

BIOMARIN PHARMACEUTICAL INC  
Form 10-K  
February 29, 2016  
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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, 2015

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

Large accelerated filer

Accelerated filer

Non-accelerated filer  (Do not check if a smaller reporting company) Smaller reporting company

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, 2015 was \$15.2 billion, based on the closing price reported for such date on the NASDAQ Global Select Market.

As of February 12, 2016, the registrant had 161,590,680 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 our annual meeting of stockholders to be held June 6, 2016, are incorporated by reference into Part III.

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BIOMARIN PHARMACEUTICAL INC.

2015 FORM 10-K ANNUAL REPORT

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Kyndrisa™ is our trademark. BioMarin®, Vimizim®, Naglazyme®, Kuvan® and Firdapse® are our registered trademarks. Aldurazyme® is a registered trademark of BioMarin/Genzyme LLC. All other brand names and service marks, trademarks and other trade names appearing in this report are the property of their respective owners.

Part I

FORWARD LOOKING STATEMENTS

This Annual Report on Form 10-K contains “forward-looking statements” as defined under federal securities laws. Many of these statements can be identified by the use of terminology such as “believes,” “expects,” “anticipates,” “plans,” “may,” “will,” “projects,” “continues,” “estimates,” “potential,” “opportunity” and similar expressions. These forward-looking statements may be found in “Risk Factors,” “Business,” “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and other sections of this Annual Report on Form 10-K. 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 “Risk Factors,” as well as those discussed in “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and elsewhere in this Annual Report on Form 10-K. You should carefully consider that information before you make an investment decision.

You should not place undue reliance on these statements, which speak only as of the date that they were made. These cautionary statements should be considered in connection with any written or oral forward-looking statements that we may make in the future. 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 to reflect the occurrence of unanticipated events.

The following discussion of our financial condition and results of operations should be read in conjunction with our Consolidated Financial Statements and the notes thereto appearing elsewhere in this Annual Report on Form 10-K. In addition to the other information in this Annual Report on Form 10-K, investors should carefully consider the following discussion and the information under “Risk Factors” when evaluating us and our business.

Item 1. Business

Overview

BioMarin Pharmaceutical Inc. (BioMarin, we, us or our) develops and commercializes innovative pharmaceuticals for serious 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 product portfolio consists of five approved products and multiple clinical and pre-clinical product candidates. Our approved products are Vimizim (elosulfase alpha), Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (laronidase) and Firdapse (amifampridine phosphate).

Vimizim received marketing approval in the United States (the U.S.) in February 2014, in the European Union (the EU) in April 2014 and subsequently in other countries. Naglazyme received marketing approval in the U.S. in May 2005, in the EU in January 2006 and subsequently in other countries. Kuvan was granted marketing approval in the U.S. and the EU in December 2007 and December 2008, respectively. Aldurazyme, which was developed in collaboration with Genzyme Corporation (Genzyme), was approved in 2003 for marketing in the U.S. and the EU, and subsequently in other countries. In December 2009, Firdapse received marketing approval in the EU.

We are conducting clinical trials on several investigational product candidates for the treatment of various diseases including: Kyndrisa (drisapersen), an exon-51 skipping compound for the potential treatment of Duchenne muscular dystrophy (DMD) amenable to exon 51 skipping; pegvaliase (formerly referred to as PEG PAL), an enzyme substitution therapy for the treatment of phenylketonuria (PKU); reveglucosidase alfa (formerly referred to as BMN 701), an enzyme replacement therapy for Pompe disease, a glycogen storage disorder; vosoritide (formerly referred to as BMN 111), a peptide therapeutic for the treatment of achondroplasia, the leading cause of dwarfism; BMN 044, BMN 045 and BMN 053 for the treatment of DMD (exons 44, 45 and 53); cerliponase alfa (formerly referred to as

BMN 190) for the treatment of late infantile neuronal ceroid lipofuscinosis (CLN2), a lysosomal storage disorder primarily affecting the brain; and BMN 270, an AAV VIII vector and Factor VIII gene therapy drug development candidate, for the treatment of hemophilia A. We are conducting or planning to conduct preclinical development of several other product candidates for genetic and other metabolic diseases, including a novel fusion of alpha-N-acetylglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2) (formerly referred to as BMN 250), for the treatment of Sanfilippo B syndrome, or mucopolysaccharidosis type IIIB (MPS IIIB). We expect to initiate a Phase 1 study for NAGLU in the first half of 2016.

## Recent Developments

### Regulatory Review of Kyndrisa

In January 2016 the U.S. Food and Drug Administration (the FDA) issued a complete response letter to our New Drug Application (NDA) for Kyndrisa for the treatment of DMD amenable to exon 51 skipping. The FDA issues a complete response letter to indicate that the review cycle for an application is complete and that the application is not ready for approval in its present form. The FDA concluded that the standard of substantial evidence of effectiveness for Kyndrisa had not been met. We are reviewing the complete response letter and will work with the FDA to determine the appropriate next steps regarding this application. A Marketing Authorization Application (MAA) for Kyndrisa for the treatment of DMD amenable to exon 51 skipping remains under review in the EU. We anticipate that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) will provide an opinion for our MAA for Kyndrisa in the second quarter of 2016. If the CHMP opinion is positive, the MAA will be referred to the European Commission (EC). The EC is expected to render a final decision for Kyndrisa in the second half of 2016. If the MAA is approved by the EC, we would receive marketing authorization for Kyndrisa in the EU. Furthermore, if we receive approval in the EU, we plan to roll out our international registration strategy for Kyndrisa using the EU approval as the basis of multiple international applications.

### Acquisition of Rights to PKU Franchise from Ares Trading S.A. (Merck Serono)

On October 1, 2015 we 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 us and Merck Serono, including the license to Kuvan granted in the License Agreement from us to Merck Serono. Also on October 1, 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, in December 2015 we paid a deposit on this transaction totaling \$371.8 million and we may 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 may also pay Merck Serono up to a maximum of €125.0 million, in cash, if future development milestones are met.

We and Merck Serono have no further rights or obligations under the License Agreement with respect to pegvaliase. The License Agreement will continue in effect in order to complete the transfer of certain assets related to Kuvan, the majority of which occurred in January 2016. Accordingly, we continue to rely on Merck Serono to provide critical transition services for sales and distribution of Kuvan until marketing authorizations can be transferred in approximately 13 remaining countries, but in no event later than December 31, 2016.

### Sale of Talazoparib to Medivation, Inc.

On October 6, 2015 we completed the sale of talazoparib (formerly referred to as BMN 673), an orally available poly-ADP ribose polymerase (PARP) inhibitor for the treatment of patients with certain cancers, to Medivation, Inc. (Medivation), under which Medivation acquired the worldwide rights to talazoparib in exchange for an upfront payment of \$410.0 million and up to an additional \$160.0 million upon the achievement of regulatory and sales-based milestones, as well as mid-single digit percentage royalties for talazoparib.





Paragraph IV Notice Letter from Dr. Reddy's Laboratories and Par Pharmaceutical, Inc.

We received a paragraph IV notice letter, dated October 3, 2014, 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 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). Additionally, we received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying us that Par has 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. Together with Merck & Cie, on March 6, 2015 we filed lawsuits against both DRL and Par in the U.S. District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan tablets and seeking an injunction to prevent Par from introducing a generic version of Kuvan tablets that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of each ANDA in accordance with the Hatch-Waxman Act, which expires in July 2017. In response, DRL and Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

In September 2015, we entered into a settlement agreement with DRL that resolved the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral tablets. Under the terms of the settlement agreement, we have granted DRL a non-exclusive license to our Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100 mg tablets in the U.S. for the indications approved for Kuvan beginning at a confidential date in the future, but which is more than five years from the settlement date, or earlier under certain circumstances.

The settlement with DRL does not affect the case against Par, and the litigation against Par is still pending. The parties submitted opening claim construction briefs on January 14, 2016, and responsive claim construction briefs are due on March 4, 2016. The Court has not yet set a date for trial in the litigation against Par.

We also received a paragraph IV notice letter, dated January 14, 2016, from Par, notifying us that Par has filed a separate ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral powder prior to the expiration of our patents listed in the FDA's Orange Book. On February 22, 2016, we filed a lawsuit against Par in the U.S. District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan powder and seeking an injunction to prevent Par from introducing a generic version of Kuvan powder that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2018.

Acquisition of Prosensa Holding N.V.

On January 29, 2015, we completed the acquisition of Prosensa Holding N.V. (Prosensa), a public limited liability company organized under the laws of the Netherlands, for a total purchase price of \$751.5 million. In connection with the acquisition of Prosensa, we recognized transaction costs of \$9.7 million, of which \$7.0 million and \$2.7 million, respectively, was recognized in the years ended December 31, 2015 and 2014.

Prosensa was an innovative biotechnology company engaged in the discovery and development of ribonucleic acid (RNA)-modulating therapeutics for the treatment of genetic disorders. Prosensa's primary focus was on rare neuromuscular and neurodegenerative disorders with a large unmet medical need, including subsets of patients with DMD, myotonic dystrophy and Huntington's disease. Prosensa's clinical portfolio of RNA-based product candidates was focused on the treatment of DMD. Each of Prosensa's DMD compounds has been granted orphan drug status in the U.S. and the EU, including Prosensa's lead product, Kyndrisa.

In connection with our acquisition of Prosensa, we made cash payments totaling \$680.1 million, which consisted of \$620.7 million for approximately 96.8% of Prosensa's ordinary shares (the Prosensa Shares), \$38.6 million for the options that vested pursuant to our tender offer for the Prosensa Shares and \$20.8 million to the remaining Prosensa shareholders that did not tender their shares under the tender offer. Additionally, for each Prosensa Share, we issued one non-transferable contingent value right (CVR), which represents the contractual right to receive a cash payment of up to \$4.14 per Prosensa Share, or an aggregate of approximately \$160.0 million (undiscounted), upon the achievement of certain product approval milestones. The fair value of the CVRs and acquired in-process research and development (IPR&D) on the acquisition date was \$71.4 million and \$772.8 million, respectively. The acquisition date fair value of the CVRs and IPR&D was estimated by applying a probability-based income approach utilizing an appropriate discount rate. Key assumptions include a discount rate and various probability factors. See Note 13 to the accompanying Condensed Consolidated Financial Statements for additional discussion regarding fair value measurements of the CVRs, which is included in contingent acquisition consideration payable.

Summary of Commercial Products and Major Development Programs

A summary of our various commercial products and major development programs, including key metrics as of December 31, 2015, is provided below:

Commercial Products	Indication	U.S. Orphan Drug Exclusivity Expiration	EU Orphan Drug Exclusivity Expiration	2015 Total Net Product Revenues (in millions)	2015 Research & Development Expense (in millions)
Vimizim	MPS IV A <sup>(1)</sup>	2021	2024	\$ 228.1	\$ 45.7
Naglazyme	MPS VI <sup>(2)</sup>	Expired	Expired	\$ 303.1	\$ 12.8
Kuvan	PKU <sup>(3)</sup>	Expired	2020 <sup>(4)</sup>	\$ 239.3	\$ 15.4
Aldurazyme <sup>(5)</sup>	MPS I <sup>(6)</sup>	Expired	Expired	\$ 98.0	\$ 3.6
Firdapse	LEMS <sup>(7)</sup>	NA <sup>(8)</sup>	2019	\$ 16.0	\$ 4.5

Major Products in Development	Target Indication	U.S. Orphan Designation	EU Orphan Designation	Stage	2015 Research & Development Expense (in millions)
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Kyndrisa	DMD <sup>(9)</sup>	Yes <sup>(11)</sup>	Yes	Clinical Phase 3	\$ 49.9
Pegvaliase	PKU <sup>(10)</sup>	Yes	Yes	Clinical Phase 3	\$ 74.0
Reveglucosidase alfa	Pompe	Yes	Yes	Clinical Phase 2/3	\$ 58.6
Vosoritide	Achondroplasia	Yes	Yes	Clinical Phase 2	\$ 49.4
Cerliponase alfa	CLN2	Yes	Yes	Clinical Phase 1/2	\$ 39.9

(1) Mucopolysaccharidosis IV Type A, or MPS IVA

(2) Mucopolysaccharidosis VI, or MPS VI

(3) Phenylketonuria, or PKU

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- (4) 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, Merck Serono marketed Kuvan in the EU until January 1, 2016 and continues to market Kuvan in certain EU countries where the regulatory approvals have not yet been transferred to us. See “Commercial Products—Kuvan” below for further discussion.
  - (5) 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. See “Commercial Products—Aldurazyme” below for further discussion.
  - (6) Mucopolysaccharidosis I, or MPS I
  - (7) Lambert Eaton Myasthenic Syndrome, or LEMS
  - (8) 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.
  - (9) DMD amenable to exon 51 skipping
  - (10) PKU, for patients 18 years of age and older
  - (11) In January 2016 the FDA issued a complete response letter to our NDA for Kyndrisa. The FDA concluded that the standard of substantial evidence of effectiveness for Kyndrisa had not been met. See “Products in Clinical Development—Kyndrisa” below for further discussion.
- See “Patents and Proprietary Rights” below for additional information on our market protection.

## Commercial Products

### 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 approximately 1,800 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 countries. We market Vimizim in the U.S., the EU, and other areas using our own existing sales force and commercial organization. Additionally, we use local distributors in several other regions to help us pursue registration and/or market Vimizim on a named patient basis. Vimizim net product revenues for the years ended December 31, 2015, 2014 and 2013 totaled \$228.1 million, \$77.3 million and \$0.1 million, respectively.

### Naglazyme

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

Naglazyme was granted marketing approval in the U.S. in May 2005, in the EU in January 2006, and subsequently in other countries. We market Naglazyme in the U.S., the EU, Canada, Latin America, Turkey and other areas using our own sales force and commercial organization. Additionally, we use local distributors in several other regions to help us pursue registration and/or market Naglazyme on a named patient basis. Naglazyme net product revenues for the years ended December 31, 2015, 2014 and 2013 totaled \$303.1 million, \$334.4 million and \$271.2 million, respectively.

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## 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 phenylketonuria (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 phenylalanine (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 has been demonstrated to reduce blood Phe levels by 30% in approximately 30% of patients.

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. This new dosage form is expected to have increasing appeal for young patients in the one to seven year age range. We commenced the commercial launch of this new form of Kuvan on February 28, 2014.

Kuvan was granted marketing approval for the treatment of PKU in the U.S. in December 2007 and in the EU in December 2008. Using our own sales force and commercial organization, we market Kuvan in the U.S. and Canada (and effective as of January 1, 2016, in the rest of the world, except for Japan and certain countries in which we have not yet completed the transfer of certain regulatory approvals from Merck Serono, as described below). Kuvan has been granted orphan drug status in the U.S., which confers market exclusivity in the U.S. for the treatment of PKU, which expired in June 2015. In addition, 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. We expect that our patents will provide market exclusivity beyond the expiration of orphan status. Kuvan net product revenues for the years ended December 31, 2015, 2014 and 2013 totaled \$239.3 million, \$203.0 million, and \$167.4 million, respectively.

In 2005, we entered into the License Agreement with Merck Serono for the further development and commercialization of Kuvan and any other product containing 6R-BH<sub>4</sub>, and pegvaliase for PKU. Through the License Agreement, as amended in 2007, Merck Serono acquired exclusive rights to market these products in all territories outside the U.S., Canada and Japan, and we retained exclusive rights to market these products in the U.S. and to market Kuvan in Canada and pegvaliase in Japan. Under the License Agreement, Merck Serono marketed Kuvan in the EU and several other countries outside the U.S., Canada and Japan. Under the License Agreement, we were entitled to receive royalties, on a country-by-country basis, until the later of the expiration of patent rights licensed to Merck Serono or ten years after the first commercial sale of the licensed product in such country. Under this arrangement, we also sold Kuvan to Merck Serono at or near 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 EU is included as a component of net product revenues in the period earned. During 2015, 2014 and 2013 we earned \$2.0 million, \$2.2 million and \$2.0 million, respectively, in net royalties on net sales of \$56.5 million, \$55.5 million and \$51.0 million of Kuvan by Merck Serono, respectively. We recorded collaborative agreement revenue associated with shared Kuvan development costs in the amounts of \$0.8 million, \$0.9 million, and \$1.0 million in 2015, 2014 and 2013, respectively.

In the fourth quarter of 2015, we entered into the A&R Kuvan Agreement to terminate 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 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, in December 2015, we paid a deposit on this transaction totaling \$371.8 million and we may 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 may pay Merck Serono up to a maximum of €125.0 million, in cash, if future development milestones are met.

We and Merck Serono have no further rights or obligations under the License Agreement with respect to pegvaliase. The License Agreement will continue in effect in order to complete the transfer of certain assets related to Kuvan, the majority of which occurred in January 2016. Accordingly, we continue to rely on Merck Serono to provide critical transition services for sales and distribution of Kuvan until marketing authorizations can be transferred in approximately 13 remaining countries, but in no event later than December 31, 2016.

