	8,593	—	8,593
U.S. government agency securities	—	6,000	—	6,000
Total cash and cash equivalents	—	250,164	—	250,164
Available-for-sale debt securities:				
Short-term:				
Certificates of deposit	—	2,800	—	2,800
Corporate debt securities	—	193,974	—	193,974
Commercial paper	—	16,075	—	16,075
U.S. government agency securities	—	168,498	—	168,498
Long-term:				
Corporate debt securities	—	437,150	—	437,150
U.S. government agency securities	—	135,427	—	135,427
Foreign and other	—	134	—	134
Total available-for-sale debt securities	—	954,058	—	954,058
Other Current Assets:				
Nonqualified Deferred Compensation Plan assets	—	163	—	163
Forward foreign currency exchange contract <sup>(1)</sup>	—	14,012	—	14,012
Restricted investments <sup>(2)</sup>	—	3,754	—	3,754
Total other current assets	—	17,929	—	17,929
Other Assets:				
Nonqualified Deferred Compensation Plan assets	—	9,121	—	9,121
Forward foreign currency exchange contract <sup>(1)</sup>	—	8,194	—	8,194
Strategic investment <sup>(3)</sup>	4,064	—	—	4,064
Total other assets	4,064	17,315	—	21,379
Total assets	\$4,064	\$ 1,239,466	\$ —	\$ 1,243,530
<b>Liabilities:</b>				

## Current Liabilities:

## Nonqualified Deferred Compensation Plan

liability	\$2,073	\$ 163	\$ —	\$2,236
Forward foreign currency exchange contract <sup>(1)</sup>	—	5,201	—	5,201
Contingent acquisition consideration payable	—	—	46,327	46,327
Total current liabilities	2,073	5,364	46,327	53,764

## Other long-term liabilities:

## Nonqualified Deferred Compensation Plan

liability	17,303	9,121	—	26,424
Forward foreign currency exchange contract <sup>(1)</sup>	—	2,342	—	2,342
Contingent acquisition consideration payable	—	—	115,310	115,310
Total other long-term liabilities	17,303	11,463	115,310	144,076
Total liabilities	\$19,376	\$ 16,827	\$ 161,637	\$197,840

(1) See Note 11 to these Consolidated Financial Statements for further information regarding the derivative instruments.

(2) The restricted investments as of December 31, 2017 and 2016 secure the Company's irrevocable standby letter of credit obtained in connection with certain commercial agreements.

(3) The Company had investments in marketable equity securities measured using quoted prices in an active market that are considered strategic investments and included in Other Assets on the Company's Balance Sheets. As of December 31, 2017, all holdings in the Company's strategic investments have been liquidated.

There were no transfers between levels during the year ended December 31, 2017.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

The Company's Level 2 securities 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. See Note 6 to these Consolidated Financial Statements for further information regarding the Company's financial instruments.

Liabilities measured at fair value using Level 3 inputs consisted of contingent acquisition consideration payable and asset retirement obligations.

The Company's contingent acquisition consideration payable is estimated using a probability-based income approach utilizing an appropriate discount rate. Key assumptions used by management to estimate the fair value of contingent acquisition consideration payable include estimated probabilities, the estimated timing of when a milestone may be attained and assumed discount periods and rates. Subsequent changes in the fair value of the contingent acquisition consideration payable, resulting from management's revision of key assumptions, will be recorded in Intangible Asset Amortization and Contingent Consideration in the Company's Consolidated Statements of Comprehensive Loss. The probability-based income approach used by management to estimate the fair value of the contingent acquisition consideration is most sensitive to changes in the estimated probabilities.

Contingent acquisition consideration payable at December 31, 2016	\$	161,637
Milestone payments to former Huxley Pharmaceuticals, Inc. shareholders		(3,500 )
Reversal of contingent liability related to revised estimate of Firdapse FDA acceptance and approval milestones		(4,245 )
Changes in the fair value of other contingent acquisition consideration payable		14,587
Foreign exchange remeasurement of Euro denominated contingent		20,487

acquisition consideration payable		
Contingent acquisition consideration payable at December 31, 2017	\$	188,966

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 lease agreement. The Company records an asset retirement obligation liability and a corresponding capital asset in an amount equal to the estimated fair value of the obligation, when estimable. In subsequent periods, for each such lease, the Company records interest expense to accrete the asset retirement obligation liability to full value and depreciates each capitalized asset retirement obligation asset, both over the term of the associated lease agreement. As of December 31, 2017, the balance of the asset retirement obligation liability was \$4.2 million.

The Company acquired intangible assets as a result of various business acquisitions. The estimated fair value of these long-lived assets was measured using Level 3 inputs as of the acquisition date.

### (13) DEBT

#### 2024 Convertible 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). The 2024 Notes were issued to the public at 98% of face value and bear interest at the rate of 0.599% per annum. The effective interest rate on the 2024 Notes the year ended December 31, 2017 was 1.1%. Interest is payable semi-annually in cash on February 1 and August 1 of each year, beginning February 1, 2018. The 2024 Notes are convertible, at the option of the holder into shares of the Company's common stock. The initial conversion rate for the 2024 Notes is 8.0212 shares per \$1,000 principal amount of the 2024 Notes, which represents a conversion price of approximately \$124.67 per share, subject to adjustment under certain conditions. Following certain corporate transactions, the Company will, in certain circumstances, increase the conversion rate for a holder that elects to convert its 2024 Notes in connection with such corporate transactions by a number of additional shares of the Company's common stock. A holder may convert fewer than all of such holder's 2024 Notes so long as the amount of the 2024 Notes converted is an integral multiple of \$1,000 principal amount. Net proceeds from the offering were \$481.7 million.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

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. These costs were deferred and are being amortized over the life of the 2024 Notes and recorded as additional interest expense.

#### 2018/2020 Convertible Notes

On October 15, 2013, the Company issued \$750.0 million in aggregate principal amount of senior subordinated convertible notes consisting of \$375.0 million in aggregate principal amount of 0.75% senior subordinated convertible notes with a maturity date of October 15, 2018 (the 2018 Notes) and \$375.0 million in aggregate principal amount of 1.50% senior subordinated convertible notes with a maturity date of October 15, 2020 (the 2020 Notes). Net proceeds from the offering were \$726.2 million.