#### Aldurazyme

Aldurazyme was approved for marketing in the U.S. in April 2003, in the EU in June 2003 and subsequently in other countries for patients with mucopolysaccharidosis I (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 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. Genzyme records sales of Aldurazyme and is required to pay us, on a quarterly basis, a 39.5% to 50% royalty on worldwide net product sales, depending on sales volume. We recognize a portion of this royalty as product transfer revenue when product is released to Genzyme and all of our obligations have been fulfilled. Genzyme's return rights for Aldurazyme are limited to defective product. 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 calculated royalty 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, 2015, 2014 and 2013 totaled \$98.0 million, \$105.6 million and \$83.6 million, respectively. The net product revenues for each of the years ended December 31, 2015, 2014 and 2013 include \$95.8 million, \$97.0 million and \$88.5 million, respectively, of royalty revenue on net Aldurazyme sales by Genzyme. Net sales of Aldurazyme by Genzyme totaled \$217.8 million, \$228.8 million and \$212.4 million for the years ended December 31, 2015, 2014 and 2013, respectively. Aldurazyme net product revenues included incremental Aldurazyme net product transfer revenue of \$2.2 million and \$8.6 million in the years ended December 31, 2015 and 2014, respectively, and previously recognized product transfer revenue of \$4.9 million in the year ended December 31, 2013. Incremental product transfer revenue that we previously recognized reflects incremental shipments of Aldurazyme to Genzyme to meet future product demand. In the future, to the extent that Genzyme Aldurazyme inventory quantities on hand remain consistent, we expect that our total Aldurazyme revenues will approximate the 39.5% to 50% royalties on net product sales by Genzyme.

#### Firdapse

Firdapse is a form of 3,4-diaminopyridine (amifampridine phosphate or 3,4-DAP) for the treatment of Lambert Myasthenic Syndrome (LEMS). Firdapse was originally developed by AGEPS, the pharmaceutical unit of the Paris Public Hospital Authority. Firdapse was granted marketing approval in the EU in December 2009. In addition, Firdapse has been granted orphan drug status in the EU, which confers ten years of market exclusivity in the EU. We launched Firdapse on a country-by-country basis in Europe in 2010. Firdapse net product revenues for the years ended



December 31, 2015, 2014 and 2013 totaled \$16.0 million, \$18.1 million and \$16.1 million, respectively. In October 2012, we licensed to Catalyst Pharmaceutical Partners, Inc. (Catalyst) the North American rights to develop and market Firdapse. In exchange for the North American rights to Firdapse, we may receive royalties of 7% to 10% on net product sales of Firdapse in North America. For the years ended December 31, 2015, 2014 and 2013 we recognized collaborative revenue of \$0.3 million, \$0.7 million and \$2.9 million, respectively, related to our agreement with Catalyst.

LEMS is a rare autoimmune disease with the primary symptoms of muscle weakness. Muscle weakness in LEMS is caused by autoantibodies to voltage gated calcium channels leading to a reduction in the amount of acetylcholine released from nerve terminals. The prevalence of LEMS is estimated at four to ten per million, or approximately 2,000 to 5,000 patients in the EU and 1,200 to 3,100 patients in the U.S. Approximately 50% of LEMS patients diagnosed have small cell lung cancer. Patients with LEMS typically present with fatigue, muscle pain and stiffness. The weakness is generally more marked in the proximal muscles particularly of the legs and trunk. Other problems include reduced reflexes, drooping of the eyelids, facial weakness and problems with swallowing. Patients often report a dry mouth, impotence, constipation and feelings of light headedness on standing. On occasion, these problems

can be life threatening when the weakness involves respiratory muscles. A diagnosis of LEMS is generally made on the basis of clinical symptoms, electromyography testing and the presence of auto antibodies against voltage gated calcium channels. Currently approved treatments of LEMS can consist of strategies directed at the underlying malignancy, if one is present. Therapy of small cell lung cancer is limited and outcomes are generally poor. Immunosuppressive agents have been tried but success is limited by toxicity and difficulty administering the regimens. A mainstay of therapy has been 3, 4-DAP, but its use in practice has been limited by the drug's availability.

## Products in Clinical Development

### Kyndrisa

We acquired Kyndrisa, Prosensa's lead candidate for a subset of DMD amenable to exon 51 skipping, on January 15, 2015. DMD is a rare genetic disease, affecting approximately 1 in 3,500 boys globally, and is invariably fatal. There is currently no approved disease-modifying therapy for DMD. The progressive muscle-wasting that characterizes DMD is caused by inadequate production of dystrophin, a protein necessary for muscle function, as a result of mutations in the dystrophin gene. The different mutations, which are mostly deletions of one or more exons found in the dystrophin gene, result in distinct sub-populations of DMD patients. Kyndrisa aims to address a specific mutation in the dystrophin gene that represents approximately 13% of all DMD patients, or approximately 10,000 patients worldwide.

Two of the Phase 2 trials of Kyndrisa investigated change in a six minute walk test (6MWT) as compared to a placebo. The first Phase 2 trial showed a mean 32-meter improvement for the Kyndrisa group compared to a 4-meter decline in the placebo group ( $p=0.014$ ). The second Phase 2 trial showed a mean 16-meter improvement for the Kyndrisa group compared to a mean 11-meter decline in the placebo group ( $p=0.069$ ). When the results of these trials were combined in a post hoc analysis, the trials showed a mean 20-meter improvement for the combined Kyndrisa group compared to a mean 11-meter decline in the placebo group ( $p=.003$ ). In the Phase 3 trial, the Kyndrisa group experienced a mean 43-meter decline compared to a mean 53-meter decline in the placebo group, although the result did not reach statistical significance ( $p=0.415$ ). In the open label extension study of 12 patients, which included patients who lost ambulation, patients receiving 6 mg/kg of Kyndrisa experienced a mean 25-meter decline on the 6MWT at 177 weeks as compared to an expected 115-meter decline at 156 weeks, based on the natural history database.

Based on this data and the results of the clinical trials, in June 2014, Prosensa announced that it would pursue an NDA filing for Kyndrisa with the FDA under an accelerated approval pathway based on existing data and in October 2014 Prosensa submitted the first module for an NDA regulatory filing for Kyndrisa to the FDA. Kyndrisa was granted Fast Track status and breakthrough therapy designation from the FDA, making it eligible for a rolling review of the NDA. Breakthrough therapy designation is a process designed to expedite the development and review of drugs that may demonstrate substantial improvement over available therapy. In the second quarter of 2015 we completed the submission of the NDA to the FDA for Kyndrisa and an MAA with the EMA for Kyndrisa.

In January 2016, the FDA issued a complete response letter to our NDA for Kyndrisa for the treatment of DMD amenable to exon 51 skipping. The FDA issues a complete response letter to indicate that the review cycle for an application is complete and that the application is not ready for approval in its present form. The FDA concluded that the standard of substantial evidence of effectiveness for Kyndrisa had not been met. We are reviewing the complete response Letter and will work with the FDA to determine the appropriate next steps regarding this application. An MAA for Kyndrisa for the treatment of DMD amenable to exon 51 skipping remains under review in the EU. We anticipate that the CHMP of the EMA will provide an opinion for our MAA for Kyndrisa in the second quarter of 2016. If the CHMP opinion is positive, the MAA will be referred to the EC. The EC is expected to render a final decision for Kyndrisa in the second half of 2016. If the MAA is approved by the EC, we would receive marketing authorization for Kyndrisa in all EU Member States. Furthermore, if we receive approval in the EU, we plan to roll out our international registration strategy for Kyndrisa using the EU approval as the basis of multiple international applications.

## Pegvaliase

Pegvaliase is an investigational enzyme substitution therapy that we are developing as a subcutaneous injection for the treatment of PKU. In June 2009, we announced results from a Phase 1 open-label, single-dose, dose-escalation clinical trial of pegvaliase for PKU. Significant reductions in blood Phe levels were observed in all patients in the fifth dosing cohort of the Phase 1 trial. In addition, there were no serious immune reactions observed and mild to moderate injection-site reactions were in line with our expectations. In September 2009, we initiated a Phase 2, open-label dose finding clinical trial of pegvaliase. The primary objective of this clinical trial was to optimize the dose and schedule that produces the most favorable safety profile and Phe reduction. The secondary objectives of the clinical trial were to evaluate the safety and tolerability of multiple dose levels of pegvaliase, to evaluate the immune response to pegvaliase, and to evaluate steady-state pharmacokinetics in all patients and accumulation of pegvaliase in a subset of patients enrolled in this clinical trial. Preliminary results from this clinical trial were presented in August 2010 and showed that of the seven patients who received at least one mg/kg per week of pegvaliase for at least four weeks, six patients had achieved Phe levels below 600 micromoles per liter. Mild to moderate self-limiting injection site reactions are the most commonly reported toxicity. In April 2011, we initiated an extension of the Phase 2 study to find a shorter induction and titration dosing regimen to an efficacious

maintenance dose. A Phase 3 clinical trial of pegvaliase was initiated in June 2013. 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. The FDA has indicated that lowering Phe blood levels in adults could form the basis for an accelerated approval and, additionally, that a favorable outcome on prospectively-specified analyses of inattention in patients with baseline problems with attention would likely be required for full approval. We expect to report results from these trials in the first quarter of 2016.

#### Reveglucosidase alfa

Reveglucosidase alfa is a novel fusion protein of acid alpha glucosidase (GAA) with a peptide derived from IGF2. We acquired the reveglucosidase alfa program in August 2010 in connection with the acquisition of ZyStor Therapeutics, Inc. (ZyStor). In January 2011, we announced the initiation of a Phase 1/2 clinical trial for reveglucosidase alfa. This clinical trial was an open-label study to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamic and clinical activity of reveglucosidase alfa administered as an intravenous infusion every two weeks at doses of up to 20 mg/kg. We completed enrollment of this study in September 2012 with 22 patients between the ages of 13 and 65 years old with late-onset Pompe disease for a treatment period of 24 weeks. The primary objectives of this study were to evaluate the safety and tolerability of reveglucosidase alfa as well as determine the antibody response to reveglucosidase alfa. The secondary objectives of the study were to determine the single and multi-dose pharmacokinetics of reveglucosidase alfa and determine mobility and functional exercise capacity in patients receiving reveglucosidase alfa. Pompe disease is a lysosomal storage disorder caused by a deficiency in GAA that prevents cells from adequately degrading glycogen. This results in the storage of glycogen in lysosomes, particularly those in muscle cells, thereby damaging those cells and causing progressive muscle weakness, which in turn can result in death due to pulmonary or cardiac insufficiency.

Results from the Phase 1/2 clinical trial, released in March 2013, exceeded our prespecified requirements. The results showed that in the 20 mg/kg every other week dose cohort, three out of 16 patients, or 19%, had a greater than 75-meter improvement in six-minute walk distance (6MWD), and that there was a 14.1% relative improvement in Maximal Expiratory Pressure (MEP) and a 27.0% relative improvement in Maximal Inspiratory Pressure (MIP) from pretreatment baseline to week 24, two important measures of overall respiratory muscle function and strength. Side effects for reveglucosidase alfa were generally consistent with those seen for other enzyme replacement therapies.

In May 2014, we initiated a Phase 3 switching trial of late onset Pompe patients who had previously been treated with alglucosidase alfa. Interim results were released in January 2016, based on 24 patients who previously had been on treatment with alglucosidase alfa and were switched to reveglucosidase alfa. At week 24, the 18 patients on treatment with reveglucosidase alfa and who completed the study demonstrated respiratory muscle improvements with a mean increase of 2.2 points from baseline in percent predicted MIP and a mean increase of 3.1 points from baseline in percent predicted MEP. Patients completing the study also experienced a mean improvement of 26.1 meters in 6MWD. In the 14 patients who met eligibility at both screening and baseline and completed the study, a mean increase of 3.8 points from baseline in percent predicted MIP also was observed. The 18 patients completing the study showed a mean decrease of 3.7 points from baseline in percent predicted Forced Vital Capacity (FVC), but were considered relatively unchanged from screening at -0.7 points in percent predicted. We will present these data at an upcoming medical meeting. Based on these results, we intend to meet with regulatory authorities to determine the appropriate next steps for the program. In addition, we have improved the manufacturing process for reveglucosidase alfa, which improved process we expect will serve as our commercial manufacturing process.

#### Vosoritide

Vosoritide (formerly referred to as BMN 111) is a peptide therapeutic in development for the treatment of achondroplasia. In September 2012, we announced the results of a Phase 1 clinical trial for vosoritide. The primary objective of the Phase 1 clinical trial was to assess the safety and tolerability of single and multiple doses of vosoritide

in normal healthy adult volunteers up to the maximum tolerated dose. Vosoritide was generally well-tolerated over the range of single and repeat doses studied. Pharmacokinetic data indicated that the dose levels studied resulted in exposure levels that are expected to stimulate growth based on non-clinical findings. In January 2014, we announced the initiation of a Phase 2 clinical trial for vosoritide for the treatment of children with achondroplasia. This international clinical trial is an open-label, sequential cohort, dose-escalation study of vosoritide in children who are 5-14 years old. The primary objective of this study is to assess the safety and tolerability of daily subcutaneous doses of vosoritide administered for 6 months. The secondary objectives will include an evaluation of change in annualized growth velocity, changes in absolute growth parameters, changes in body proportions and other medically relevant and functional aspects of achondroplasia, such as sleep apnea and joint range of motion. Prior to enrolling in the Phase 2 study, all patients will have participated in a six month natural history study to determine baseline growth velocity data. We completed enrollment in the first three cohorts of this study in November 2014. In June 2015 we reported six-month data for the 26 patients in the first three cohorts of the Phase 2 study, which showed a 50% increase in mean annualized growth velocity in the 15 µg/kg/day dose group of 10 patients compared with their own pre-treatment growth velocity. Vosoritide was also well tolerated across all three dose cohorts. The Phase 2 findings support the advancement of vosoritide into pivotal development, which we are currently discussing with regulatory authorities.

## Cerliponase alfa

Cerliponase alfa is a recombinant human tripeptidyl peptidase 1 in development 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 1,200-1,600 cases exist worldwide. In September 2013, we announced the initiation of a Phase 1/2 study for cerliponase alfa. This clinical trial is an open-label, dose-escalation study in patients with CLN2. The primary objectives are to evaluate the safety and tolerability of cerliponase alfa and to evaluate effectiveness using a CLN2-specific rating scale score in comparison with natural history data after 48 weeks of treatment. Secondary objectives are to evaluate the impact of treatment on brain atrophy in comparison with CLN2 natural history after 48 weeks of treatment and to characterize pharmacokinetics and immunogenicity. This study was fully enrolled in December 2014 with 24 patients. In January 2015, we announced interim data from the study, which indicates that in all nine of the patients in the trial who have been followed for at least six months and up to 15 months, the treatment appears to show stabilization of the disease compared to the natural history based on a standardized measure of motor and language function. All patients were tolerating the therapeutic dose. We expect to announce complete results in March 2016.

## BMN 044, BMN 045, and BMN 053

We acquired BMN 044, BMN 045, and BMN 053, Prosensa's additional candidates for the treatment of subsets of DMD, on January 15, 2015.

BMN 044 (formerly referred to as PRO 044), an exon 44 skipping compound, aims to address a specific mutation in the dystrophin gene that represents approximately 6% of all DMD patients. Prosensa initiated a dose-escalation trial, assessing six doses (0.5, 1.5, 5, 8, 10 and 12 mg/kg/week) in 18 DMD patients in December 2009. Enrollment was completed in the first quarter of 2013, and an extension study has been initiated.

BMN 045 (formerly referred to as PRO 045), an exon 45 skipping compound, aims to address a specific mutation in the dystrophin gene that represents approximately 8% of all DMD patients. Prosensa commenced a Phase 1/2 study of BMN 045 in 15 DMD patients in the first quarter of 2013, which is ongoing.

BMN 053 (formerly referred to as PRO 053), an exon 53 skipping compound, aims to address a specific mutation in the dystrophin gene that represents approximately 8% of all DMD patients. Prosensa commenced a Phase 1/2 study for BMN 053 in nine DMD patients in September 2013, which is ongoing.

## BMN 270

BMN 270 is an AAV-factor VIII vector, designed to restore factor VIII plasma concentrations, essential for blood clotting in patients with hemophilia A. Hemophilia A, also called factor VIII (FVIII) deficiency or classic hemophilia, is a genetic disorder caused by missing or defective factor VIII, a clotting protein. People living with the disease 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. The gene therapy program for hemophilia A was originally licensed from University College London and St. Jude Children's Research Hospital in February 2013 and has since been developed at our facilities. In September 2015, we announced the initiation of a Phase 1/2 study of BMN 270. The Phase 1/2 study will evaluate the safety and efficacy of BMN 270 gene therapy in up to 12 patients with severe hemophilia A. The primary endpoints are to assess the safety of a single intravenous administration of a recombinant AAV, human-coagulation Factor VIII vector and to determine the change from baseline of Factor VIII expression level at 16 weeks after infusion. The kinetics, duration and magnitude of AAV-mediated Factor VIII activity in individuals with hemophilia A will be determined and correlated to an appropriate BMN 270 dose. This is a dose escalation study with the goal of observing an increase in Factor VIII levels. Secondary endpoints include assessing the impact of BMN 270 on the frequency of Factor VIII

replacement therapy, the number of bleeding episodes requiring treatment and any potential immune responses. Patients will be monitored for safety for five years.

#### Manufacturing

We manufacture Naglazyme, Aldurazyme, Vimizim, pegvaliase, vosoritide and cerliponase alfa in our production facility located in Novato, California. These facilities have demonstrated compliance with GMPs to the satisfaction of the FDA, the EC and health agencies in other countries for the commercial production of Aldurazyme, Naglazyme and Vimizim. Vialing and packaging are performed by contract manufacturers. We believe that we have ample manufacturing capacity to support commercial demand for both Naglazyme and Aldurazyme for at least the next five years.

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In August 2011, we acquired a bulk biologics manufacturing plant located in Shanbally, County of Cork, Ireland. Our manufacturing facility in Shanbally, Cork, Ireland has been approved by the Health Product Regulatory Authorities, FDA, the EC, and health agencies in other countries for the testing and release of Vimizim. We recently began manufacturing Vimizim in this facility. The addition of the Shanbally facility, once approved for bulk substance production, will enhance our business continuity and increase our operating capacity to support the anticipated commercial demand of Vimizim for the next five years. We believe that with this facility and our Novato, California facility, we have ample manufacturing capacity to support commercial demand for Vimizim for at least the next five years. Additionally, we intend to manufacture cerliponase alfa, pegvaliase and vosoritide in this facility.

Kuvan tablet and powder sachet, Firdapse, Kyndrisa, reveglucosidase alfa and BMN 270 are each manufactured on a contract basis by a third party. In general, we expect to continue to contract with outside service providers for certain manufacturing services, including drug substance, active pharmaceutical ingredient (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 GMP 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.

#### 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 Vimizim, Naglazyme and Kuvan. 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.

In the U.S., our products are marketed through our commercial teams, including sales representatives and supporting staff members, who promote Vimizim, Naglazyme and Kuvan, directly to physicians in specialties appropriate for each product. Outside of the U.S., our sales representatives and supporting staff members market Vimizim, Naglazyme, Kuvan and Firdapse. We believe that with moderate changes in 2016, the size of our sales force will be appropriate to effectively reach our target audience in markets where Vimizim, Naglazyme, Kuvan and Firdapse are directly marketed. Further growth of our current marketed products and the launch of any future products 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.

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

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## Customers

Our Vimizim, Naglazyme, Kuvan and Firdapse customers include a limited number of specialty pharmacies and end-users, such as hospitals and foreign government agencies. We also sell Vimizim and Naglazyme 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, particularly in Latin America, governments place large periodic orders for Vimizim and Naglazyme. The timing of these orders can be inconsistent and can create significant quarter to quarter variation in our revenue. During 2015, 42% of our net Vimizim, Naglazyme, Kuvan and Firdapse 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 commercial 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 approved products.

### Naglazyme, Aldurazyme and Vimizim

In the mucopolysaccharidosis field, several companies are researching treatments using small molecules, gene therapy, and other novel technologies. 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 have filed paragraph IV certifications and submitted ANDAs to produce sapropterin dihydrochloride tablets and powder. See the ANDA discussion under “The Hatch-Waxman Act” for additional information.

## Firdapse

There are no other approved drugs for the treatment of LEMS, and Firdapse is the only approved version of 3,4-DAP. In some countries, 3,4-DAP is available, as a base, through various compounding pharmacies, as a special or magistral formulation, or through investigator sponsored studies. One U.S. company has begun a clinical trial of a compounded version of 3,4-DAP to treat LEMS.

## Pipeline Products

Reveglucosidase alfa has competition from Genzyme's marketed enzyme replacement products, Myozyme (alglucosidase alfa) and Lumizyme (alglucosidase alfa), and from a third Genzyme product in development. Kyndrisa has competition from Sarepta Therapeutics, Inc.'s product eteplirsen, which like Kyndrisa remains subject to regulatory approval. Our cerliponase alfa program for the treatment of CLN2 has competition from earlier stage products, including a product candidate currently under development by Spark Therapeutics, Inc. Our other pipeline products have competition from earlier stage products, either using similar technology to our programs or different treatment strategies.

## 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.

## Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 10-K

As of January 25, 2016, the number of our worldwide issued patents now stands at 633, including 82 patents issued by the U.S. Patent and Trademark Office (the USPTO). Furthermore, our portfolio of pending patent applications totals 407 applications, including 86 pending U.S. applications.

With respect to Naglazyme, we have 52 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 107 issued patents including 14 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. We have no issued patents in the U.S. 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 39 issued patents including 14 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. 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 investigational new drug application (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 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, biologics license applications (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 current Good Manufacturing Practices (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.

#### 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. Disclosure of the results of these trials can be delayed until the new product or new indication being studied has been approved. 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.

#### 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 “Good Manufacturing Practices,” or GMPs.

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.

## Health 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 imposes a new fee on certain manufacturers and importers of branded prescription drugs (excluding orphan drugs under certain conditions). The annual fee will be 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 new law, which have varying effective dates, may also affect us and will likely increase 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 expands the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance and includes 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, which could increase the amount of the Medicaid drug rebates paid to states. Substantial new provisions affecting compliance also have been added, which may require us to modify our business practices with health care 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.

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, 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.

#### 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, or 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 the EMA approval. In many countries outside of the U.S., coverage, pricing and reimbursement approvals 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 health care 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 will also be subject to an additional rebate.

The statutory definition of AMP was amended in 2010, and there are many ambiguities in the revised provision. CMS has released the final rule pertaining to AMP and other aspects of the Medicaid drug rebate program. This final rule will be 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 January 25, 2016, we had 2,158 full-time employees, 882 of whom were in operations, 648 of whom were in research and development, 299 of whom were in sales and marketing and 329 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 2015, 2014 and 2013, see Item 7, Management's Discussion and Analysis of Financial Condition and Results of Operations—Research and Development.

## 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 Vimizim, Naglazyme, Kuvan and Firdapse 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. Net product revenues from Genzyme consist of royalties on worldwide net Aldurazyme sales and incremental product transfer revenues. Although Genzyme sells Aldurazyme worldwide, the royalties 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 following table outlines net product revenues by geographic area (in thousands):

	Years Ended December 31,		
	2015	2014	2013
Net product revenues:			
Net product revenues marketed by BioMarin:			
United States	\$343,513	\$270,094	\$193,950
Europe	179,481	139,940	116,896
Latin America	142,305	118,562	67,338
Rest of the world	121,311	104,204	76,631
Total net product revenues marketed by BioMarin	786,610	632,800	454,815
Aldurazyme net product revenues marketed by Genzyme	97,912	105,616	83,545
Total net product revenues	\$884,522	\$738,416	\$538,360

Total revenues generated outside the U.S. was \$445.8 million, \$371.0 million and \$264.2 million, in the years ended December 31, 2015, 2014 and 2013, respectively.

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

	December 31,		
	2015	2014	2013
Non-monetary long-lived assets:			
United States	\$940,512	\$827,884	\$631,706
Europe	865,233	102,451	80,638
Rest of World	2,253	1,630	988
Total long-lived assets	\$1,807,998	\$931,965	\$713,332

The increase in non-monetary long-lived assets in 2015 compared to 2014 was primarily attributable to increased IPR&D. The increase in intangible assets was primarily attributable to Kyndrisa and other exon-skipping in-process research and development assets we acquired in connection with our acquisition of Prosensa in January 2015. The increase in non-monetary long-lived assets in 2014 compared to 2013 was primarily attributable to increases in property, plant and equipment and deferred tax assets.

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 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 drugs, or if approval is delayed, we will be unable to generate revenue from the sale of these products, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will be increased.

We must obtain and maintain regulatory approval to market and sell our drug products in the U.S. and in jurisdictions outside of the U.S. In the U.S., we must obtain FDA approval for each drug that we intend to commercialize. The FDA approval process is typically lengthy and expensive, and approval is never certain. For example, in January 2016, the FDA issued a complete response letter to our NDA for Kyndrisa for the treatment of DMD amenable to exon 51 skipping in which the FDA concluded that the standard of substantial evidence of Kyndrisa's effectiveness had not been met. Products distributed abroad are also subject to government regulation by international regulatory authorities. The approval process in the EU and other countries can also be lengthy and expensive and regulatory approval is also never certain. An MAA for Kyndrisa for the treatment of DMD amenable to exon 51 skipping remains under review in the EU. We anticipate that the CHMP of the EMA will provide an opinion for our MAA for Kyndrisa in the second quarter of 2016. If the CHMP opinion is positive, the MAA will be referred to the EC. The EC is expected to render a final decision for Kyndrisa in the second half of 2016. If the MAA is not approved by the EC, we will not receive marketing authorization for Kyndrisa in the EU and will be unable to sell Kyndrisa in the EU or roll out our international registration strategy for Kyndrisa using the EU approval as the basis of multiple international applications.

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 drug products. 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, including cerliponase alfa, 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 separate 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 our product candidates, we engage in discussions with the FDA and comparable international regulatory authorities regarding the regulatory requirements for our development programs. To the extent appropriate, we accommodate the requests of the regulatory authorities. However, we are often unable to determine the outcome of such deliberations until they are final. 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 approved 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.

Naglazyme, Aldurazyme, Kuvan and Vimizim have received regulatory approval to be commercially marketed and sold in the U.S., the EU and other countries. Firdapse has received regulatory approval to be commercially marketed only 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. We also are subject to periodic unannounced inspections by the FDA to monitor and ensure compliance with cGMP and other regulations.

In addition, the FDA often requires post-marketing testing and surveillance to monitor the effects of approved 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 such 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 drug products is limited, orphan drug designation is especially important for our products that are eligible for orphan drug designation. For eligible drugs, 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 drug 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 biological products approved through an abbreviated regulatory pathway.

Our Naglazyme, Aldurazyme 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 biologics license application (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 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 data exclusivity for reference products, which protects the data in the original BLA by prohibiting sponsors of biosimilars from gaining FDA approval based in part on reference to data in the original BLA. Aldurazyme's data exclusivity under the BPCIA expired in 2015, Naglazyme's data exclusivity under the BPCIA expires in 2017, and Vimizim's data 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 regulatory approval process we must conduct, at our own expense, preclinical studies in the laboratory 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, the number and size of clinical trials required for approval increase based on the expected patient population that may be treated with a drug. We may need to perform multiple preclinical studies using various doses and formulations before we can begin clinical trials, which could result in delays in our ability to market any of our product candidates. 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 drug products 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 have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Our future clinical trial results for any of our product candidates may not be successful.

Adverse or inconclusive clinical results would stop us from filing for 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;
- longer treatment time required to demonstrate efficacy;
- lack of sufficient supplies of the product candidate;
- adverse medical events or side effects in treated patients;
- lack of effectiveness of the product candidate being tested; and
- regulatory requests for additional clinical trials or pre-clinical studies.

Typically, if a drug product is intended to treat a chronic disease, as is the case with some of our product candidates, safety and efficacy data must be gathered over an extended period of time, which can range from nine months to three years or more. We also rely on independent third-party CROs to perform most of our clinical studies and many 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, or if there is any dispute or disruption in our relationship with our CROs, our clinical trials may be delayed. Moreover, in our regulatory submissions, we rely on the quality and validity of the clinical work performed by third-party CROs. If any of our CROs' processes, methodologies or results were determined to be not conducted in accordance with current good clinical practices, invalid or inadequate, our own clinical data and results and related regulatory approvals could adversely be impacted.

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 such as 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 Vimizim, Naglazyme, Kuvan and Firdapse, the successful continued commercialization of Aldurazyme by Genzyme, the receipt of regulatory approval of our product candidates, our ability to successfully manufacture and market any approved drugs, 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.

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, 2015, we had cash, cash equivalents and short and long-term investments totaling \$1,018.3 million and long-term debt obligations of \$781.4 million (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 paid a deposit on this transaction totaling \$371.8 million, in December 2015, 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 January 2015, we paid \$620.7 million for approximately 35 million ordinary shares (the Prosensa Shares) of Prosensa, representing approximately 96.8% of all outstanding Prosensa Shares, and \$38.6 million for the options that vested pursuant to the definitive purchase agreement. In February 2015, we completed the Prosensa asset transfer and paid \$20.8 million to the remaining Prosensa shareholders. We (through our indirect wholly-owned subsidiaries) funded the acquisition with our available cash balances. We expect to pay up to an additional \$80.0 million if certain development milestones are attained. 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. We will need cash to not only repay the principal amount of our 0.75% senior subordinated convertible notes due 2018 (the 2018 Notes) and 1.50% senior subordinated convertible notes due in 2020 (the 2020 Notes and, together with the 2018 Notes, the Notes) but also the ongoing interest due on the Notes during their term. We will require additional financing to fund the repayment of our Notes, future milestone payments and our future operations, including the commercialization of our approved drugs and drug 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 Vimizim, Naglazyme, Kuvan and Firdapse;
  - Genzyme's ability to continue to successfully commercialize Aldurazyme;
- 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 time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities;
- the progress of research programs carried out by us;
- our possible achievement of milestones identified in our purchase agreements with the former stockholders of LEAD Therapeutics, Inc., ZyStor, Huxley Pharmaceuticals, Inc., and Zacharon Pharmaceuticals Inc., under the non-transferable CVRs issued in connection with the acquisition of Prosensa that trigger related milestone payments and under 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; 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.

In March 2014, we completed an offering of 1,500,000 shares of our common stock at a price of \$78.45 per share and received net proceeds of \$117.5 million. In January 2015, we completed an offering of 9,775,000 shares of our common stock at a price of \$93.25 per share and received net proceeds of approximately \$888.3 million. 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, 2015, we had \$781.4 million (undiscounted) principal amount of indebtedness, including \$375.0 million (undiscounted) of indebtedness under the 2018 Notes and \$375.0 million (undiscounted) principal amount of



indebtedness under the 2020 Notes. Our indebtedness may:

- limit our ability to borrow additional funds for working capital, capital expenditures, acquisitions or other general business purposes;

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

Our indebtedness consists primarily of Notes, which, if not converted, will be required to be repaid in cash at maturity in 2018 and 2020. In addition, in the event the conditional conversion feature of the Notes is triggered, holders of Notes will be entitled to convert the Notes at any time during specified periods at their option. We intend to settle the principal amount of our conversion obligation in cash, which could adversely affect our liquidity. Even if holders do not elect to convert their Notes, we could be required under applicable accounting rules to reclassify all or a portion of the outstanding principal of the Notes as a current rather than long-term liability, which would result in a material reduction of our net working capital. Moreover, if we are unable to refinance the Notes, we must repay the Notes. 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.

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 inspection by the FDA and international regulatory authorities, before and after product approval. Our manufacturing facility in the U.S. has been approved by the FDA, the European Commission (the EC), and health agencies in other countries for the manufacture of Aldurazyme, 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. In addition, our third-party manufacturers' facilities involved with the manufacture of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse 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 Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse 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 drug 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 drug 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 may require us to complete clinical trials to receive regulatory approval of any manufacturing improvements.

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 Naglazyme, Aldurazyme 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 Kuvan, Firdapse and Kyndrisa, if those manufacturers are unwilling or unable to fulfill their contractual obligations, we may be unable to meet demand for Kuvan and Firdapse 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 Kuvan, Firdapse and Kyndrisa. We also rely on third-parties for portions of the manufacture of Naglazyme, Aldurazyme 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 may incur significant costs in complying with these laws and regulations.

If we are unable to effectively address manufacturing issues, 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 Naglazyme, Aldurazyme 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 Naglazyme, Aldurazyme and Vimizim or our third-party manufacturers' ability to manufacture Kuvan or Firdapse.

Our Galli Drive facility located in Novato, California is currently our only manufacturing facility for Naglazyme and Aldurazyme and is one of two manufacturing facilities for 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, 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 Naglazyme, Aldurazyme and Vimizim, or to have Kuvan or Firdapse 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.

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

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

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

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

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

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 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 for genetic diseases, 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 drug products by third-party payers, the sales of our drugs 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 health care 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 may exceed 12 months. Even after a price is negotiated, countries frequently request or require adjustments 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. 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. 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) 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 health care has led to increased pressure on the health care 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 health care reforms and cost reductions. A number of federal and state proposals to control the cost of health care, 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 health care 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 Naglazyme. 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 drug 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 health care reform could increase our costs, and would 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 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. 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, included a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or “donut hole,” and imposed a new fee on certain manufacturers and importers of branded prescription drugs (excluding orphan drugs under certain conditions). The law also revised the definition of “average manufacturer price” for reporting purposes, which could increase the amount of the Medicaid drug rebates paid to states, and created a new Patient-Centered Outcomes Research Institute to oversee clinical effectiveness research.

In addition, other legislative changes have been adopted since the PPACA was enacted. These changes include aggregate reductions in Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, following passage of the Bipartisan Budget Act of 2015, will remain in effect through 2025 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result 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. Further, there have been judicial and Congressional challenges to certain aspects of the PPACA, and we expect there will be additional challenges and amendments to the PPACA in the future. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payers. 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.

We face credit risks from 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 health care laws, we may be required to pay a penalty or be suspended from participation in federal or state health care programs, which may adversely affect our business, financial condition and results of operation.

We are subject to various federal and state health care 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 health care 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 health care 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. 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 health care 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 health care 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 health care 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 health care 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, curtailment of our operations, debarment, suspension or exclusion from participation in federal or state health care programs, any of which could

adversely affect our business, financial condition and results of operation.