The 2018 Notes and the 2020 Notes bear interest at a rate of 0.75% and 1.50% per year, respectively, which is payable semiannually in arrears on April 15 and October 15 of each year. The effective interest rate on the 2018 Notes and 2020 Notes the year ended December 31, 2017 was 6.0% and 7.5%, respectively.

The 2018 Notes and the 2020 Notes are senior unsecured obligations, and rank (i) subordinated to any of the Company's existing and future unsecured senior debt, (ii) equally to any of the Company's existing and future senior subordinated debt, (iii) senior to any of the Company's future indebtedness that is expressly subordinated to the 2018 Notes and the 2020 Notes, and (iv) effectively junior to any secured indebtedness to the extent of the value of the assets securing such indebtedness. Upon the occurrence of a "fundamental change", as defined in the indenture, the holders may require the Company to repurchase all or a portion of the 2018 Notes and the 2020 Notes for cash at 100% of the principal amount of the Notes being purchased, plus any accrued and unpaid interest.

The initial conversion rate for the 2018 Notes is 10.6213 shares per \$1,000 principal amount of the 2018 Notes, which represents a conversion price of approximately \$94.15 per share, and the initial conversion rate for the 2020 Notes is 10.6213 shares per \$1,000 principal amount of the 2020 Notes, which represents a conversion price of approximately \$94.15 per share. Such conversion rates are subject to adjustment under certain conditions. Holders may convert their 2018 Notes or 2020 Notes at their option at any time prior to July 15, 2018, in the case of the 2018 Notes, and July 15, 2020, in the case of the 2020 Notes, only under the following circumstances: (1) during any calendar quarter commencing after the calendar quarter ending on March 31, 2014 (and only during such calendar quarter), if the last



reported sale price of the Company's common stock for at least 20 trading days (whether or not consecutive) during a period of 30 consecutive trading days ending on the last trading day of the immediately preceding calendar quarter is greater than or equal to 130% of the applicable conversion price on each applicable trading day; (2) during the five business day period after any five consecutive trading day period (the measurement period) in which the trading price per \$1,000 principal amount of the relevant notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price of the Company's common stock and the applicable conversion rate on each such trading day; or (3) upon the occurrence of specified corporate events. On or after July 15, 2018, in the case of the 2018 Notes, or July 15, 2020, in the case of the 2020 Notes, until the close of business on the second scheduled trading day immediately preceding the applicable maturity date, holders may convert their notes at any time, regardless of the foregoing circumstances. Upon conversion of the 2018 Notes and the 2020 Notes, the Company may pay cash, shares of the Company's common stock or a combination of cash and stock, as determined by the Company in its discretion.

The Company has separately accounted for the liability and equity components of the 2018 Notes and the 2020 Notes by allocating the proceeds from issuance of the 2018 Notes and the 2020 Notes between the liability component and the embedded conversion option, or equity component. This allocation was done by first

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BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

estimating an interest rate at the time of issuance for similar notes that do not include the embedded conversion option. The Company allocated \$156.2 million to the equity component, net of offering costs of \$5.1 million. The Company recorded a discount on the notes of \$161.3 million which will be accreted and recorded as additional interest expense over the life of the 2018 Notes and the 2020 Notes. Additionally, in connection with the issuance of the 2018 Notes and the 2020 Notes, the Company incurred \$23.8 million of issuance costs, which are being amortized and recorded as additional interest expense over the life of the Notes.

To minimize the impact of potential dilution upon conversion of the 2018 Notes and the 2020 Notes, the Company entered into capped call transactions separate from the issuance of the Notes with certain counterparties covering 3,982,988 shares of the Company's common stock, subject to adjustment. The capped calls have a strike price of \$94.15 and a cap price of \$121.05 and are exercisable when and if the Notes are converted. If upon conversion of the Notes, the price of the Company's common stock is above the strike price of the capped calls, the counterparties will deliver shares of the Company's common stock and/or cash with an aggregate value equal to the difference between the price of the Company's common stock at the conversion date and the strike price, multiplied by the number of shares of the Company's common stock related to the capped calls being exercised. The Company paid \$29.8 million for these capped calls transactions, which was recorded as additional paid-in capital.

#### 2017 Convertible Notes

In April 2007, the Company sold \$324.9 million in aggregate principal amount of senior subordinated convertible notes due in April 2017 (the 2017 Notes). The 2017 Notes were issued at face value and bore interest at the rate of 1.875% per annum, payable semi-annually in cash. The 2017 Notes were convertible, at the option of the holder, at any time prior to maturity or repurchase, into shares of the Company's common stock at a conversion rate of 49.1171 shares per \$1,000 principal amount of the 2017 Notes, which is equal to a conversion price of approximately \$20.36 per share, subject to adjustment in certain circumstances. The 2017 Notes did not include a call provision and the Company was unable to unilaterally redeem the 2017 Notes prior to maturity on April 23, 2017. During the twelve months ended December 31, 2017, 2016 and 2015, certain existing holders of the 2017 Notes elected to convert \$22.5 million, \$8.9 million and \$8.1 million in aggregate principal amount of the 2017 Notes into 1,103,998, 438,462 and 399,469 shares, respectively, of the Company's common stock. The 2017 Notes matured on April 23, 2017 with conversion of all principal amounts except for a final cash settlement of \$26,000.

## 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 summarizes information regarding the Company's convertible debt at December 31:

	2017	2016
Convertible Notes due 2017	\$—	\$22,503
Unamortized deferred offering costs	—	(25 )
Convertible Notes due 2017, net	—	22,478
Convertible Notes due 2018	374,980	374,980
Unamortized discount	(12,488 )	(27,566 )
Unamortized deferred offering costs	(1,543 )	(3,484 )
Convertible Notes due 2018, net	360,949	343,930
Convertible Notes due 2020	374,993	374,993
Unamortized discount	(40,287 )	(53,239 )
Unamortized deferred offering costs	(3,631 )	(4,923 )
Convertible Notes due 2020, net	331,075	316,831
Convertible Notes due in 2024	495,000	—
Unamortized discount	(9,355 )	—
Unamortized deferred offering costs	(3,199 )	—
Convertible Notes due in 2024, net	482,446	—
Total convertible debt, net	\$ 1,174,470	\$ 683,239
Fair value of fixed rate convertible debt		
Convertible Notes due in 2017 <sup>(1)</sup>	\$—	\$90,977
Convertible Notes due in 2018 <sup>(1)</sup>	403,955	423,202
Convertible Notes due in 2020 <sup>(1)</sup>	446,470	442,754
Convertible Notes due in 2024 <sup>(1)</sup>	493,894	—
Total	\$ 1,344,319	\$ 956,933

(1) The fair value of the Company's fixed rate convertible debt is based on open market trades and is classified as Level 1 in the fair value hierarchy.