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, Naglazyme and Vimizim, and all of the sales of Firdapse are 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;

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- 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 and exposure to fluctuations in foreign currency exchange rates;
  - 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.

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.

From time to time, we may 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 Naglazyme, Aldurazyme 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 Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse. 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:

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- 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 no meaningful 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.
- 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. We may not have the financial ability to sustain a patent infringement action, or it may not be financially reasonable to do so.
- 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.

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, data submitted to the EMA in MAAs may be subject to public disclosure. Exactly how the new disclosure policy will be implemented is unclear; however, it could result in the EMA’s public disclosure of certain of our clinical study reports, including pre-clinical data, and patient level data. 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 pre-clinical and clinical 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 Kyndrisa and other antisense oligonucleotides, reveglucosidase alfa and BMN 270,



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.

Furthermore, 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, for example, reveglucosidase alfa and talazoparib (sold to Medivation in October 2015), and several of our former and current product programs have been developed through licensing or collaborative arrangements, such as Naglazyme, Aldurazyme, Kuvan and Firdapse. 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. Since 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 use litigation and 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 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. In addition to our patent protection, we have received three-year Hatch-Waxman exclusivity for a New Patient Population for Kuvan that expires in October 2017, including pediatric exclusivity. Thus, depending on the proposed labeling of a generic product, generic versions of Kuvan may be prohibited until October 2017, though it is possible that an ANDA

applicant could propose to carve out information in the Kuvan labeling protected by the New Patient Population exclusivity and obtain approval earlier.

We 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 Orange Book. Additionally, we received a paragraph IV notice letter, dated January 22, 2015, from Par, notifying us that Par has 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. Together with Merck & Cie, on March 6, 2015 we filed lawsuits against both DRL and Par in the U.S. District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan tablets and seeking an injunction to prevent Par from introducing a generic version of Kuvan tablets that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of each ANDA in accordance with the Hatch-Waxman Act, which expires in July 2017. In response, DRL and Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

In September 2015, we entered into a settlement agreement with DRL that resolved the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral tablets. Under the terms of the settlement agreement, we have granted DRL a non-exclusive license to our Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100 mg tablets in the U.S. for the indications approved for Kuvan beginning at a confidential date in the future, but which is more than five years from the settlement date, or earlier under certain circumstances.

The settlement with DRL does not affect the case against Par, and the litigation against Par is still pending. The parties submitted opening claim construction briefs on January 14, 2016, and responsive claim construction briefs are due on March 4, 2016. The Court has not yet set a date for trial in the litigation against Par.

We also received a paragraph IV notice letter, dated January 14, 2016, from Par, notifying us that Par has filed a separate ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral powder prior to the expiration of our patents listed in the FDA's Orange Book. On February 22, 2016, we filed a lawsuit against Par in the U.S. District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan powder and seeking an injunction to prevent Par from introducing a generic version of Kuvan powder that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2018.

The filing of DRL's and Par's purported ANDAs in respect to Kuvan 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 Par 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 following the settlement described above 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 acquire in the future may be intended for patient populations that are significantly larger than any of Mucopolysaccharidosis I (MPS I), Mucopolysaccharidosis VI (MPS VI), PKU or Lambert Eaton Myasthenic Syndrome (LEMS). 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 drug products are approved, if doctors elect a course of treatment which does not include our drug products, this decision would reduce demand for our drug 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 Naglazyme, Vimizim, and Aldurazyme in MPS diseases, could be greatly reduced. Changes in treatment method can be caused by the introduction of other companies' products or the development of new technologies or surgical procedures which may not directly compete with ours, but which have the effect of changing how doctors decide to treat a disease.

If 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.

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 health care 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.

For the year ended December 31, 2015, 5% of our net product revenues were from Italy, Spain, Portugal, Greece and Russia. Approximately 9% of our total accounts receivable as of December 31, 2015 are related to these countries and we have included an allowance for doubtful accounts for certain accounts receivable from Greece. If the financial conditions of these countries continues to decline, a substantial portion of the receivables may be uncollectable, which would mean we would have to provide for additional

allowances for doubtful accounts or cease selling products in these countries, either of which could adversely affect our results of operations. Additionally, if one or more of these countries were unable to purchase our products, our revenue would be adversely affected. We also sell our products in other countries that face economic crises and local currency devaluation. Although we have historically collected receivables from customers in those countries, sustained weakness or further deterioration of the local economies and currencies may cause our customers in those countries to be unable to pay for our products with the same negative effect on our operations.

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.

Recent and future regulatory actions and other events may adversely affect the trading price and liquidity of our senior subordinated convertible notes.

We expect that many investors in, and potential purchasers of, the Notes will employ, or seek to employ, a convertible arbitrage strategy with respect to the Notes. Investors would typically implement such a strategy by selling short the common stock underlying the Notes and dynamically adjusting their short position while continuing to hold the Notes. Investors may also implement this type of strategy by entering into swaps on our common stock in lieu of or in addition to short selling the common stock.

The SEC and other regulatory and self-regulatory authorities have implemented various rules and taken certain actions, and may in the future adopt additional rules and take other actions, that may impact those engaging in short selling activity involving equity securities (including our common stock). Such rules and actions include Rule 201 of SEC Regulation SHO, the adoption by the Financial Industry Regulatory Authority, Inc. of a "Limit Up-Limit Down" program, the imposition of market-wide circuit breakers that halt trading of securities for certain periods following specific market declines, and the implementation of certain regulatory reforms required by the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010. Any governmental or regulatory action that restricts the ability of investors in, or potential purchasers of, the Notes to effect short sales of our common stock or enter into swaps on our common stock could adversely affect the trading price and the liquidity of the Notes.

In addition, if investors and potential purchasers seeking to employ a convertible arbitrage strategy are unable to borrow or enter into swaps on our common stock, in each case on commercially reasonable terms, the trading price and liquidity of the Notes may be adversely affected.

Risks Related to our Acquisition of Rights to Kuvan and Pegvaliase (the Phenylketonuria (PKU) Franchise)

from Merck Serono

If we are unable to successfully integrate the expanded PKU franchise we acquired from Merck Serono into our existing business operations, our business could be adversely affected.

We will need to successfully integrate the PKU franchise rights we acquired from Merck Serono with our other business operations. Before the transaction with Merck Serono, we had exclusive rights to Kuvan in the U.S. and Canada and to pegvaliase in the U.S. and Japan. After the closing of the transaction with Merck Serono, we now have exclusive worldwide rights to Kuvan and pegvaliase, with the exception of Kuvan in Japan. Integrating the expanded PKU business with our existing business will be a complex and time-consuming process. There may be substantial difficulties, costs and delays involved in any integration of the expanded PKU business with that of our existing operations. These may include:

- distracting management from day-to-day operations;
- an inability to achieve synergies as planned;
- costs and delays in transitioning activities from Merck Serono, particularly with respect to the transfer of the Kuvan marketing authorizations;
- impact of unforeseen country level regulatory changes, delays and actions;
- difficulties in establishing distribution arrangements for Kuvan in all territories;
- reliance on Merck Serono to provide critical transition services for sales and distribution of Kuvan until marketing authorizations can be transferred in all countries;
- difficulties with respect to the timing and results of ongoing and future clinical trials of pegvaliase;
- an inability to manufacture both Kuvan and pegvaliase (pending regulatory approval) in the quantity and configuration required for each jurisdiction and intended use; and

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- increased challenges in managing our business due to the global expansion of the PKU franchise, including, for example, generic competition to Kuvan.

Many of these risks may be accentuated because the acquired rights relate to activities outside of the U.S. Any one or all of these factors may increase operating costs or lower anticipated financial performance. Many of these factors are also outside of our control. Achieving anticipated synergies and the potential benefits underlying our reasons for the acquisition will depend on successful integration of the PKU franchise rights. The failure to integrate the expanded PKU business successfully would have a material adverse effect on our business, financial condition and results of operations.

The actual impact of the acquisition of the PKU franchise rights on our financial results may be worse than the assumptions we have used.

Even if the integration of the PKU franchise rights is successful, we have made certain assumptions relating to the impact on our financial results in respect of the acquisition. These assumptions relate to numerous matters, including:

- the amount of intangible assets that will result from the acquisition;
- the impact of fair value adjustments to contingent acquisition consideration payable as a result of changes in estimated probability and timing of achieving the milestones;
- acquisition costs, including transaction and integration costs;
- the impact of impairment and other charges if the commercialization of Pegvaliase is unsuccessful; and
- other financial and strategic risks of the acquisition.

Irrespective of our assumptions, we may incur higher than expected operating, transaction and integration costs, and we may encounter general economic and business conditions that adversely affect us following the acquisition. If one or more of these assumptions are incorrect, it could have an adverse effect on our business and operating results, and the perceived benefits from the acquisition may not be realized.

#### Risks Related to our Acquisition of Prosensa Holding N.V.

The actual impact of the acquisition of Prosensa on our capital structure and financial results may be worse than the assumptions we have used.

We have made certain assumptions relating to the impact on our capital structure and financial results in respect of the acquisition. These assumptions relate to numerous matters, including:

- our expected capital structure after the acquisition;
- the amount of goodwill and intangibles that will result from the acquisition;
- the impact of fair value adjustments to contingent acquisition consideration payable as a result of changes in estimated probability and timing of achieving the milestones;
- the impact of additional impairment and other charges if the commercialization of Kyndrisa is unsuccessful;
- acquisition costs, including restructuring charges and transaction costs; and
- other financial and strategic risks of the acquisition.

Irrespective of our assumptions, we may incur higher than expected operating, transaction and integration costs, and we may encounter general economic and business conditions that adversely affect the combined company following the acquisition. If one or more of these assumptions are incorrect, it could have an adverse effect on our business and operating results, and the perceived benefits from the acquisition may not be realized.

We may have exposure to additional tax liabilities as a result of the acquisition of Prosensa.

As a multinational corporation, we are subject to income taxes as well as non-income based taxes, in both the U.S. and various foreign jurisdictions. Significant judgment is required in determining our worldwide provision for income taxes and other tax liabilities. Changes in tax laws or tax rulings may have a significantly adverse impact on our

effective tax rate. Proposals by the current U.S. administration for fundamental U.S. international tax reform, including without limitation provisions that would limit the ability of U.S. multinationals to defer U.S. taxes on foreign income, if enacted, could have a significant adverse impact on our effective tax rate following the acquisition.

We are subject to a variety of additional risks as a result of the acquisition of Prosensa that may negatively impact our operations.

As a result of the acquisition, we are subject to new and additional risks associated with the business and operations of Prosensa and its global operations. The additional risks we may be exposed to include but are not limited to the following:

- tariffs and trade barriers;
- regulations related to customs and import/export matters (including sanctions);
- longer payment cycles;
- tax issues, such as tax law changes and variations in tax laws as compared to the jurisdictions in which we already operate;
- operating under regulations in new jurisdictions related to obtaining eligibility for government or private payer reimbursement for our products at the wholesale/retail level;
- cultural and language differences in the new jurisdictions in which we operate;
- complying with additional employment regulations in the new jurisdictions in which we operate; and
- risks related to crimes, strikes, riots, civil disturbances, terrorist attacks and wars in new geographical locations.

We cannot assure you that we will be able to adequately address these additional risks. If we are unable to do so, our operations might suffer.

Additionally, although prior to the acquisition we had international operations, as a result of the acquisition, we operate on an expanded global basis with additional offices or activities in Europe. We will face increased exposure to risks inherent in conducting business internationally, including compliance with international laws and regulations and laws and regulations of the U.S. and various other countries that apply to our international operations. Compliance with these laws and regulations may increase our cost of doing business in foreign jurisdictions. These laws and regulations include laws relating to the pharmaceutical industry, data privacy requirements, labor relations laws, tax laws, anti-competition regulations, import and trade restrictions, export requirements, U.S. laws such as the FCPA, other U.S. federal statutes and regulations, including those established by the Office of Foreign Assets Control, and local laws which prohibit payments to governmental officials. Given the high level of complexity of these laws, however, there is a risk that some provisions may be inadvertently breached by us, for example through fraudulent or negligent behavior of individual employees, our failure to comply with certain formal documentation requirements, or otherwise. Violations of these laws and regulations could result in fines, criminal sanctions against us, our officers or our employees, requirements to obtain export licenses, cessation of business activities in sanctioned countries, implementation of compliance programs, and prohibitions on the conduct of our business. Any such violations could include prohibitions on our ability to offer our products in one or more countries and could materially damage our reputation, our brand, our international expansion efforts, our ability to attract and retain employees, our business and our operating results. Our success depends, in part, on our ability to anticipate these risks and manage these challenges. These factors or any combination of these factors may adversely affect our revenue or our overall financial performance.

If we are unable to commercialize Kyndrisa or experience significant delays in doing so, our business, financial condition and results of operations will be materially adversely affected.

Our ability to generate product revenues from our acquisition of Prosensa will depend heavily on the successful development and eventual commercialization of Kyndrisa, if approved.

In September 2013, Prosensa announced that the Phase 3 clinical trial of Kyndrisa did not meet its primary endpoint. Although we believe that the collective data from Prosensa's various Phase 2 and Phase 3 clinical trials of Kyndrisa, including retrospective and subgroup analyses, provide strong support for concluding that Kyndrisa showed clinically meaningful improvements over placebo in these trials, we cannot be sure that Prosensa's data will be sufficient to obtain regulatory approval in any jurisdiction.

In January 2016 the FDA issued a complete response letter to our NDA for Kyndrisa for the treatment of DMD amenable to exon 51 skipping. The FDA issues complete response letters to indicate that the review cycle for an application is complete and that the application is not ready for approval in its present form. The FDA concluded that the standard of substantial evidence of effectiveness for Kyndrisa had not been met. An MAA for Kyndrisa for the treatment of DMD amenable to exon 51 skipping remains under review in the EU. We anticipate that the CHMP of the EMA will provide an opinion for our MAA for Kyndrisa in the second quarter of 2016. If the CHMP opinion is positive, the MAA will be referred to the EC. The EC is expected to render a final decision for Kyndrisa in the second half of 2016. If the MAA is not approved by the EC, we will not receive marketing authorization for Kyndrisa in the EU and will be unable to sell Kyndrisa in the EU or roll out our international registration strategy for Kyndrisa using the EU approval as the basis of multiple international applications. If our current applications for marketing approval are denied, we may need to conduct additional clinical trials at significant delay and cost or abandon development of Kyndrisa altogether. If we are

ultimately unable to obtain regulatory approval to commercially market and sell Kyndrisa, we may need to write-off all or a significant portion of the costs associated with our acquisition of Prosensa in addition to the impairment charge we recorded in 2015.

Even if we receive regulatory approval for and are able to commercialize Kyndrisa, our success will be subject to the following risks:

- we may not achieve market acceptance of Kyndrisa by physicians, patients and third-party payers;
- Kyndrisa may not have an acceptable safety profile following approval;
- we may not be able to manufacture Kyndrisa in compliance with requirements of the EMA, the FDA and similar regulatory agencies in commercial quantities sufficient to meet market demand;
- we may not achieve sufficient pricing for Kyndrisa to compensate for future development and commercialization costs and to recoup our cost to acquire Prosensa;
- we may not compete successfully with any alternative therapies for DMD; and
- we may not successfully enforce and defend our intellectual property rights and claims.

The occurrence of any of these events could materially adversely affect our business, financial condition and results of operations.

Our conclusions regarding the efficacy of Kyndrisa are based on retrospective analyses of the results of Prosensa's clinical trials, and these analyses may be considered less reliable indicators of efficacy than pre-specified analyses.

After determining that it did not achieve the primary efficacy endpoint in the completed Phase 3 clinical trial of Kyndrisa, Prosensa performed retrospective and subgroup analyses of the Phase 3 clinical trial and prior Phase 2 clinical trials of Kyndrisa that we believe provide strong support for concluding that Kyndrisa showed clinically meaningful improvements over placebo in these trials. Although Prosensa believed that these additional analyses were warranted, a retrospective analysis performed after unblinding trial results can result in the introduction of bias if the analysis is inappropriately tailored or influenced by knowledge of the data and actual results. Because of these limitations, regulatory authorities typically give greatest weight to results from pre-specified analyses and less weight to results from post-hoc, retrospective analyses. Thus, this increases the likelihood that we will have to conduct additional clinical trials of Kyndrisa or may need to abandon development of Kyndrisa altogether if our current applications for marketing approval are denied.

Because Prosensa was developing product candidates for the treatment of diseases in which there is little clinical experience and, in some cases, using new endpoints or methodologies, there is more risk that the outcome of clinical trials for Prosensa's product candidates will not be favorable.

There is currently no approved disease-modifying therapy for DMD. In addition, there has been limited historical clinical trial experience generally for the development of drugs to treat the underlying cause of DMD. As a result, the design and conduct of clinical trials for this disease, particularly for drugs to address the underlying cause of this disease, are subject to increased risks. In particular, regulatory authorities in the U.S. and the EU have not issued definitive guidance as to how to measure and achieve efficacy.