See Note 14 to these Consolidated Financial Statements for further discussion of the effect of conversion on net loss per common share.

## Revolving Credit Facility

In November 2016, the Company entered into a credit agreement (Credit Agreement) providing for up to \$100.0 million (Revolving Credit Facility), a \$10.0 million letter of credit subfacility and a \$15.0 million swing line loan subfacility. The maturity date of the Revolving Credit Facility will occur on November 29, 2018. Interest on any

outstanding balance of the Revolving Credit Facility is payable quarterly and draws may be voluntary prepaid at any time without penalty. In connection with entering into the Credit Agreement, \$0.6 million in financing costs was incurred and will be amortized as Interest Expense over the term of the Credit Agreement. As of December 31, 2017, there were no outstanding amounts due under the Revolving Credit Facility.

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## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

In connection with the Revolving Credit Facility, the Company and certain of its subsidiaries are required to comply with covenants, including, among other things, restrictions on the Company's and such subsidiaries' ability to incur additional indebtedness, dispose of its assets, incur liens, make investments, and pay dividends or other distributions, in each case subject to specified exceptions. The Credit Agreement also contains customary indemnification obligations and customary events of default. If the Company's Global Liquidity, which is defined as the sum of the market value of unrestricted cash, marketable securities and other assets to the extent constituting "cash and cash equivalents," "short-term investments" or "long-term investments" as reflected in the Company's Consolidated Balance Sheet, in each case, held by the Company or certain of the Company's subsidiaries at such time, regardless of where such assets are domiciled, falls below \$225.0 million at the end of any month or at the time of any borrowing or issuance of a letter of credit under the Revolving Credit Facility, then the Company's obligations under the Credit Agreement will also be secured by the assets held by the Company in the custody account, which was established in the first quarter of 2017. As of December 31, 2017, the Company and certain of its subsidiaries that serve as guarantors are in compliance with all covenants.

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

	Years Ended December 31,		
	2017	2016	2015
Coupon interest	\$10,407	\$9,555	\$9,750
Amortization of debt issuance costs	3,725	3,367	3,294
Accretion of discount on convertible notes	28,575	26,577	25,200
Total interest expense on convertible debt	\$42,707	\$39,499	\$38,244

## (14) NET 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 restricted stock units (RSUs), common stock held by the NQDC and contingent issuances of common stock related to convertible debt.

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

	Years Ended December 31,		
	2017	2016	2015
Numerator:			
Net loss, basic	\$(117,042)	\$(630,210)	\$(171,799)

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Less: gain on common stock held by the NQDC	—	(3,184 )	—
Net loss, diluted	(117,042)	(633,394)	(171,799)
Denominator:			
Weighted-average common shares outstanding, basic	174,427	165,985	160,025
Effect of dilutive securities:			
Common stock held by the NQDC	—	234	—
Weighted-average common shares outstanding, diluted	174,427	166,219	160,025
Net loss per common share, basic	\$(0.67 )	\$(3.80 )	\$(1.07 )
Net loss per common share, diluted	\$(0.67 )	\$(3.81 )	\$(1.07 )

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## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

The table below presents potential shares of common stock that were excluded from the computation of basic and diluted earnings per common share as they were anti-dilutive using the if-converted or treasury stock method (in thousands):

	Years Ended December		
	31, 2017	2016	2015
Options to purchase common stock	8,108	8,856	10,323
Common stock issuable under the 2017 Notes	—	1,105	1,544
Common stock issuable under the 2018 Notes	3,983	3,983	3,983
Common stock issuable under the 2020 Notes	3,983	3,983	3,983
Common stock issuable under the 2024 Notes	3,970	—	—
Unvested restricted stock units	2,911	2,618	1,743
Common stock potentially issuable for ESPP purchases	436	404	312
Common stock held by the NQDC	220	—	243
Total number of potentially issuable shares	23,611	20,949	22,131

The potential effect of the capped call transactions with respect to the 2018 Notes and the 2020 Notes was excluded from the diluted net income/loss per share as the Company's closing stock price on December 31, 2017 and 2016 did not exceed the conversion price of \$94.15 per share for the 2018 Notes and the 2020 Notes. Although the Company's stock price exceeded the conversion price \$94.15 at December 31, 2015, the potential shares issuable under the 2018 Notes and the 2020 Notes were excluded from the calculation of diluted loss per share as they were anti-dilutive using the if-converted method. There is no similar capped call transaction associated with the 2024 Notes. See Note 13 to these Consolidated Financial Statements for information on the Company's debt.

## (15) INCOME TAXES

The provision for (benefit from) income taxes is based on loss before income taxes as follows:

	Years Ended December 31,		
	2017	2016	2015
U.S. Source	\$(19,461)	\$10,696	\$182,215
Non-U.S. Source	(16,414)	(841,746)	(336,939)
Loss before income taxes	\$(35,875)	\$(831,050)	\$(154,724)

The U.S. and foreign components of the provision for (benefit from) income taxes are as follows:

	Years Ended December 31,		
	2017	2016	2015
Provision for current income tax expense:			
Federal	\$29,848	\$22,239	\$84,743
State and local	2,880	1,418	5,323
Foreign	3,975	3,557	3,836
	36,703	27,214	93,902
Provision for (benefit from) deferred income tax			
expense:			
Federal	12,446	(78,428 )	(17,741)
State and local	32,336	(6,012 )	(8,770 )
Foreign	(318 )	(143,614)	(50,316)
	44,464	(228,054)	(76,827)
Provision for (benefit from) income taxes	\$81,167	\$(200,840)	\$17,075

On December 22, 2017, the bill known as the Tax Cuts and Jobs Act (the 2017 Tax Act) was signed into law. The new law has resulted in significant changes to the U.S. corporate income tax system. These changes include a federal statutory rate reduction from 35% to 21% and the elimination or reduction of certain domestic deductions and credits, including a 50% reduction in the orphan drug credit benefit. The 2017 Tax Act changed U.S. international taxation from a worldwide basis to a modified territorial system that includes base erosion



## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

prevention measures on foreign earnings. This will result in the Company's foreign subsidiaries being subject to U.S. taxation in the future. These changes are effective in 2018. Changes to tax laws and tax rates are required to be accounted for in the period of the enactment, therefore the Company's tax expense for the year ended December 31, 2017 included the impact of the 2017 Tax Act. The Company's deferred tax assets and liabilities are measured at the enacted tax rate expected to apply when these temporary differences are expected to be realized or settled. The Company recorded a provisional expense of \$42.3 million related to the 2017 Tax Act, which included \$33.1 million for the re-measurement of the net deferred tax assets at the lower enacted corporate tax rate and \$9.2 million related to the increased limitations on executive compensation. The Company also assessed the impact of the 2017 Tax Act on its financial projections and concluded that it is more likely than not that certain of its state tax credits will not be utilized in the foreseeable future, and recognized \$41.4 million of income tax expense during the fourth quarter of 2017 to establish a valuation allowance against those state tax credits. These credits do not expire, however, the Company projects that it will be generating more credits than it will utilize on an annual basis. The 2017 Tax Act also includes a one-time mandatory deemed repatriation toll tax on accumulated earnings of our foreign subsidiaries that did not impact the Company due to a net deficit in these foreign subsidiaries.

The incremental net income tax expense recognized as a result of the 2017 Tax Act was an estimate and the measurement of deferred tax assets is subject to further analysis and potential correlative adjustments as developing interpretations and guidance from the U.S. Treasury Department, the IRS, and other standard setting bodies provide additional clarifications of the provisions of the 2017 Tax Act. Updated guidance and regulations could result in changes to this estimate during 2018 when the analysis is complete. The Company has not yet elected an accounting method regarding whether to record deferred tax assets and liabilities for expected amounts of Global Intangible Low-Taxed Income (GILTI) inclusions or whether to treat such amounts as a period cost.

For the year ended December 31, 2016, the Company's Dutch operations had a book net loss of \$539.2 million, which included the impairment of the Kyndrisa IPR&D assets and a resulting deferred tax benefit of \$143.5 million associated with the reversal of the deferred tax liability of such IPR&D assets.

The following is a reconciliation of the statutory federal income tax rate to the Company's effective income tax rate expressed as a percentage of loss before income taxes:

	Years Ended December 31,		
	2017	2016	2015
Federal statutory income tax rate	35.0 %	35.0 %	35.0 %
State and local taxes	(20.3 )%	0.4 %	(2.2 )%
Orphan Drug & General Business Credit	93.9 %	7.5 %	34.8 %
Stock compensation expense	19.1 %	4.6 %	(2.8 )%
Changes in the fair value of contingent acquisition consideration payable	(3.1 )%	0.9 %	0.2 %
Subpart F income	(84.1 )%	— %	(8.4 )%
Foreign tax rate differential	(26.2 )%	(18.6)%	(46.2)%
Section 162(m) limitation	(26.5 )%	(5.4 )%	(1.3 )%
Tax Cuts and Jobs Act of 2017	(118.0)%	— %	— %

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Other	1.9	%	0.3	%	(1.6)	)%
Valuation allowance/deferred benefit	(97.9)	)%	(0.5)	)%	(18.5)	)%
Effective income tax rate	(226.2)	)%	24.2	%	(11.0)	)%

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## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

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

	December 31,	
	2017	2016
Net deferred tax assets:		
Net operating loss carryforwards	\$48,374	\$49,787
Tax credit carryforwards	384,381	352,535
Accrued expenses, reserves, and prepaids	54,565	77,904
Intangible assets	17,556	26,751
Stock-based compensation	31,371	47,713
Inventory	13,206	15,581
Impairment	2,609	5,017
Other	2,358	1,519
Valuation allowance	(111,001)	(73,037)
Total deferred tax assets	443,419	503,770
Joint venture basis difference	(1,229 )	(1,714 )
Acquired intangibles	(3,332 )	(8,773 )
Convertible notes discount	(10,100 )	(24,394 )
Property, plant and equipment	(29,663 )	(22,103 )
Total deferred tax liabilities	(44,324 )	(56,984 )
Net deferred tax assets	\$399,095	\$446,786

In 2017, the increase in the valuation allowance was primarily due to the state tax credits discussed above. The incremental expense was partially offset by a reduction in the valuation allowance related to fully valued assets that expired in 2017.

As of December 31, 2017, the Company had the following net operating loss and tax credit carryforwards, which if not utilized, will expire as follows (dollars in thousands):

Type	Amount	Year
Federal net operating loss carryforwards	\$15,554	2028 – 2033
Federal R&D and orphan drug credit carryforwards	\$403,828	2030 – 2037
State net operating loss carryforwards	\$108,133	2019 – 2036
Dutch net operating loss carryforwards	\$145,150	2020 – 2025

The \$9.5 million of Irish net operating losses and \$83.7 million of state research credit carryovers 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.

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## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

The financial statement recognition of the benefit for a tax position is dependent upon the benefit being more likely than not to be sustainable upon audit by the applicable taxing authority. If this threshold is met, the tax benefit is then measured and recognized at the largest amount that is greater than 50% likely of being realized upon ultimate settlement. A reconciliation of the beginning and ending amount of unrecognized tax benefits for the years ended December 31, 2017 and 2016 is as follows:

	December 31,	
	2017	2016
Balance at beginning of period	\$ 103,210	\$ 86,731
Additions based on tax positions related to the		
current year	11,042	15,982
(Deletions) Additions for tax positions of prior years	(766 )	497
Balance at end of period	\$ 113,486	\$ 103,210

Included in the balance of unrecognized tax benefits at December 31, 2017 are potential benefits of \$113.5 million that, if recognized, would affect the effective tax rate. The Company's policy for classifying interest and penalties associated with unrecognized income tax benefits is to include such items in the income tax expense. The total amount of accrued interest and penalties was not significant as of December 31, 2017.

The Company files income tax returns in the U.S. 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. Currently, the Company is under audit by the Internal Revenue Service for the year 2014.

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 \$10.7 million as of December 31, 2017, which will be indefinitely reinvested; deferred income taxes have not been provided on such foreign earnings.

## (16) EQUITY COMPENSATION PLANS

## Share Incentive Plan

The Company's stock-based compensation plans include the 2017 Equity Incentive Plan and the ESPP. The 2017 Equity Incentive Plan, which was approved by the Company's stockholders on June 6, 2017 and became effective that

same date, and is the successor to and continuation of the Company's Amended and Restated 2006 Share Incentive Plan (the 2006 Share Incentive Plan), provides for awards of RSUs and stock options as well as other forms of equity compensation. 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. 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. RSUs granted to directors generally vest in full one year after the grant date.

Shares formerly reserved for future issuance under the 2006 Share Incentive Plan were transferred to the 2017 Equity Incentive Plan, from which future shares shall be issued. The Company's stock-based compensation plans are administered by the Company's Board of Directors, 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. As of December 31, 2017, options to purchase approximately 8.1 million shares were outstanding under the Company's stock option plans. As of December 31, 2017, approximately 10.9 million shares were authorized for future issuance under the 2017 Equity Incentive Plan.

#### Employee Stock Purchase Plan

Under BioMarin's ESPP, which was initially approved in June 2006, replacing the Company's previous plan, and was further amended on March 5, 2014, employees meeting specific employment qualifications are eligible to participate and can purchase shares on established dates (each purchase date) semi-annually through

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

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 IRC. During the year ended December 31, 2017, the Company issued 0.2 million shares under the ESPP.

As of December 31, 2017, there were approximately 3.5 million shares were authorized and 0.6 million shares reserved for future issuance under the ESPP.

#### Board of Director Grants

On September 28, 2017, the Board of Directors (the Board) approved revised compensation for the Independent Directors of the Company as follows. 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 \$375,000, with the number of RSUs to be granted calculated based on the three month trailing average closing price of our 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.

Prior to September 28, 2017, each Independent Director was automatically granted an initial equity grant valued at \$550,000, with the number of equity awards to be granted calculated based on the three month trailing average closing price of our common stock on the Nasdaq Global Select Market. The initial value was allocated 40% to RSUs and 60% to options to purchase shares of the Company’s common stock (stock options) on the date that such person first becomes an Independent Director. The stock options subject to the initial grant vest quarterly over three years and the initial RSU grant vest annually over three years. On the date of the Company’s annual meeting of stockholders, each re-elected Independent Director was granted an additional equity grant valued at \$375,000 with such valuation allocated 50% to RSUs and 50% to stock options. The stock options subject to the annual grant vest quarterly over one year and the additional annual RSUs vest in full on the one-year anniversary of the grant date. The additional stock option grant or RSU grant for a director that served for less than a year was prorated to the nearest quarter. These stock options and RSUs continue to vest only while the Independent Director serves on the Board. The exercise price per share of each of these stock options is 100% of the fair market value of a share of the Company’s common stock on the date of the grant. These stock options have a contractual term of 10 years from grant date.

See Note 3 to these Consolidated Financial Statements for discussion regarding the valuation of equity awards.

#### Shares Available Under Equity Compensation Plans

At December 31, 2017, an aggregate of approximately 30.0 million unissued shares was authorized for future issuance under the Company’s stock plans, which includes shares issuable under the 2017 Equity Incentive Plan, the ESPP and the Company’s expired plans. Under the 2017 Equity Incentive Plan, shares issued under 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.

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## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

## (17) STOCK-BASED COMPENSATION

## Stock Options

The following table summarizes activity under the Company's stock option plans, including the 2012 and 2014 Inducement Plans and those suspended upon the adoption of the 2017 Share Incentive Plan, for the year ended December 31, 2017. All 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, 2016	8,856,208	\$ 51.13	5.4	\$ 304,356
Granted	801,170	\$ 87.74		
Exercised	(1,367,307)	\$ 34.96		
Expired and forfeited	(182,090 )	\$ 92.98		
Options outstanding as of				
December 31, 2017	8,107,981	\$ 56.53	5.1	\$ 281,141
Options invested at				
December 31, 2017	1,462,941	\$ 88.19	8.5	\$ 6,313
Exercisable at December 31, 2017	6,645,032	\$ 49.56	4.3	\$ 274,828

(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 \$89.17, the closing price of the Company's common stock on the Nasdaq Global Select Market on December 29, 2017, the last trading day of the year.

The weighted-average fair value per option granted in the years ended December 31, 2017, 2016 and 2015 were \$36.07, \$40.70 and \$56.76, respectively. The total intrinsic value of options exercised during the years ended December 31, 2017, 2016 and 2015 was \$77.0 million, \$127.4 million and \$146.6 million, respectively. The aggregate intrinsic value of options exercised was determined as of the date of option exercise. Upon the exercise of the options, the Company issues new common stock from its authorized shares. There were 7.4 million options that were

in-the-money at December 31, 2017.

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,		
	2017	2016	2015
Expected volatility	38 – 40%	36 – 44%	36 – 45%
Dividend yield	0.00%	0.00%	0.00%
Expected life	4.9 – 6.6 years	5.0 – 8.1 years	6.4 – 8.0 years
Risk-free interest rate	1.8 – 2.2%	1.1 – 2.3%	1.5 – 2.2%

The Company recorded \$36.7 million, \$45.5 million and \$41.5 million of compensation costs related to current period vesting of stock options for the years ended December 31, 2017, 2016 and 2015, respectively. As of December 31, 2017, the total unrecognized compensation cost related to unvested stock options was \$49.2 million. These costs are expected to be recognized over a weighted average period of 2.4 years. The net tax benefit from stock options exercised during the year ended December 31, 2017 was \$7.8 million.

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## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

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,		
	2017	2016	2015
Expected volatility	28 – 42%	42 – 50%	36 – 38%
Dividend yield	0.00%	0.00%	0.00%
Expected life	6 – 24 months	6 – 24 months	6 – 24 months
Risk-free interest rate	1.0 – 1.6%	0.4 – 0.8%	0.1 – 0.8%

The Company recorded \$11.7 million, \$10.1 million and \$7.1 million of compensation costs related to shares granted under the ESPP for the years ended December 31, 2017, 2016 and 2015, respectively. As of December 31, 2017, there was \$12.9 million of total unrecognized compensation cost related to unvested stock options issuable under the ESPP. These costs are expected to be recognized over a weighted average period of 1.4 years.

## Restricted Stock Unit Awards with Service-Based Vesting Conditions

RSUs are generally subject to forfeiture if employment terminates prior to the release of vesting restrictions.