In the last several years, the six-minute walk test (6MWT) has been used in several trials of product candidates for patients with DMD, and is accepted by U.S. and European regulators to be an appropriate primary outcome measure for DMD trials. Because of the limited clinical experience in this indication however, regulators have not yet established what difference in the six-minute walk distance (6MWD) is required to be demonstrated in a clinical trial of a DMD therapy in order to signify a clinically meaningful result and/or obtain regulatory approvals. As a result, it is not clear what is required in terms of 6MWD or other end points to obtain regulatory approval for Kyndrisa and our other product candidates acquired from Prosensa. If we are required to conduct additional clinical trials of Kyndrisa, the design of such trials could be subject to such uncertainties.



We could also face similar challenges in designing clinical trials and obtaining regulatory approval for future product candidates, including any that we may develop for myotonic dystrophy or Huntington's disease because there is also limited historical clinical trial experience for the development of drugs to treat these diseases.

## 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 Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- manufacturing, supply or distribution of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- progress of our integration of the PKU franchise rights acquired from Merck Serono;
- 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;
- results relating to our lawsuits against Par to protect our patents relating to Kuvan and generic competition to Kuvan relating to our settlement with DRL;
- government regulatory action affecting our product candidates or our competitors' drug 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;
- broad market fluctuations in the U.S., the EU or in other parts of the world;
- actual or anticipated fluctuations in our operating results;
- changes in company assessments or financial estimates by securities analysts; and
- sales of our shares of stock by us, our significant shareholders, 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 our company more difficult, even if a change in control would be beneficial to the stockholders. Our anti-takeover provisions include provisions in our certificate of incorporation providing that stockholders' meetings may only be called by our Board of Directors and provisions in our bylaws providing 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 our company. 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 over our company.

The terms of the Notes require us to repurchase the Notes in the event of a fundamental change. A takeover of our company 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 our company that would otherwise be beneficial to our stockholders or investors in the Notes.

#### 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, 2015:

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Location	Approximate Square Feet	Use	Lease Expiration Date
Several locations in Novato, California	225,000	Office, laboratory and warehouse	2016-2020
San Rafael facility, San Rafael, California	226,100	Corporate headquarters, laboratory and office	NA: owned property
Galli Drive facility, Novato, California	98,100	Clinical and commercial manufacturing and laboratory	NA: owned property
Bel Marin Keys facilities, Novato, California	83,000	Technical operations, finance, administration, and laboratory	NA: owned property
Digital Drive facility, Novato, California	47,000	Office and laboratory	NA: owned property
Leveroni Drive facility, Novato, California	38,300	Warehouse	NA: owned property
London, England	18,300	Office	2025
Dublin, Ireland	9,100	Office	2024
Shanbally facility, Cork, Ireland	142,000	Manufacturing	NA: owned property

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 January 22, 2015, from Par, notifying us that Par 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. Together with Merck & Cie, on March 6, 2015, we filed a lawsuit against Par in the United States District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan tablets and seeking an injunction to prevent Par from introducing a generic version of Kuvan tablets that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2017. In response, Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

The parties submitted opening claim construction briefs on January 14, 2016, and responsive claim construction briefs are due on March 4, 2016. The Court has not yet set a date for trial in this litigation.

We also received a paragraph IV notice letter, dated January 14, 2016, from Par, notifying us that Par has filed a separate ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral powder prior to the expiration of our patents listed in the FDA's Orange Book. On February 22, 2016, we filed a lawsuit against Par in the United States District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan powder and seeking an injunction to prevent Par from introducing a generic version of Kuvan powder that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2018.

### 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
2015	Fourth Quarter	\$118.48	\$91.21
2015	Third Quarter	\$151.75	\$95.09
2015	Second Quarter	\$141.51	\$110.50
2015	First Quarter	\$133.54	\$88.51
2014	Fourth Quarter	\$96.36	\$65.91
2014	Third Quarter	\$73.35	\$55.36
2014	Second Quarter	\$70.42	\$55.04
2014	First Quarter	\$84.25	\$64.61

On February 12, 2016, the last reported sale price on the NASDAQ Global Select Market for our common stock was \$70.08. 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, 2015.

## Issuer Purchases of Equity Securities

We did not make any purchases of our common stock during the year ended December 31, 2015.

## Holders

As of February 12, 2016, there were 47 holders of record of 161,590,680 outstanding shares of our common stock. Additionally, on such date, options to acquire 10.2 million shares of our common stock were outstanding.

## 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 of \$100 on December 31, 2010 in BioMarin common stock, the NASDAQ Composite Index (U.S.), the NASDAQ Biotechnology Index and a customized peer group of ten companies that includes: Alexion Pharmaceuticals, Inc., Alkermes Public Limited Company, Endo International Public Limited Company, Incyte Corporation, Jazz Pharmaceuticals Public Limited Company, Medivation, Inc., Regeneron Pharmaceuticals, Inc., Seattle Genetics, Inc., United Therapeutics Corporation and Vertex Pharmaceuticals Incorporated. All values assume reinvestment of the pretax value of dividends paid by companies included in these indices and are calculated as of December 31 of each year. 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, 2010 in stock or index, including reinvestment of dividends.

	Fiscal Year Ending December 31,					
	2010	2011	2012	2013	2014	2015
BioMarin Pharmaceutical Inc.	\$ 100.00	\$ 127.66	\$ 182.70	\$ 261.23	\$ 335.69	\$ 389.01
NASDAQ Composite Index	100.00	100.53	116.92	166.19	188.78	199.95
NASDAQ Biotechnology Index	100.00	113.92	153.97	263.29	348.49	369.06
Peer Group	100.00	127.05	186.94	324.76	452.81	519.92



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Item 6. Selected Consolidated Financial Data

We derived the selected consolidated statements of operations data for the years ended December 31, 2015, 2014 and 2013 and the selected consolidated balance sheet data as of December 31, 2015 and 2014 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, 2012 and 2011 and the selected consolidated balance sheet data as of December 31, 2013, 2012 and 2011 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 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 8 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 thousands, except for per share data)				
	2015	2014	2013	2012	2011
<b>Consolidated statements of operations data:</b>					
<b>REVENUES:</b>					
Net product revenues	\$884,522	\$738,416	\$538,360	\$496,497	\$437,647
Collaborative agreement revenues	1,018	1,592	3,918	1,955	468
Royalty, license and other revenues	4,355	9,276	6,207	2,271	3,243
Total revenues	889,895	749,284	548,485	500,723	441,358
<b>OPERATING EXPENSES:</b>					
Cost of sales (excludes amortization of intangible assets)	152,008	122,267	88,391	84,479	82,859
Research and development	634,806	461,543	354,780	302,218	214,374
Selling, general and administrative	402,271	302,156	235,356	198,173	175,423
Intangible asset amortization and contingent consideration	(17,690 )	23,709	25,026	19,361	2,592
Impairment of intangible asset	198,700	—	939	6,707	—
Gain on sale of intangible asset	(369,498 )	(67,500 )	—	—	—
Total operating expenses	1,000,597	842,175	704,492	610,938	475,248
<b>LOSS FROM OPERATIONS</b>	<b>(110,702 )</b>	<b>(92,891 )</b>	<b>(156,007)</b>	<b>(110,215)</b>	<b>(33,890 )</b>
Equity in the loss of BioMarin/Genzyme LLC	(817 )	(877 )	(1,149 )	(1,221 )	(2,426 )
Interest income	4,501	5,937	3,083	2,584	2,934
Interest expense	(38,244 )	(36,642 )	(10,447 )	(7,639 )	(8,409 )
Debt conversion expense	(163 )	(674 )	(12,965 )	—	(1,896 )
Other income (expense)	(9,299 )	279	982	(1,787 )	60
<b>LOSS BEFORE INCOME TAXES</b>	<b>(154,724 )</b>	<b>(124,868)</b>	<b>(176,503)</b>	<b>(118,278)</b>	<b>(43,627 )</b>
Provision for (benefit from) income taxes	17,075	9,101	(150 )	(3,931 )	10,209
<b>NET LOSS</b>	<b>\$(171,799 )</b>	<b>\$(133,969)</b>	<b>\$(176,353)</b>	<b>\$(114,347)</b>	<b>\$(53,836 )</b>
<b>NET LOSS PER SHARE, BASIC AND DILUTED</b>	<b>\$(1.07 )</b>	<b>\$(0.92 )</b>	<b>\$(1.28 )</b>	<b>\$(0.95 )</b>	<b>\$(0.48 )</b>
Weighted average common shares outstanding, basic and diluted	160,025	146,349	137,755	120,271	112,122

December 31,  
(in thousands)  
2015      2014      2013      2012      2011

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Consolidated balance sheets data:

Cash, cash equivalents and investments	\$1,018,271	\$1,043,048	\$1,052,423	\$563,798	\$289,477
Total current assets	1,089,639	1,397,640	1,103,668	711,048	469,802
Total assets	3,729,368	2,475,379	2,225,497	1,564,645	1,268,557
Long-term convertible senior notes, net	662,286	642,902	637,003	321,157	348,329
Total stockholders' equity	2,400,847	1,527,894	1,341,041	1,015,763	773,048

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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 thousands, except per share data, unaudited)			
				December
	March 31,	June 30,	September 30,	31,
<b>2015:</b>				
Total revenues	\$202,920	\$250,135	\$ 208,904	\$227,936
Net income (loss)	(67,501 )	(81,989 )	(90,926 )	68,617
Net income (loss) per share, basic	(0.43 )	(0.51 )	(0.57 )	0.43
Net income (loss) per share, diluted	(0.43 )	(0.51 )	(0.60 )	0.39
<b>2014:</b>				
Total revenues	\$151,552	\$191,114	\$ 176,549	\$230,069
Net income (loss)	(38,115 )	(33,502 )	7,445	(69,797 )
Net income (loss) per share, basic	(0.26 )	(0.23 )	0.05	(0.47 )
Net income (loss) per share, diluted	(0.27 )	(0.23 )	0.05	(0.47 )

## 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 (the MD&A) is intended to help the reader understand our results of operations and financial condition. The MD&A is provided as a supplement to, and should be read in conjunction with, our audited Consolidated Financial Statements and the accompanying notes to the Consolidated Financial Statements and other disclosures included in this Annual Report on Form 10-K, including the disclosures under "Risk Factors" in Part I, Item 1A of this Annual Report on Form 10-K. 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.

### Overview

We develop and commercialize innovative biopharmaceuticals for serious 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 product portfolio consists of five approved products and multiple clinical and pre-clinical product candidates. Our approved products are Vimizim (elosulfase alpha), Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (laronidase) and Firdapse (amifampridine phosphate).

### Business Developments

During 2015, we continued to grow our commercial business and advance our product pipeline. We believe that the combination of our internal research programs, acquisitions and partnerships will allow us to continue develop and commercialize innovative biopharmaceuticals for serious diseases and medical conditions. Below is a summary of our significant business developments:

- In January 2015, we acquired Prosensa Holding N.V. (Prosensa) for a total purchase price of \$751.5 million. See Note 5 to the accompanying Condensed Consolidated Financial Statements for additional discussion.
- In January 2016 the Food and Drug Administration (FDA) issued a complete response letter to our New Drug Application (NDA) for Kyndrisa for the treatment of Duchenne muscular dystrophy (DMD) amenable to exon 51 skipping. The FDA concluded that the standard of substantial evidence of effectiveness for Kyndrisa had not been met. Based on the current U.S. development efforts, we recognized an impairment of the Kyndrisa IPR&D assets totaling \$198.7 million in the fourth quarter of 2015.
- The Marketing Authorization Application (MAA) for Kyndrisa remains under review in the EU. We anticipate that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) will provide an opinion for our MAA for Kyndrisa in the second quarter of 2016. If the CHMP opinion is positive, the MAA will be referred to the European Commission (EC). If the MAA is approved by the EC, we would receive marketing authorization for Kyndrisa in all EU Member States.
- In October 2015, we entered into a definitive agreement to acquire all global rights to Kuvan and pegvaliase from Ares Trading, S.A. (Merck Serono), with the exception of Kuvan in Japan. In December 2015, we paid a deposit on this transaction totaling \$371.8 million. Under the terms of the agreement, we have also agreed to pay Merck Serono additional consideration in future periods up to a maximum of €60.0 million in milestone payments if certain Kuvan sales milestones are attained and up to a maximum of €125.0 million in milestone payments if certain pegvaliase development milestones are met. Our agreement with Merck Serono was effective January 1, 2016. Prior to January 1, 2016, we had exclusive rights to Kuvan in the U.S. and Canada and pegvaliase in the U.S. and Japan.
- In September 2015, we entered into a settlement agreement with Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL) that resolved the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral tablets. Under the terms of the settlement agreement, we have granted DRL a non-exclusive license to our Kuvan related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100 mg tablets in the U.S. for the indications approved for Kuvan beginning at a confidential date in the future, but which is more than

five years from the settlement date, or earlier under certain circumstances.

- In October 2015, we entered into an asset purchase agreement with Medivation, Inc. (Medivation), under which Medivation acquired the worldwide rights to talazoparib in exchange for payment of \$410.0 million and up to an additional \$160.0 million upon the achievement of regulatory and sales-based milestones as well as mid-single digit percentage royalties for talazoparib.
- In September 2015, we announced the enrollment of the first patient in our Phase 1/2 trial for BMN 270, an investigational gene therapy for the treatment of patients with Hemophilia A.

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

- We reported total revenues of \$889.9 million for the year ended December 31, 2015, compared to \$749.3 million and \$548.5 million for the years ended December 31, 2014 and 2013, respectively.
- We announced positive results of a Phase 2 proof-of-concept and dose finding study of vosoritide, formerly referred to as BMN 111, for the treatment of achondroplasia.
- We shared interim data from nine patients in our Phase 1/2 study of cerliponase alfa in patients with late infantile neuronal ceroid lipofuscinosis (CLN2) who have been followed for at least six months and up to 15 months. These data suggest that treatment with cerliponase alfa may result in stabilization of CLN2 disease compared to the natural history based on a standardized measure of motor and language function. We expect to release the complete results from this Phase 1/2 trial in March 2016.

## Outlook 2016

In 2016, 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 existing products and pipeline of new drug 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 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 2016 operating objectives.

## 2015 Financial Highlights

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

	Years Ended December 31,		
	2015	2014	2013
Net product revenues	\$884.5	\$738.4	\$538.4
Cost of sales (excluding amortization of intangible assets)	152.0	122.3	88.4
R&D expense	634.8	461.5	354.8
Selling, general and administrative (SG&A) expense	402.3	302.2	235.4
Intangible asset amortization and			
contingent consideration expense	(17.7 )	23.7	25.0
Net loss	(171.8)	(134.0)	(176.4)
Stock-based compensation expense	111.5	86.4	64.4

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

Total net product revenues were as follows (in millions):

	Years Ended December		
	31,		
	2015	2014	2013
Vimizim	\$228.1	\$77.3	\$0.1
Naglazyme	303.1	334.4	271.2
Kuvan	239.3	203.0	167.4
Aldurazyme	98.0	105.6	83.6
Firdapse	16.0	18.1	16.1
Total net product revenues	\$884.5	\$738.4	\$538.4

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

Our cash, cash equivalents and investments totaled \$1,018.3 million as of December 31, 2015, compared to \$1,043.1 million as of December 31, 2014. 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, even after giving effect to our January 2015 equity offering. 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 or cash equivalents 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 and Estimates

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. We base our assumptions, judgments and estimates on historical experience and various other factors that we believe to be reasonable under the circumstances. Actual results could differ materially from these estimates under different assumptions or conditions. On a regular basis, we evaluate our assumptions, judgments and estimates. We also discuss our critical accounting policies and estimates with the Audit Committee of our Board of Directors.

We believe that the assumptions, judgments and estimates involved in the accounting for business combinations, contingent acquisition consideration payable, income taxes, long-lived assets, revenue recognition and inventory have the greatest impact on our Consolidated Financial Statements, so we consider these to be our critical accounting policies. Historically, our assumptions, judgments and estimates relative to our critical accounting policies have not differed materially from actual results.

#### Business Combinations

We allocate 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, we estimate the fair value of contingent acquisition consideration payments utilizing a probability-based income approach inclusive of an estimated discount rate.

Although we believe the assumptions and estimates made are reasonable, they are based in part on historical experience and information obtained from the management of the acquired businesses and are inherently uncertain. Examples of critical estimates in valuing any contingent acquisition consideration issued or that may be issued and the intangible assets we have acquired or may acquire in the future include but are not limited to:

- the feasibility and timing of achievement of development, regulatory and commercial milestones;
  - expected costs to develop the IPR&D into commercially viable products; and
- future expected cash flows from product sales.

Unanticipated events and circumstances may occur that may affect the accuracy or validity of such assumptions, estimates or actual results.



### Valuation of Contingent Acquisition Consideration Payable

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. Increases or decreases in the fair value of the contingent acquisition consideration payable can result from changes in estimated probability adjustments 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. Significant judgment is employed in determining the appropriateness of these assumptions each period. Accordingly, future business and economic conditions, as well as changes in any of the assumptions described in the accounting for business combinations above can materially impact the amount of contingent consideration expense that we record in any given period.