Below is a summary of RSU activity under the plan for the year ended December 31, 2017:

	Shares	Weighted		
		Average	Weighted	
		Grant	Average	Aggregate
		Date	Remaining	Intrinsic
		Fair	Years	Value
		Value		
Non-vested units as of December 31, 2016	2,444,966	\$ 92.70	1.4	\$ 202,541
Granted	1,382,900	\$ 87.88		
Vested	(837,926 )	\$ 92.00		
Forfeited	(310,406 )	\$ 96.02		
Non-vested units as of December 31, 2017	2,679,534	\$ 90.04	1.4	\$ 238,934

The weighted-average grant date fair value per share of RSUs granted during the years ended December 31, 2017, 2016 and 2015, was \$87.88, \$84.18 and \$119.86, respectively. The total intrinsic value of restricted stock that vested and was released in the years ended December 31, 2017, 2016 and 2015 was \$76.5 million, \$63.5 million and \$59.5

million, respectively.

The Company recorded \$86.5 million, \$74.7 million and \$47.9 million of compensation costs related to RSUs with service-based vesting conditions for the years ended December 31, 2017, 2016 and 2015, respectively. As of December 31, 2017, there was \$173.7 million of total unrecognized compensation cost related to unvested RSUs with service-based vesting conditions. These costs are expected to be recognized over a weighted average period of 1.3 years.

#### Restricted Stock Unit Awards with Performance 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 to certain executive officers. In March 2017, the Compensation Committee and Board approved the grant of 133,250 RSUs (base RSUs) with performance-based vesting conditions. This award is contingent upon the achievement of a 2017 revenue target and the number of shares that may be earned range between 50% and 200% of the base RSUs, dependent on the percentage of 2017 “managed revenues” (defined as the Company’s net product revenues, excluding net revenues attributable to Aldurazyme, and determined using fixed foreign currency exchange rates) achieved against the target managed revenues, with a threshold achievement level of 75% of target and a ceiling achievement level of 125% of target. RSUs with performance-based vesting conditions with similar performance conditions were granted in 2016 and 2015.

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## 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 details the base RSUs granted, RSUs earned and expected to vest and the performance multiplier achieved and compensation expense recognized for the RSUs with performance-based vesting conditions for the years ended December 31, 2017, 2016 and 2015, respectively (dollars in thousands, except per RSU amounts):

Date of Grant	Base RSUs Granted	Grant Date Fair Value per RSU	Multiplier Achieved	RSUs Earned	Compensation Expense for the Years Ended December 31,		
					2017	2016	2015
March 2017 <sup>(1)</sup>	133,250	\$87.42	103 %	131,651	\$4,141	\$—	\$—
March 2016 <sup>(2)</sup>	130,310	\$83.43	103 %	134,219	\$3,928	\$2,956	\$—
March 2015 <sup>(2)</sup>	58,300	\$108.36	111 %	64,713	\$2,291	\$2,342	\$1,805

(1)Based on the Company's performance against the 2017 revenue target, the Company expects its Compensation Committee to approve a multiplier of 103% and the participating executive officers to earn 131,651 RSUs, which will continue to vest ratably over a three-year service period.

(2)The RSUs with performance-based vesting conditions granted in 2016 and 2015 were earned on the one year anniversary upon achievement of the respective performance target and vest ratably over a three-year service period.

As of December 31, 2017, total unrecognized compensation expense of \$12.4 million related to RSU awards with performance-vesting conditions is expected to be recognized over a weighted average period of 1.8 years.

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

	Years Ended December 31,		
	2017	2016	2015
Cost of sales	\$10,636	\$9,121	\$6,836
Research and development	53,112	58,279	49,399
Selling, general and administrative	76,515	67,241	55,290
Total stock-based compensation expense	\$140,263	\$134,641	\$111,525

Stock-based compensation of \$16.1 million, \$11.4 million and \$11.1 million was capitalized into inventory, for the years ended December 31, 2017, 2016 and 2015, respectively. Capitalized stock-based compensation is recognized as

cost of sales when the related product is sold.

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## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

## (18) COMPREHENSIVE INCOME

The following table summarizes amounts reclassified out of Accumulated Other Comprehensive Income (AOCI) and their effect on the Company's Consolidated Statements of Operations for the years ended December 31, 2017 and 2016.

Details about AOCI Components	Amount Reclassified from AOCI (Gain) Loss		Consolidated Statement of Operations Classification
	Years Ended		
	December 31, 2017	2016	
Gains (losses) on cash flow hedges:			
Forward foreign currency exchange			
contracts	\$ (5,377 )	\$ 6,112	Net product revenues
Forward foreign currency exchange			
contracts	264	4,161	Selling, general and administrative
Total gain (loss) on cash flow			
hedges	(5,113 )	10,273	
Gain (loss) on sale of available-for-sale			
debt securities	3,252	(115 )	Other income (expense)
Less income tax effect of the above	1,191	42	Provision for (benefit from) income taxes
	\$ (3,052 )	\$ 10,116	Net loss

The following table summarizes changes in the accumulated balances for each component of other comprehensive loss, including current period reclassifications out of AOCI and other amounts of current-period other comprehensive income, for the years ended December 31, 2017 and 2016.

Year Ended Balance at December 31, 2017			
Gains and Losses on Cash Flow	Unrealized Gains on Available-for-Sale Debt Securities	Foreign Currency Items	Total

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	Hedges					
AOCI balance at December 31, 2016	13,006	(178	)	(12	) 12,816	
Other comprehensive income (loss) before						
reclassifications	(38,351)	(755	)	5	(39,101)	
Less: gain (loss) reclassified from AOCI	(5,113	)	3,252		(1,861	)
Tax effect	—	1,463		—	1,463	
Net current-period other comprehensive income (loss)	(33,238)	(2,544	)	5	(35,777)	
AOCI balance at December 31, 2017	\$(20,232)	\$ (2,722	)	\$ (7	) \$(22,961)	

	Year Ended Balance at December 31, 2016					
	Gains and Losses					
	on Cash Flow Hedges	Unrealized Gains on Available-for-Sale Debt Securities	Foreign Currency Items	Total		
AOCI balance at December 31, 2015	13,602	7,441	(10	) 21,033		
Other comprehensive income (loss) before						
reclassifications	9,677	(12,104	)	(2	) (2,429)	
Less: net gain (loss) reclassified from AOCI	10,273	(115	)		10,158	
Tax effect	—	4,370		—	4,370	
Net current-period other comprehensive loss	(596	)	(7,619	)	(2	) (8,217)
AOCI balance at December 31, 2016	13,006	(178	)	(12	) 12,816	



## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

## (19) REVENUE AND CREDIT CONCENTRATIONS

Net Product Revenue - The Company considers there to be revenue concentration risks for regions where net product revenue exceeds 10% of consolidated net product revenue. The concentration of the Company's net product revenue within the regions below may have a material adverse effect on the Company's revenue and results of operations if sales in the respective regions experience difficulties.