### Income Taxes

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

Our Consolidated Balance Sheets reflect net deferred tax assets and liabilities. The 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. Each quarter, we evaluate the need to retain all or a portion of the valuation allowance on our net deferred tax assets. Our evaluation considers historical earnings, estimated future taxable income and ongoing prudent and feasible tax planning strategies. Adjustments to the valuation allowance increase or decrease net income/loss in the period such adjustments are made. The deferred tax liabilities primarily represent the tax implications of future amortization or possible impairments associated with acquired IPR&D. If our estimates require adjustments, it could have a significant impact on our Consolidated Financial Statements. We continually review the adequacy and necessity of the valuation allowance. Changes in tax laws and rates could also affect recorded deferred tax assets in the future. Management is not aware of any such changes that would have a material effect on our Consolidated Financial Statements.

#### Impairment of Long-Lived Assets

Our long-lived assets include property, plant and equipment, intangible assets and goodwill. We review the carrying value of plant and equipment and finite-lived intangible assets for impairment whenever events or changes in circumstances indicate that the carrying amount of an asset may not be recoverable. If such circumstances exist, an estimate of undiscounted future cash flows to be generated by the long-lived asset is compared to the carrying value to determine whether an impairment exists. If an asset is determined to be impaired, the loss is measured based on the difference between the asset's fair value and its carrying value.

Indefinite-lived intangible assets, composed primarily of IPR&D projects acquired in business combinations that have not reached technological feasibility, are reviewed annually for impairment and whenever events or changes in circumstances indicate that the carrying amount may not be recoverable. We determine impairment by comparing the fair value of the asset to its carrying value. If the asset's carrying value exceeds its fair value, an impairment charge is recorded for the difference and its carrying value is reduced accordingly.

Estimating future cash flows of an IPR&D product candidate for purposes of an impairment analysis requires us to make significant estimates and assumptions regarding the amount and timing of costs to complete the project and the amount, timing and probability of achieving revenues from the completed product similar to how the acquisition date fair value of the project was determined, as described above. There are often major risks and uncertainties associated with IPR&D projects as we are required to obtain regulatory approvals in order to be able to market these products. Such approvals require completing clinical trials that demonstrate a product candidate is safe and effective. Consequently, the eventual realized value of the acquired IPR&D project may vary from its estimated fair value at the date of acquisition, and IPR&D impairment charges may occur in future periods which could have a material adverse effect on our results of operations.

We believe our estimations of future cash flows used for assessing impairment of long-lived assets are based on reasonable assumptions given the facts and circumstances as of the related dates of the assessments.

When reviewing goodwill for impairment, we assess whether goodwill should be allocated to operating levels lower than our single operating segment for which discrete financial information is available and reviewed for decision-making purposes. These lower levels are referred to as reporting units. Currently, we have identified only one reporting unit as per Financial Accounting Standards Board (FASB) Accounting Standards Codification (ASC) Topic 350-20, Intangibles—Goodwill and Other. We perform our annual impairment review of goodwill during the fourth quarter and whenever events or circumstances indicate that the carrying amount of an asset may not be recoverable.

Our impairment review was based on a qualitative assessment or performing a quantitative analysis in determining whether it is more likely than not that the fair value of the net assets are below their carrying amounts. Examples of qualitative factors assessed in 2015 include industry and market considerations and other entity specific factors that may have a significant impact on the fair value of our goodwill. Based on our qualitative assessment, we determined that the fair value of our goodwill is greater than its carrying amount at December 31, 2015.

#### Revenue Recognition

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.

Net Product Revenues—We recognize 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 our Consolidated Statements of Operations, in that taxes billed to customers are not included as a component of net product revenues.

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

In the U.S., our commercial products are generally sold to specialty pharmacies or end-users, such as hospitals, which act as retailers. Through December 31, 2015, we sold Kuvan to 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 EU was included as a component of net product revenues in the period earned and approximates 4% of Merck Serono's world-wide sales. 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.

We receive a 39.5% to 50% royalty on worldwide net Aldurazyme sales by Genzyme Corporation (Genzyme) depending on sales volume, which is included in Net Product Revenues in our Consolidated Statements of Operations. We recognize a portion of this amount as product transfer revenue when the product is 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 calculated royalty rate when the product is sold by Genzyme. We record the Aldurazyme royalty revenue 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. As of December 31, 2015 and 2014, accounts receivable included \$36.1 million and \$34.5 million, respectively, of unbilled accounts receivable related to net incremental Aldurazyme product transfers to Genzyme.

We record reserves for rebates payable under Medicaid and other government programs as a reduction of revenue at the time product revenues are recorded. Our reserve calculations require estimates, including estimates of customer mix, to determine which sales will be subject to rebates and the amount of such rebates. We update our estimates and assumptions each quarter and record any necessary adjustments to our reserves. We record fees paid to distributors as a reduction of revenue.

We record allowances for product returns, if appropriate, as a reduction of revenue at the time product sales are recorded. Several factors are considered in determining whether an allowance for product returns is required, including market exclusivity of the products based on their orphan drug status, the patient population, the customers' limited return rights and our experience with returns. Because of the pricing of our products, the limited number of patients and 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, we have not experienced an increase in product returns and do not believe these buying patterns increase the risk of product returns. We rely on historical return rates to estimate returns for our commercial products. Genzyme's contractual return rights for Aldurazyme are limited to defective product. Based on these factors and the fact that we have 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 changes, an allowance for product returns may be required.

Bad debt reserves are based on estimated uncollectible accounts receivable. Given our historical experience with bad debts, combined with our credit management policies and practices, we do not presently maintain significant bad debt reserves. However some of our customers are based in countries where the economic conditions continue to present challenges. We continue to monitor these conditions and associated impacts on the financial performance and credit worthiness of our large customers so that we can properly assess and respond to changes in customer credit profiles. As of December 31, 2015 and 2014, our allowance for doubtful accounts was \$0.1 million and \$0.5 million,

respectively.

The nature and amount of our current estimates of the applicable revenue dilution items that are currently applied to aggregate world-wide gross product sales of Vimizim, Naglazyme, Kuvan and Firdapse to derive net sales are described in the table below.

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

Gross Revenue to Net Revenue Adjustments	Percentage of Gross Sales		Description
	Years Ended December 31, 2015	2014	
			Rebates payable to state
			Medicaid, other government programs and certain managed care providers
Rebates	0.2 – 5.7%	0.3 – 7.3%	Fees paid to authorized distributors
Distributor Fees	0.2 – 5.6%	0.2 – 3.4%	Discounts offered to customers for prompt payment of accounts receivable
Cash Discounts	0.5 – 1.8%	1.0 – 1.8%	
Total	0.9 – 13.1%	1.5 – 12.5%	

Collaborative Agreement Revenues—Collaborative agreement revenues include both license revenue and contract research revenue.

Activities under collaborative agreements are evaluated to determine if they represent a multiple element revenue arrangement. We identify the deliverables included within the agreement and evaluate which deliverables represent separate units of accounting. We account for those components as separate units of accounting if the following two criteria are met:

- The delivered item or items have value to the customer on a stand-alone basis; and
- If there is a general right of return relative to the delivered items, delivery or performance of the undelivered items is considered probable and within our control.

Factors considered in this determination include, among other things, whether any other vendors sell the items separately and if the licensee could use the delivered item for its intended purpose without the receipt of the remaining deliverables. If multiple deliverables included in an arrangement are separable into different units of accounting, we allocate the arrangement consideration to those units of accounting. The amount of allocable arrangement consideration is limited to amounts that are fixed or determinable. Arrangement consideration is allocated at the inception of the arrangement to the identified units of accounting based on their relative estimated selling price. Revenue is recognized for each unit of accounting when the appropriate revenue recognition criteria are met.

Nonrefundable up-front license fees where we have continuing involvement through performance of research and development activities are initially deferred and recognized as collaborative agreement license revenue over the estimated period for we continue to have a performance obligation.

Future milestone payments that are contingent upon the achievement of a substantive milestone are recognized in their entirety in the period in which the milestone is achieved. A milestone is substantive if:

- It can only be achieved based in whole or in part on either our performance or on the occurrence of a specific outcome resulting from our performance;
- There is substantive uncertainty at the date an arrangement is entered into that the event will be achieved; and
- It would result in additional payments being due to us.

Royalty, license and other revenues—Royalty, license and other revenues includes royalties on net sales of products with which we have no direct involvement and is recognized based on data reported by licensees or sublicensees and rental income associated with tenants in the San Rafael Corporate Center (the SRCC) is recognized on a straight-line basis over the term of the respective leases. Royalties are recognized as earned in accordance with the contract terms when the royalty amount is fixed or determinable based on information received from the sublicensee and when collectibility is reasonably assured.

Due to the significant role we play in the operations of Aldurazyme and Kuvan, primarily the manufacturing and regulatory activities, as well as the rights and responsibilities to deliver the products to Genzyme and Merck Serono, respectively, we elected not to classify the Aldurazyme and Kuvan royalties earned as royalty, license and other revenues and instead to include them as a component of net product revenues.

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

### Inventory

We value our inventory at the lower of cost or net realizable value and determine the cost of inventory using the average-cost method. Inventories consist of currently marketed products and may contain certain products awaiting regulatory approval. In evaluating the recoverability of inventories produced in preparation for product launches, we consider the likelihood that revenue will be obtained from the future sale of the related inventory together with the status of the product within the regulatory approval process.

We analyze our inventory levels quarterly and write down inventory that has become obsolete, or has a cost basis in excess of its expected net realizable value and inventory quantities in excess of expected requirements.

### Inventories Produced in Preparation for Product Launches

We capitalize inventories produced in preparation for product launches sufficient to support estimated initial market demand. Typically, capitalization of such inventory begins when positive results have been obtained for the clinical trials that we believe are necessary to support regulatory approval, uncertainties regarding ultimate regulatory approval have been significantly reduced and we have determined it is probable that these capitalized costs will provide future economic benefit in excess of capitalized costs. The factors considered by us in evaluating these uncertainties include the receipt and analysis of positive pivotal clinical trial results for the underlying product candidate, results from meetings with the relevant regulatory authorities prior to the filing of regulatory applications, and the compilation of the regulatory application. We closely monitor the status of each respective product within the regulatory approval process, including all relevant communication with regulatory authorities. We also consider our historical experience with manufacturing and commercializing similar products and the relevant product candidate. If we are aware of any specific material risks or contingencies other than the normal regulatory review and approval process or if there are any specific issues identified relating to safety, efficacy, manufacturing, marketing or labeling, the related inventory would generally not be capitalized.

For inventories that are capitalized in preparation of product launch, anticipated future sales, expected approval date and shelf lives are evaluated in assessing realizability. The shelf life of a product is determined as part of the regulatory approval process; however in evaluating whether to capitalize pre-launch inventory production costs, we consider the product stability data of all of the pre-approval production to date to determine whether there is adequate expected shelf life for the capitalized pre-launch production costs. 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. Expired inventory is disposed of and the related costs are recognized as Cost of Sales in our Consolidated Statements of Operations.

### Recent Accounting Pronouncements

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



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

## Results of Operations

## Net Loss

Our net loss for the year ended December 31, 2015 was \$171.8 million, compared to a net loss of \$134.0 million and \$176.4 million for the years ended December 31, 2014 and 2013, respectively. The decrease in net loss was primarily a result of the following (in millions):

	Years Ended December 31,			2015 vs. 2014	2014 vs. 2013
	2015	2014	2013		
Total revenues	\$889.9	\$749.3	\$548.5	\$140.6	\$200.8
Cost of sales	152.0	122.3	88.4	29.7	33.9
R&D expense	634.8	461.5	354.8	173.3	106.7
SG&A expense	402.3	302.2	235.4	100.1	66.8
Intangible asset amortization and contingent consideration	(17.7 )	23.7	25.0	(41.4 )	(1.3 )
Impairment of intangible asset	198.7	—	0.9	198.7	(0.9 )
Gain on sale of intangible asset	(369.5)	(67.5 )	-	(302.0)	(67.5 )
Other, net	(44.0 )	(32.0 )	(20.6 )	(12.0 )	(11.4 )
Provision for (benefit from) income taxes	17.1	9.1	(0.2 )	8.0	9.3
Net loss	\$(171.8)	\$(134.0)	\$(176.4)	\$(37.8 )	\$42.4

See below for additional information related to the primary net loss fluctuations presented above, including details of our operating expense fluctuations.

## Net Product Revenues, Cost of Sales and Gross Margins

Net product revenues were as follows (in millions):

	Years Ended December 31,			2015 vs. 2014	2014 vs. 2013
	2015	2014	2013		
Vimizim	\$228.1	\$77.3	\$0.1	\$ 150.8	\$ 77.2
Naglazyme	303.1	334.4	271.2	(31.3 )	63.2
Kuvan	239.3	203.0	167.4	36.3	35.6
Aldurazyme	98.0	105.6	83.6	(7.6 )	22.0
Firdapse	16.0	18.1	16.1	(2.1 )	2.0
Total net product revenues	\$884.5	\$738.4	\$538.4	\$ 146.1	\$ 200.0

Net product revenues attributable to our collaboration with Genzyme were as follows (in millions):

	Years Ended December 31,			2015 vs. 2014	2014 vs. 2013
	2015	2014	2013		
Aldurazyme revenue reported by Genzyme	\$217.8	\$228.8	\$212.4	\$ (11.0 )	\$ 16.4

Royalties earned from Genzyme	\$95.8	\$97.0	\$88.5	\$ (1.2	)	\$ 8.5
Incremental (previously recognized) Aldurazyme						
product transfer revenue	2.2	8.6	(4.9	)	(6.4	) 13.5
Total Aldurazyme net product revenues	\$98.0	\$105.6	\$83.6	\$ (7.6	)	\$ 22.0

2015 compared to 2014

The FDA and the EMA granted marketing approval for Vimizim in February 2014 and April 2014, respectively, and Vimizim subsequently received marketing approval in other countries. We began marketing Vimizim immediately following approval in each of these markets. Vimizim net product revenues for the year ended December 31, 2015 totaled \$228.1 million, compared to \$77.3 million for the year ended December 31, 2014. Vimizim net product revenues earned from customers based outside the U.S. during the year ended December 31, 2015 totaled \$150.5 million, compared to \$36.6 million for the year ended December 31, 2014. The

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

increase in Vimizim net product revenues for the year ended December 31, 2015 was attributable to new patients initiating therapy. The impact of foreign currency exchange rates on Vimizim sales was negative by \$18.9 million for the year ended December 31, 2015. Gross margin (net product revenues less costs of sales, expressed as a percentage of net product revenues) for Vimizim gross margin was 84% for the year ended December 31, 2015, compared to gross margin of 87% for the year ended December 31, 2014. Vimizim gross margin in 2014 benefited from the sale of product manufactured in 2013 prior to us commencing capitalization of Vimizim manufacturing costs. In future periods, we do not expect Vimizim gross margins to fluctuate significantly.

Naglazyme net product revenues for the year ended December 31, 2015 totaled \$303.1 million, compared to \$334.4 million for the year ended December 31, 2014. Naglazyme net product revenues earned from customers based outside the U.S. totaled \$260.4 million for the year ended December 31, 2015, compared to \$294.6 million for the year ended December 31, 2014. The decrease in Naglazyme net product revenues was attributable to the negative impact of foreign currency exchange rates and significant purchases from certain government entities occurring in 2014, offset by new patients initiating therapy. The impact of foreign currency exchange rates on Naglazyme during 2015 was negative by \$12.5 million compared to a negative impact of \$0.2 million during 2014. Our gross margin for Naglazyme was 87% for the year ended December 31, 2015, compared to 88% for the year ended December 31, 2014. In future periods, we do not expect Naglazyme gross margins to fluctuate significantly.

Kuvan net product revenues for the year ended December 31, 2015 totaled \$239.3 million, compared to \$203.0 million for the year ended December 31, 2014. The increase in Kuvan net product revenues was attributable to new patients initiating therapy. Our gross margin for Kuvan was 83% for the year ended December 31, 2015, compared to 85% for the year ended December 31, 2014. Cost of goods sold for each of the years ended December 31, 2015 and 2014 reflect royalties paid to third-parties of approximately 10%. The royalties earned from Merck Serono's net sales of Kuvan for the year ended December 31, 2015 were \$2.0 million, compared to \$2.2 million for the year ended December 31, 2014.

In 2016, we expect Kuvan net product revenues to increase over 2015 levels as a result of our agreement with Merck Serono to acquire global rights to Kuvan and pegvaliase, with the exception of Japan, which was effective January 1, 2016. Kuvan gross margins are not expected to fluctuate significantly in the future. However we expect to see generic competition for Kuvan in the future.

In September 2015, we entered into a settlement agreement with DRL that resolved patent litigation with DRL in the U.S. related to its ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral tablets. Under the terms of the settlement agreement, we have granted DRL a non-exclusive license to our Kuvan related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100mg tablets in the U.S. for the indications approved for Kuvan beginning at a confidential date in the future, but which is more than five years from the settlement date, or earlier under certain circumstances. The settlement does not affect the case against Par Pharmaceutical, Inc. (Par) with respect to its 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. Par has also filed a separate ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral powder.

Our settlement with DRL and the filing of Par's purported ANDAs with respect to Kuvan could have an adverse impact on our stock price, and litigation to enforce our patents is likely 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 Par 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 following the settlement described above could have a material adverse effect on our revenue

and results of operations.

Aldurazyme net product revenues for the year ended December 31, 2015 totaled \$98.0 million, compared to \$105.6 million for the year ended December 31, 2014. The decrease in Aldurazyme net product revenues was primarily attributable to a decrease in Genzyme reported Aldurazyme sales. Our gross margin on Aldurazyme was 68% for the year ended December 31, 2015, compared to 65% for the year ended December 31, 2014. Aldurazyme gross margins reflect the profit earned on royalty revenue and net incremental product transfer revenue. Aldurazyme gross margins are expected to fluctuate depending on the mix of royalty revenue, from which we earn higher gross profit, and product transfer revenue, from which we earn lower gross profit.

Total cost of sales for the years ended December 31, 2015 and 2014 were \$152.0 million and \$122.3 million, respectively. The increase in cost of sales was primarily attributable to the increase in product sales.

2014 compared to 2013

Vimizim net product revenues for the year ended December 31, 2014 totaled \$77.3 million, of which \$36.6 million, was earned from customers based outside the U.S. Net product revenues for Vimizim for the year ended December 31, 2013 totaled \$0.1 million. Our gross margin for Vimizim was 87% for the year ended December 31, 2014.

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

Naglazyme net product revenues for the year ended December 31, 2014 totaled \$334.4 million, of which \$294.6 million was earned from customers based outside the U.S., compared to \$271.2 million for the year ended December 31, 2013, of which \$233.5 million was earned from customers based outside the U.S. The increase in Naglazyme net product revenues for the year ended December 31, 2014 was attributable to new patients initiating therapy. The impact of foreign currency exchange rates on Naglazyme sales was negative by \$0.2 million for the year ended December 31, 2014. Our gross margin for Naglazyme was 88% for the years ended December 31, 2014 and 2013.

Kuvan net product revenues for the year ended December 31, 2014 totaled \$203.0 million, compared to \$167.4 million for the year ended December 31, 2013. The increase in Kuvan net product revenues was attributable to new patients initiating therapy. Our gross margin for Kuvan was 85% for the year ended December 31, 2014 and 2013. Cost of goods sold for each of the years ended December 31, 2014 and 2013 reflect royalties paid to third-parties of approximately 10%. The royalties earned from Merck Serono's net sales of Kuvan for the year ended December 31, 2014 were \$2.2 million, compared to \$2.0 million for the year ended December 31, 2013.

Aldurazyme net product revenues for the year ended December 31, 2014 totaled \$105.6 million, compared to \$83.6 million for the year ended December 31, 2013. The increase in net Aldurazyme product revenues was attributable to new patients initiating therapy and an increase in shipments to Genzyme. Our gross margin for Aldurazyme was 65% for the year ended December 31, 2014, respectively, compared to 68% for the year ended December 31, 2013.

Aldurazyme gross margins reflect the profit earned on royalty revenue and net incremental product transfer revenue. Aldurazyme gross margins are expected to fluctuate depending on the mix of royalty revenue, from which we earn higher gross profit, and product transfer revenue, from which we earn lower gross profit.

Total cost of sales for the year ended December 31, 2014 were \$122.3 million, compared to \$88.4 million for the year ended December 31, 2013. The increase in cost of sales was primarily attributable to the increase in product sales.

### Research and Development

A summary of our various major development programs, including key metrics as of December 31, 2015, is provided below:

Major Products in Development	Target Indication	U.S. Orphan Designation	EU Orphan Designation	Stage
Kyndrisa	DMD <sup>(1)</sup>	Yes <sup>(5)</sup>	Yes	Clinical Phase 3
Pegvaliase	PKU <sup>(2)</sup>	Yes	Yes	Clinical Phase 3
Reveglucosidase alfa	Pompe <sup>(3)</sup>	Yes	Yes	Clinical Phase 2/3
Vosoritide	Achondroplasia	Yes	Yes	Clinical Phase 2
Cerliponase alfa	CLN2 <sup>(4)</sup>	Yes	Yes	Clinical Phase 1/2

(1) Duchenne muscular dystrophy, or DMD, amenable to exon 51 skipping. In January 2015 we acquired Kyndrisa (formerly referred to as drisapersen) from Prosensa.

(2) Phenylketonuria, or PKU in patients age 18 and older

(3) Pompe disease

(4) CLN2, or late infantile neuronal ceroid lipofuscinosis

(5) In January 2016 the FDA issued a complete response letter to our NDA for Kyndrisa. The FDA concluded that the standard of substantial evidence of effectiveness for Kyndrisa had not been met.

We manage our R&D expense by identifying the research and development 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 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 increased to \$634.8 million for the year ended December 31, 2015, compared to \$461.5 million and \$354.8 million for the years ended December 31, 2014 and 2013, respectively. R&D expense consisted of the following (in millions):

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

	Years Ended December				
	31,			2015	2014
	2015	2014	2013	vs. 2014	vs. 2013
Pegvaliase	\$74.0	\$70.5	\$54.5	\$3.5	\$16.0
Talazoparib <sup>(1)</sup>	65.2	59.8	29.5	5.4	30.3
Reveglucosidase alfa	58.6	51.1	45.6	7.5	5.5
Kyndrisa	49.9	—	—	49.9	—
Vosoritide	49.4	22.5	15.0	26.9	7.5
Vimizim	45.7	63.6	82.0	(17.9)	(18.4)
Cerliponase alfa	39.9	39.6	13.8	0.3	25.8
Other approved products	36.3	31.8	37.3	4.5	(5.5)
Early stage programs	76.9	57.4	24.7	19.5	32.7
Other and non-allocated	138.9	65.2	52.4	73.7	12.8
Total	\$634.8	\$461.5	\$354.8	\$173.3	\$106.7

(1) In October 2015, we sold talazoparib to Medivation. Under the Asset Purchase Agreement, Medivation is responsible for all research, development, regulatory and commercialization activities for all indications on a global basis.

2015 compared to 2014

The increase in talazoparib, reveglucosidase alfa, vosoritide and pegvaliase expense was attributable to increased clinical trial activities related to these product candidates. The development expenses for Kyndrisa relate to clinical and regulatory activities for this product candidate, which was acquired with Prosensa in January 2015. The increase in development expense on early development stage programs was primarily attributable to the pre-clinical activity related to BMN 270 and NAGLU, a novel fusion of alpha-N-acetylglucosaminidase with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo B syndrome, or mucopolysaccharidosis type IIIB (MPS IIIB) (formerly referred to as BMN 250). The increase in non-allocated R&D expense was primarily attributable to an increase in R&D personnel costs and facility costs that are not allocated to specific programs. The increase in R&D personnel costs was attributable to an increase in the number of R&D employees and increased stock-based compensation due to the increase in the number of equity awards outstanding and the weighted-average fair value of the equity awards granted in 2015. Non-allocated R&D expense for the year ended December 31, 2014, included a \$6.1 million gain on early lease termination of our San Rafael Corporate Center (SRCC) lease resulting from the recognition of the remaining deferred rent and asset retirement liabilities upon acquisition of SRCC. There was no similar gain during the year ended December 31, 2015.

During 2016, we expect our R&D spending to increase over 2015 levels due to our Kyndrisa, pegvaliase, reveglucosidase alfa, vosoritide and cerliponase alfa programs progressing in their development. We also expect increased spending on pre-clinical and clinical activities for our early development stage programs BMN 270, NAGLU and other pre-clinical programs. Additionally, we expect to continue incurring significant 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 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 manufacturing activities may 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.

2014 compared to 2013

The increase in development expense on early development stage programs was primarily attributable to the pre-clinical activity related to BMN 270 and NAGLU and development costs related to the other pre-clinical programs. The increase in talazoparib development expense was attributable to increased clinical trial activities related to this product candidate and upfront licensing fees of \$11.5 million paid to a third party to secure licenses related to the development of talazoparib. The increase in pegvaliase development expense was attributable to increased clinical trial activities related to this product candidate. The increases in cerliponase alfa and vosoritide development expense were attributable to increased clinical activities related to these product candidates. The decrease in reveglucosidase alfa development expense was attributable to a decline in clinical manufacturing costs related to the product candidate, offset by an increase in clinical trial expense. The increase in non-allocated R&D expense is primarily attributable to an increase in R&D personnel costs and facility costs that are not allocated to specific programs, which includes a \$6.1 million gain on early lease termination of our SRCC lease resulting from the recognition of the remaining deferred rent and asset retirement liabilities upon acquisition of the SRCC where our corporate headquarters are located. The increase in R&D personnel costs was attributable to an increase in the number of R&D employees and increased stock-based compensation is attributable to an increase in the number of options outstanding due to an increased number of employees, an increase in the weighted-average fair value of the equity awards granted during 2014.

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

## Selling, General and Administrative

SG&A expense increased to \$402.3 million for the year ended December 31, 2015, compared to \$302.2 million and \$235.4 million for the years ended December 31, 2014 and 2013, respectively. SG&A expenses consisted of the following (in millions):

	Years Ended December 31,			2015 vs. 2014	
	2015	2014	2013	2014	2013
Sales and marketing (S&M) expense	\$202.9	\$150.8	\$121.7	\$52.1	\$29.1
General and administrative (G&A) expense	199.4	151.4	113.7	48.0	37.7
Total SG&A expense	\$402.3	\$302.2	\$235.4	\$100.1	\$66.8

	Years Ended December 31,			2015 vs. 2014	
	2015	2014	2013	2014	2013
S&M expense by product:					
Vimizim	\$56.4	\$43.0	\$19.0	\$13.4	\$24.0
Naglazyme	46.8	51.9	53.7	(5.1)	(1.8)
Kuvan	40.7	34.9	31.4	5.8	3.5
Other and non-allocated	59.0	21.0	17.6	38.0	3.4
Total S&M expense	\$202.9	\$150.8	\$121.7	\$52.1	\$29.1

2015 compared to 2014

S&M expense primarily consists of employee-related expenses for our sales group, brand marketing, patient support groups and pre-commercialization expenses related to our product candidates. We received regulatory approval to market Vimizim in the U.S. and the EU during 2014 and subsequently in other countries. The increase in Vimizim S&M expense is consistent with the timing of these approvals and its continued world-wide commercial launch. We continue to incur S&M expense for Naglazyme and Kuvan as a result of continued expansion of our international and U.S. activities, respectively. The increase in other and non-allocated S&M expense was driven by an increase in the number of commercial employees and pre-commercialization expense for Kyndrisa and vosoritide.

G&A expenses primarily consists of corporate support and other administrative expenses, which increased primarily due to increased employee-related expenses as a result of an increase in the number of administrative employees, increased stock based compensation due to the increase in the number of equity awards outstanding and the weighted-average fair value of the equity awards granted in 2015, transaction costs related to the acquisition of Prosensa, consulting fees, legal fees and information technology expenses. G&A expenses for the year ended December 31, 2014, included a \$2.7 million gain on early lease termination of our SRCC lease resulting from the recognition of the remaining deferred rent and asset retirement liabilities upon acquisition of SRCC, which is where our corporate headquarters are located. There was no similar gain during the year ended December 31, 2015.

We expect SG&A expense to increase in future periods as a result of pre-commercialization expense related to product candidates, the international expansion of Naglazyme, Vimizim and Kuvan, and the increase in administrative

support required for our expanding operations.

2014 compared to 2013

The increase Vimizim S&M expense was driven by the regulatory approvals and the product's commercial launch. We continue to incur S&M expense for Naglazyme and Kuvan as a result of continued expansion of our international and U.S. activities, respectively. Other S&M expense primarily consists of employee-related expenses for our sales group, brand marketing, patient support groups and pre-commercialization expenses related to our product candidates.

G&A expenses, which increased primarily due to increased employee-related expenses as a result of an increase in the number of administrative employees, increased stock based compensation due to the increase in the number of equity awards outstanding and the weighted-average fair value of the equity awards granted in 2014 and the recognition of approximately \$10.1 million of expense related to performance awards granted to certain executive officers, consulting fees, and information technology expenses. The increase in corporate support and other administrative expenses is primarily attributable to increases in employee related expenses due to the increase in commercial and administrative headcount, professional service fees related to the acquisition of Prosensa, consulting fees, and information and technology expenses, which were offset by a \$2.7 million gain on the early termination of our SRCC lease

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

and the recognition of the remaining deferred rent and asset retirement liabilities upon acquisition of the SRCC.

## 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,		
	2015	2014	2013
Increases (decreases) in the fair value of contingent acquisition			
consideration payable	\$(28.5)	\$13.0	\$14.5
Amortization of intangible assets	10.8	10.7	10.5
Total intangible asset amortization and contingent consideration	\$(17.7)	\$23.7	\$25.0

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. During the year ended December 31, 2015, the majority of the changes related to the development progress of Kyndrisa that included the reversal of the liability for the contingent value rights payable upon FDA approval by a specified time. Based on the current U.S. development efforts, management determined the U.S. approval milestone would not be attained by May 15, 2016 as required under the terms of our agreement with the former Prosensa stockholders. During the year ended December 31, 2014, the majority of the changes related to the development progress of reveglucosidase alfa.

## Impairment of Intangible Asset

We recorded an impairment charge of \$198.7 million related to the Kyndrisa IPR&D assets based on the 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.

## Gain on Sale of Intangible Asset

We recognized a net gain of \$369.5 million for the sale of talazoparib to Medivation in the year ended December 31, 2015. See Note 8 to the accompanying Consolidated Financial Statements for additional information for additional discussion.

## Interest Income

We invest our cash, short-term and long-term investments in U.S. government securities and other high credit quality securities in order to limit default and market risk. Interest income totaled \$4.5 million for the year ended December 31, 2015, compared to \$5.9 million and \$3.1 million for the years ended December 31, 2014 and 2013, respectively. The decrease in interest income during the year ended December 31, 2015, as compared to the years ended December 31, 2014 and 2013 was primarily due to lower yields on our investments. We do not expect interest income to fluctuate significantly over the next twelve months.

## Interest Expense and Debt Conversion Expense

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

	Years Ended		
	December 31,		
	2015	2014	2013
Coupon interest	\$9.8	\$9.4	\$4.5
Amortization of debt issuance costs	3.3	3.3	1.1
Accretion of discount on convertible notes	25.2	23.9	4.8
Total interest expense	\$38.3	\$36.6	\$10.4

Interest expense for the years ended December 31, 2015 and 2014 was primarily attributable to our October 2013 issuance of \$750.0 million in aggregate principal amount of senior subordinated convertible debt of which \$375.0 million is due in October 2018 and \$375.0 million is due in October 2020. The increased interest expense in the year ended December 31, 2015, compared to the year ended December 31, 2014 is attributed to an increase in the accretion of the discount on our October 2013 issuance using the effective interest rate method. The increased interest expense in the year ended December 31, 2014, compared to the year ended December 31,

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

2013 was attributable to our October 2013 issuance of \$750.0 million in aggregate principal amount of senior subordinated convertible debt. We do not expect interest expense to fluctuate significantly over the next 12 months. See Note 14 to the accompanying Consolidated Financial Statements for additional information regarding our Convertible Debt.

During 2015, we recognized Debt Conversion Expense of \$0.2 million in connection with the early conversion of \$8.1 million in aggregate principal amount of our senior subordinated convertible notes due in April 2017 (the 2017 Notes), compared to the year ended December 31, 2014 when we recognized Debt Conversion Expense of \$0.7 million in connection with the early conversion of \$16.5 million in aggregate principal amount of the 2017 Notes. During the year ended December 31, 2013, we recognized debt conversion expense of \$13.0 million in connection with the early conversion of \$262.8 million in aggregate principal amount of the 2017 Notes.

#### Provision for (Benefit from) Income Taxes

For the year ended December 31, 2015 we recognized income tax expense of \$17.1 million, compared to income tax expense of \$9.1 million and an income tax benefit of \$0.2 million in the years ended December 31, 2014 and 2013, respectively. Provision for (benefit from) income taxes for 2015, 2014 and 2013 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, 2015, 2014 and 2013 were further reduced by the following discrete items:

- 2015 deferred tax benefit of \$49.7 million associated with the GAAP impairment of the Kyndrisa IPR&D that was 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.
- 2014 a renewable energy investment tax credit under the flow-through method.
- The 2013 deferred tax benefits were offset by a \$1.6 million increase in the valuation allowance related to California net operating losses that we believe are likely to expire unutilized.

During all periods, the federal R&D credit was reinstated retroactively. In accordance with ASC Topic 740, Income Taxes (ASC 740), we accounted for the effects of change in the tax law in the period that included the enactment date of the change, resulting in the recognition of a deferred tax benefit related to R&D expenses incurred during the reinstatement period. See Note 16 to the accompanying Consolidated Financial Statements for additional information regarding the components of our Provision for (Benefit from) Income Taxes.

The consolidated U.S. GAAP loss includes all of our foreign subsidiaries. In accordance with ASC 740, 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 research and development 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 research and development 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 \$234.8 million of foreign net losses. Dutch operations had a GAAP loss of \$106.0 million, which included the impairment of the Kyndrisa IPR&D and a resulting deferred tax benefit of \$49.7 million associated with the reversal of the deferred tax liability of such IPR&D. Other foreign operations generated U.S. GAAP income of approximately \$3.9 million with an effective tax rate of approximately 82%.