The table below summarizes consolidated net product revenue concentrations based on patient location for Brineura, Firdapse, Kuvan, Naglazyme, and Vimizim, which are sold directly by the Company, and global sales of Aldurazyme, which is marketed by Genzyme. Genzyme is the Company's sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third parties.

	Years Ended December 31,					
	2017	2016	2015			
<b>Region:</b>						
United States	39 %	37 %	39 %			
Europe	21 %	23 %	19 %			
Latin America	14 %	13 %	16 %			
Rest of world	19 %	19 %	15 %			
Total net product revenues marketed by the						
Company	93 %	92 %	89 %			
Aldurazyme net product revenues marketed by						
Genzyme	7 %	8 %	11 %			
Total net product revenue	100 %	100 %	100 %			

The following table illustrates the percentage of the Company's consolidated net product revenues attributed to the Company's largest customers.

	For the Years Ended December 31,					
	2017	2016	2015			
Customer A	18 %	19 %	15 %			
Customer B	14 %	13 %	13 %			
Customer C	10 %	10 %	—			
Customer D	7 %	8 %	11 %			
Customer E	7 %	6 %	10 %			

Total                    56   %                    56   %                    49   %

On a consolidated basis, the Company’s two largest customers accounted for 21% and 18% of the December 31, 2017 accounts receivable balance, respectively, compared to December 31, 2016 when the two largest customers accounted for 26% and 20% of the accounts receivable balance, respectively. As of December 31, 2017 and 2016, accounts receivable balance for Genzyme included \$18.1 million and \$30.7 million, respectively, of unbilled accounts receivable related to net incremental Aldurazyme product transfers to Genzyme. The Company does not require collateral from its customers, but does perform periodic credit evaluations of its customers’ financial condition and requires immediate payment in certain circumstances.

The Company sells its products in countries that face economic volatility and weakness. Although the Company has historically collected receivables from customers in such countries, sustained weakness or further deterioration of the local economies and currencies may cause customers in those countries to be unable to pay for the Company’s products. The Company has not historically experienced a significant level of uncollected receivables and has received continued payments from its more aged accounts in these countries. The Company believes that the allowances for doubtful accounts related to these countries, if any, is adequate based on its analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

## (20) SEGMENT 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.

	Years Ended December 31,		
	2017	2016	2015
Net product revenues by product:			
Aldurazyme	\$89,959	\$93,749	\$97,912
Brineura	8,595	—	—
Firdapse	18,890	18,028	16,037
Kuvan	407,542	348,009	239,336
Naglazyme	332,208	296,537	303,090
Vimizim	413,251	354,058	228,147
Total net product revenues	\$1,270,445	\$1,110,381	\$884,522

The following table summarizes 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 based on the location of Genzyme's headquarters. Although Genzyme sells Aldurazyme worldwide, the revenues earned by the Company based on Genzyme's net sales are included in the U.S. region as the transactions are with Genzyme whose headquarters are located in the U.S.

	Years Ended December 31,		
	2017	2016	2015
Total revenues by geographic region:			
United States	\$588,243	\$507,539	\$444,075
Europe	301,487	252,633	171,216
Latin America	182,438	147,471	142,305
Rest of world	241,478	209,211	132,299
Total revenues	\$1,313,646	\$1,116,854	\$889,895

The following table summarizes non-monetary long-lived assets by geographic region. Non-monetary long-lived assets primarily consist of property, plant and equipment, intangible assets, deferred tax assets and goodwill.

December 31,

	2017	2016
Long-lived assets by geography:		
United States	\$1,183,791	\$1,183,938
Europe	823,657	812,833
Rest of world	2,694	2,568
Total long-lived assets	\$2,010,142	\$1,999,339

## (21) COLLABORATION AND LICENSE AGREEMENTS

In July 2017, the Company executed a license agreement and a settlement agreement (the Sarepta Agreements) 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. The Sarepta Agreements resolved the ongoing worldwide patent proceedings related to the use of EXONDYS 51 and all future exon-skipping products for the treatment of DMD. Pursuant to the Sarepta Agreements, Sarepta paid the Company a one-time upfront fee of \$35.0 million, which was recognized as license revenue. Under the Sarepta 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, Sarepta will pay the Company 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

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BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

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.

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 90 days written notice.

On October 6, 2015, the Company completed the sale of talazoparib to Medivation Inc. (Medivation) pursuant to an asset purchase agreement (the Medivation Asset Purchase Agreement). Pursuant to the Medivation Asset Purchase Agreement, Medivation paid the Company an upfront payment of \$410.0 million upon the closing of the transaction. In addition, contingent upon the successful development and commercialization of talazoparib, In September 2016, Pfizer Inc. acquired Medivation, therefore obligations under the Medivation Asset Purchase Agreement transferred to Pfizer. In accordance with the Medivation Asset Purchase Agreement, Pfizer will pay the Company milestone payments of up to \$160.0 million and mid-single digit percentage royalties on net sales of talazoparib. During the fourth quarter of 2015, the Company recognized a net gain of \$369.5 million related to the sale of the talazoparib intangible assets.

In October 2012, the Company licensed to Catalyst Pharmaceutical Partners, Inc., (Catalyst) the North American rights to develop and market Firdapse. In consideration of this licensing arrangement, the Company received from Catalyst a \$5.0 million convertible promissory note. Under the terms of the note agreement, the Company received 6.7 million shares of Catalyst common stock upon the automatic conversion of the convertible promissory note on December 10, 2012. In exchange for the North American rights to Firdapse the Company may receive royalties of 7% to 10% on net product sales of Firdapse in North America. As of December 31, 2017, there were no amounts due from Catalyst for reimbursable development costs and the Company no longer held shares of Catalyst common stock.

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.

## (22) COMPENSATION AGREEMENTS AND PLANS

### 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). Most employees (Participants) are eligible to participate following the start of their employment, at the beginning of each calendar month. Participants may contribute to the 401(k) Plan up to the lesser of 100% 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 matched 100% of each Participant's contributions, up to a maximum of the lesser of 6% of the employee's annual compensation or \$14,000 per year (\$16,000 per year effective January 1, 2018). The Company's matching contribution vests over four years from employment commencement and was approximately \$19.8 million, \$16.0 million and \$15.1 million for the years ended December 31, 2017, 2016 and 2015, respectively. Employer contributions not vested upon employee termination are forfeited.

#### Deferred Compensation Plan

In December 2005, the Company adopted the Deferred Compensation Plan. All of the investments held in the NQDC Plan are classified as trading securities and recorded at fair value with changes in the investments' fair values recognized as earnings in the period they occur. Company stock issued and held by the NQDC Plan is accounted for similarly to treasury stock in that the value of the employer stock is determined on the date the restricted stock vests and the shares are issued into the NQDC Plan. The restricted stock issued into the NQDC Plan is recorded as stockholders' equity and changes in the fair value of the corresponding liability are recognized in earnings as incurred. The corresponding liabilities for the NQDC Plan are included in Accounts Payable and Accrued Liabilities and Other Long-Term Liabilities in the Company's Consolidated Balance Sheets. The corresponding assets for the NQDC Plan are included in Other Current Assets and Other Assets in the Company's Consolidated Balance Sheets.

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## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

As of December 31, 2017 and 2016, the fair value of Company stock held by the Deferred Compensation Plan, was \$19.6 million and \$19.4 million, respectively, which is included in current and non-current liabilities. The change in market value amounted to a loss of \$1.4 million, a gain of \$5.0 million and a gain of \$2.5 million in the years 2017, 2016 and 2015, respectively. See Note 12 to these Consolidated Financial Statements for additional discussion regarding the fair value of the Deferred Compensation Plan assets and liabilities.

## (23) COMMITMENTS AND CONTINGENCIES

## Lease Commitments

The Company leases office space and research, testing and manufacturing laboratory space in various facilities under operating agreements expiring at various dates through 2026. Certain of the leases provide for options by the Company to extend the lease for multiple five-year renewal periods and also provide for annual minimum increases in rent, usually based on a consumer price index or annual minimum increases. Minimum lease payments for future years are as follows:

2018	\$9,697
2019	7,671
2020	5,996
2021	4,948
2022	3,961
Thereafter	5,851
Total	\$38,124

Rent expense for the years ended December 31, 2017, 2016 and 2015 was \$11.4 million, \$11.6 million and \$9.3 million, respectively. Deferred rent accruals at December 31, 2017 totaled \$2.3 million, of which \$2.0 million was current. Deferred rent accruals at December 31, 2016 totaled \$2.4 million, of which \$2.0 million was current.

## Research and Development Funding and Technology Licenses

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. The Company has also licensed technology, for which it is required to pay royalties upon future sales, subject to certain annual minimums.

## Other Commitments

In the normal course of business, the Company enters into various firm purchase commitments primarily related to active pharmaceutical ingredients and certain inventory related items. As of December 31, 2017, these commitments

for the next five years were approximately \$52.8 million. The amounts primarily related to active pharmaceutical ingredients represent minimum purchase requirements and post marketing commitments related to the Company's approved products.

#### Contingencies

From time to time the Company is involved in legal actions arising in the normal course of its business. The most significant of these actions are described below.

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 and 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.

#### Paragraph IV Notices

The Company received a paragraph IV notice letter, dated December 23, 2016, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL), notifying the Company that DRL had



BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

filed an abbreviated new drug application (ANDA) seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral powder prior to the expiration of the Company's patents listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). The Company filed a lawsuit alleging patent infringement against DRL. In August 2017, the Company entered into a settlement agreement with DRL (the DRL Powder Settlement Agreement) that resolved the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral powder. Under the terms of the DRL Powder Settlement Agreement, the Company granted DRL a non-exclusive license to its Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride in oral powder form in 100 mg and 500 mg packet formulations in the U.S. for the indications approved for Kuvan beginning on October 1, 2020, or earlier under certain circumstances.

The Company also received two separate paragraph IV notice letters, dated January 14, 2016 and January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying the Company that Par had filed an ANDA seeking approval of proposed generic versions of Kuvan 100 mg oral powder and Kuvan 100 mg oral tablets, respectively, prior to the expiration of the Company's patents listed in the FDA's Orange Book. The Company filed two lawsuits alleging patent infringement against Par (the lawsuit against Par pertaining to the proposed generic version of Kuvan 100 mg oral tablets was filed together with Merck & Cie), and the two Par cases were consolidated. In April 2017, the Company and Merck & Cie entered into a settlement agreement with Par (the Par Settlement Agreement) that resolved both cases against Par. Under the Par Settlement Agreement, the Company granted Par a non-exclusive license to its Kuvan-related patents to allow Par to market a generic version of sapropterin dihydrochloride in 100 mg oral tablets and oral powder in 100 mg and 500 mg packet formulations in the U.S. for the indications approved for Kuvan beginning on: April 1, 2021 if Par is not entitled to the statutory 180-day first filer exclusivity period; October 1, 2020 if Par is entitled to the statutory 180-day first filer exclusivity period; or earlier under certain circumstances.

The Company also received a paragraph IV notice letter, dated October 3, 2014, from DRL notifying the Company that DRL had filed an ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral tablets prior to the expiration of the Company's patents listed in the FDA's Orange Book. The Company, together with Merck & Cie, filed a lawsuit alleging patent infringement against DRL. In September 2015, the Company and Merck & Cie entered into a settlement agreement with DRL (the DRL Tablet Settlement Agreement) that resolved the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral tablets. Under the terms of the DRL Tablet Settlement Agreement, the Company granted DRL a non-exclusive license to its Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100 mg oral tablets in the U.S. for the indications approved for Kuvan beginning on October 1, 2020, or earlier under certain circumstances.

#### Contingent Payments

As of December 31, 2017, the Company is also subject to contingent payments totaling approximately \$604.6 million upon achievement of development and regulatory activities and commercial sales and licensing milestones if they occur before certain dates in the future. Of this amount, \$222.0 million (or €185 million based on the exchange rate of 1.20 USD per Euro in effect on December 31, 2017) relates to the Merck PKU Business acquisition and \$53.4 million relates to programs that are no longer being developed.

As of December 31, 2017, the Company has recorded \$189.0 million of contingent acquisition consideration payable on its Consolidated Balance Sheets in Short-term and Long-term Contingent Acquisition Consideration Payable, of

which \$53.6 million is expected to be paid in the next twelve months.

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