#### Financial Position, Liquidity and Capital Resources

We expect to fund our operations with our net product revenues from our commercial products, cash, cash equivalents, short-term and long-term investments, supplemented by proceeds from equity or debt financings and loans or collaborative agreements with corporate partners, each to the extent necessary. This expectation could change depending on 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. 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.

We do not record U.S. tax expense on the undistributed earnings of our controlled foreign subsidiaries as these earnings are intended to be permanently reinvested offshore. At December 31, 2015, the cumulative amount of these earnings was approximately \$1.7 million.

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

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

provided for U.S. federal or state income taxes on these undistributed foreign earnings. As of December 31, 2015, \$186.5 million of our \$1,018.3 million balance of cash, cash equivalents and marketable securities was held in foreign subsidiaries, a significant portion of which is required to fund the liquidity needs of these foreign subsidiaries. See Note 16 to the 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. Some of the factors that could affect our business include: future changes to healthcare reform in the U.S., a continuation of uncertainty with respect to, or worsening of global economic conditions, patent expirations of competitive products and the launch of generic competitors, continued government pricing pressures internationally and the potential volatility in foreign currency exchange rates. We will continue to monitor these conditions and will attempt to adjust our business processes, as appropriate, to mitigate these risks to our business.

Our liquidity and capital resources as of December 31 were as follows (in millions):

	2015	2014	2013	2015 vs. 2014	2014 vs. 2013
Cash and cash equivalents	\$397.0	\$875.5	\$568.8	\$(478.5)	\$306.7
Short-term investments	195.6	69.7	215.9	125.9	(146.2)
Long-term investments	425.7	97.9	267.7	327.8	(169.8)
Cash, cash equivalents and investments	\$1,018.3	\$1,043.1	\$1,052.4	\$(24.8)	\$(9.3)
Current assets	\$1,089.6	\$1,397.6	\$1,103.7	\$(308.0)	\$293.9
Current liabilities	445.5	235.7	183.3	209.8	52.4
Working capital	\$644.1	\$1,161.9	\$920.4	\$(517.8)	\$241.5
Convertible debt, net	\$662.3	\$642.9	\$637.0	\$19.4	\$5.9

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

	2015	2014	2013	2015 vs. 2014	2014 vs. 2013
Cash & cash equivalents at the beginning of the period	\$875.5	\$568.8	\$180.5	\$306.7	\$388.3
Net cash used in operating activities	(221.7)	(71.9)	(57.3)	(149.8)	(14.6)
Net cash provided by (used in) investing activities	(1,179.6)	196.3	(298.9)	(1,375.9)	495.2
Net cash provided by financing activities	927.9	185.7	746.7	742.2	(561.0)
Foreign exchange impact	(5.1)	(3.4)	(2.2)	(1.7)	(1.2)
Cash & cash equivalents at the end of the period	\$397.0	\$875.5	\$568.8	\$(478.5)	\$306.7
Short-term and long-term investments	621.3	167.6	483.6	453.7	(316.0)
Cash, cash equivalents and investments	\$1,018.3	\$1,043.1	\$1,052.4	\$(24.8)	\$(9.3)
Working Capital					

Working capital decreased by \$517.8 million, from \$1,161.9 million at December 31, 2014 to \$644.1 million at December 31, 2015. The decrease in working capital was attributable to the following (in millions):

Working capital at December 31, 2014	\$1,161.9
Decreased cash, cash equivalents and short-term investments	(352.6)
Increased current liabilities	(209.7)
Net increase in other current operating assets	44.5

Working capital at December 31, 2015	\$644.1
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The decrease in cash, cash equivalents and short-term investments was primarily attributable to \$538.4 million net cash used to acquire Prosensa, \$221.7 million of cash used to fund operating activities, \$227.7 million net cash invested in property, plant, and equipment, a \$371.8 million deposit for the acquisition of the global rights to Kuvan and pegvaliase, excluding Japan, and the net purchase of \$327.8 million of long-term investments, partially offset by net proceeds of \$888.3 million from our January 2015 public offering of common stock, \$410.0 million in proceeds from the sale of talazoparib and \$40.1 million of net proceeds from employee equity transactions. The increase in current liabilities primarily consisted of a \$49.1 million increase in short-term contingent acquisition consideration payable to the former Prosensa stockholders for the EU approval of Kyndrisa, a \$39.8 million increase in



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

accounts payable and accrued operating expenses, a \$32.9 million increase in accrued compensation and a \$17.7 million increase in accrued rebates payable. The net increase in other operating assets consisted of increased accounts receivable and inventory, offset by a decrease in other current assets. The increase in accounts receivable was attributable to increased revenues and the timing of net product revenues and cash receipts from customers. The increase in inventory was primarily attributable to building of inventories for all commercial products to meet anticipated future sales demand. The decrease in other current assets was primarily attributable to the absence of a promissory note receivable in 2015 that was outstanding at December 31, 2014.

The increase in cash, cash equivalents and short-term investments at December 31, 2014 from December 31, 2013 was primarily attributable to the net proceeds of \$117.5 million from our March 2014 public offering of common stock, net proceeds of million \$72.1 from employee equity transactions and \$67.5 million net proceeds from the 2014 sale of a Priority Review Voucher (PRV).

Our product sales to government-owned or government-funded customers in certain countries, including Russia, Greece, Spain, Italy and Portugal, are subject to payment terms that are imposed by government authorities. Because these customers are government-owned or government-funded, we may be impacted by declines in sovereign credit ratings or sovereign defaults in these countries. A significant or further decline in sovereign credit ratings, or default in these countries, may decrease the likelihood that we will collect accounts receivable or may increase the discount rates and the length of time until receivables are collected, which could result in a negative impact to our operating results. Historically we have not experienced a significant level of uncollected receivables and have received continued payments from our more aged accounts. We believe that the allowances for doubtful accounts for these countries are adequate based on our analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries. As of December 31, 2015, approximately 9% of our outstanding accounts receivable relate to such countries. See Note 20 to the accompanying Consolidated Financial Statements for additional discussion. We also sell our products in other countries that face economic crises and local currency devaluation. Although we have historically collected receivables from customers in those countries, sustained weakness or further deterioration of the local economies and currencies may cause our customers in those countries to be unable to pay for our products with the same negative effect on our operations.

#### Cash Used in Operating Activities

Cash used in operating activities for the year ended December 31, 2015 was \$221.7 million, compared to cash used in operating activities of \$71.9 million for the year ended December 31, 2014. Our net loss in the year ended December 31, 2015 excluding the \$369.5 million gain on the sale of talazoparib and the \$211.5 million asset impairment charges increased \$128.3 million compared to our net loss for the year ended December 31, 2014 excluding the \$67.5 million net gain on the sale of the PRV. The increase in cash used in operating activities is primarily attributable to increased R&D expense related to clinical trial activities for Kyndrisa and vosoritide and increased inventory purchases.

Cash used in operating activities for the year ended December 31, 2014 was \$71.9 million, compared to cash used in operating activities of \$57.3 million for the year ended December 31, 2013. Our net loss in the year ended December 31, 2014 excluding the \$67.5 million net gain from the sale of the PRV increased \$25.1 million compared to our net loss in the year ended December 31, 2013. The increase in our net loss is primarily attributable to increased R&D expense related to increased clinical trial activities for our product candidates: pegvaliase, talazoparib and reveglucosidase alfa and increased sales and marketing expense related to the commercial launch of Vimizim in the U.S. and the EU and a decrease in cash collected from accounts receivable.

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

Net cash used in investing activities during the year ended December 31, 2015 was \$1,179.6 million, compared to net cash provided by investing activities of \$196.3 million and net cash used in investing activities of \$298.9 million during the years ended December 31, 2014 and 2013, respectively. The increase in net cash used in investing activities for the year ended December 31, 2015 compared to the year ended December 31, 2014 primarily consisted of the \$538.4 million paid to acquire Prosensa, a \$371.8 million deposit to acquire the global rights to Kuvan and pegvaliase, with the exception of Kuvan Japan, a \$110.6 million increase in the purchases of property, plant and equipment and a \$749.7 million increase in net purchases of available-for-sale securities. Those increases were partially offset by a \$342.5 million increase in proceeds related to the sale of intangible assets. The increase in net cash provided by investing activities for the year ended December 31, 2014 compared to the year ended December 31, 2013 primarily consisted of a \$407.7 million increase in net maturities of investments, the \$67.5 million in proceeds for the sale of the PRV, a \$116.5 million decrease in restricted funds held in escrow for the purchase of the SRCC and a \$9.9 million decrease in business acquisitions, offset by a \$51.9 million increase in capital expenditures and a \$52.3 million increase in the investment in convertible promissory notes. During 2016, we expect to make significant capital investments in our manufacturing facilities and our corporate headquarters to accommodate anticipated headcount growth.

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

Cash Provided by Financing Activities

Net cash provided by financing activities for the year ended December 31, 2015 was \$927.9 million, compared to net cash provided by financing activities of \$185.7 million and \$746.7 million for the years ended December 31, 2014 and 2013, respectively. The increase in net cash provided by financing activities for the year ended December 31, 2015 was primarily attributable to a \$770.8 million increase in net proceeds from our January 2015 equity offering compared to the March 2014 equity offering, offset by a \$32.1 million decrease in proceeds from employee equity transactions. The decrease in net cash provided by financing activities for the year ended December 31, 2014 was primarily attributable to of the net proceeds of \$117.5 million from our March 2014 equity offering, a \$12.3 million decrease in debt conversion expense and increased net proceeds from equity transactions of \$6.0 million, partially offset by a \$1.6 million increase in payments of contingent acquisition consideration.

Other Information

Our \$781.4 million (undiscounted) of total convertible debt as of December 31, 2015 will impact our liquidity due to the semi-annual cash interest payments and will further impact our liquidity if we elect to settle all or portions of the 2018 Notes or the 2020 Notes in cash upon conversion or if the holders of our 2017 Notes do not convert on or prior to the scheduled repayments of the debt. 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 or cash equivalents to repurchase our convertible debt or other securities.

On January 15, 2015, we closed the initial offering period related to our offer to purchase all of the ordinary shares of Prosensa (Prosensa Shares), a public limited liability company organized under the laws of the Netherlands, purchasing 93.4% of the Prosensa Shares and immediately launched a subsequent offering period that expired on January 29, 2015. As of the expiration of the subsequent offering period, we paid \$620.7 million for approximately 35 million Prosensa Shares, representing 96.8% of all the outstanding Prosensa Shares. Additionally, we paid approximately \$38.6 million for the options that vested pursuant to the definitive purchase agreement. On February 12, 2015, we completed an asset transfer and we paid \$20.8 million to the remaining Prosensa shareholders. Effective February 12, 2015, Prosensa has been dissolved and was liquidated in January 2016 under Dutch law.

On January 27, 2015, we sold 9.8 million shares of our common stock at a price of \$93.25 per share in an underwritten public offering pursuant to an effective registration statement previously filed with the SEC. We received net proceeds of approximately \$888.3 million from this public offering after accounting for the underwriting discount and offering costs.

On October 6, 2015, we completed the sale of talazoparib to Medivation, under which Medivation acquired the worldwide rights to talazoparib in exchange for payment of \$410.0 million and up to an additional \$160.0 million upon the achievement of regulatory and sales-based milestones and mid-single digit percentage royalties for talazoparib.

In October 2015, we entered into entered into a definitive agreement to acquire all global rights to Kuvan and pegvaliase from Merck Serono, with the exception of Kuvan in Japan. In December 2015, we paid a deposit on this transaction totaling \$371.8 million. Under the terms of the agreement we also agreed to pay Merck Serono additional consideration in future periods up to a maximum of €60.0 million in milestone payments if certain Kuvan sales milestones are met and up to a maximum of €125.0 million in milestone payments if certain pegvaliase development milestones are met. Our agreement with Merck Serono was effective January 1, 2016.

## 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 drugs, or if approval is delayed, we will be unable to generate revenue from the sale of these products, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will be increased;
- If we are unable to successfully develop and maintain manufacturing processes for our drug 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.

## 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 products 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 major programs were as follows (in millions):

	Since Program Inception
Vimizim	\$ 403.1
Talazoparib <sup>(1)</sup>	181.6
Reveglucosidase alfa	206.9
Vosoritide	118.8
Cerliponase alfa	111.0
Pegvaliase	312.2
Kyndrisa	49.9
Other approved products	439.6
Other and non-allocated	Not meaningful

(1) In October 2015, we sold talazoparib to Medivation. Under the Asset Purchase Agreement, Medivation will be responsible for all research, development, regulatory and commercialization activities for all indications on a global basis.

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 Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse; preclinical 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 Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- manufacturing, supply or distribution of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- progress of our integration of the PKU franchise rights acquired from Merck Serono;
- 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;
- results relating to our lawsuits against Par to protect our patents relating to Kuvan and generic competition to Kuvan relating to our settlement with DRL;
- government regulatory action affecting our product candidates or our competitors' drug 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;
- broad market fluctuations in the U.S., the EU or in other parts of the world;
- actual or anticipated fluctuations in our operating results;

- changes in company assessments or financial estimates by securities analysts; and
- sales of our shares of stock by us, our significant stockholders, or members of our management or Board of Directors.

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

## Off-Balance Sheet Arrangements

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

## Contractual and Commercial Obligations

We have contractual and commercial obligations under our 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, 2015 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	
2017 Notes and related interest	\$0.6	\$31.7	\$—	\$—	\$32.3
2018 Notes and related interest	2.8	380.6	—	—	383.4
2020 Notes and related interest	5.6	11.2	386.2	—	403.0
Operating leases	7.2	12.0	5.3	10.0	34.5
R&D and purchase commitments	42.4	6.4	2.2	—	51.0
Total	\$58.6	\$441.9	\$393.7	\$ 10.0	\$904.2

We are also subject to contingent payments totaling approximately \$675.4 million as of December 31, 2015 that are due upon achievement of certain regulatory and licensing milestones if they occur before certain dates in the future. Of this amount, \$80.0 million relates to the acquisition of Prosensa and \$22.7 million relates to programs that are no longer being developed. The total contingent payments referenced above, do not include the contingent payments of up to €185.0 million payable to Merck Serono pursuant to our acquisition of the global rights for Kuvan and pegvaliase, which closed in January 2016, if certain Kuvan sales milestones and pegvaliase regulatory and sales milestones are attained.

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 countries outside the U.S, including Europe and Brazil. As a result our financial results can 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, British Pound and Brazilian Real. 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 2015, approximately 33% of our net product sales were denominated in foreign currencies and 25% of our operating expenses were denominated foreign currencies. To partially mitigate the impact of changes in currency exchange rates on net cash flows from our Euro-denominated sales and foreign currency denominated operating expenses, we may enter into forward foreign currency contracts. We also hedge certain monetary assets and liabilities 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, 2015 and 2014, we had open forward foreign currency exchange contracts with notional amounts of \$260.9 million and \$264.6 million, respectively. A hypothetical 10% strengthening in foreign currency exchange rates compared with the U.S. dollar relative to exchange rates at December 31, 2015 would have resulted in a reduction in the value received over the remaining life of these contracts of approximately \$25.5 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, 2014, would have resulted in a reduction of the value received over the remaining life of the contracts by approximately \$24.1 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, 2015, 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 open forward foreign currency exchange contracts, and investments by approximately \$1.0 million. We expect to enter into new 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.

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 \$31.4 million of the 2017 Notes, \$375.0 million of the 2018 Notes and \$375.0 million of the 2020 Notes. The interest rates on these notes are fixed and therefore they do not expose us to risk related to rising interest rates. At December 31, 2015 the fair value of our convertible debt was \$1,147.3 million.

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 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, 2015, our investment portfolio did not include any investments with significant exposure to the subprime mortgage market issues or the European debt crisis. Based on our investment portfolio and interest rates at December 31, 2015, 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 \$7.8 million. Changes in interest rates may affect the fair value of our investment portfolio. However, we will not recognize such gains or losses in our Consolidated Statement 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, 2015 (in millions):

	Expected Maturity						Total
	2016	2017	2018	2019	2020	Thereafter	
Available-for-sale securities	\$195.6	\$261.1	\$140.7	\$16.9	\$5.9	\$ 1.1	\$621.3
Average interest rate	0.5 %	1.2 %	1.6 %	1.5 %	2.1 %	0.6 %	1.1 %

Market price sensitive financial instruments

As of December 31, 2015 and 2014, we were also exposed to price risk on equity securities included in our portfolio of investments, which were acquired primarily for the promotion of business and strategic objectives. These strategic investments are generally in small capitalization stocks in the biotechnology industry sector. Price risk relative to our equity investment portfolio as of December 31, 2015 and 2014, was not material.

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 and requires placing exposure limits on the amount with any individual counterparty. 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 on pages F-1 to F-46 of this report.

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 are effective to ensure that the information required to be disclosed by us in the reports we file or submit under the Exchange Act was recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms.

##### 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

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in Rule 13a-15(f) under the Exchange Act as of December 31, 2015. 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, 2015 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.

#### Scope of the Effectiveness of Controls

Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with GAAP. Our internal control over financial reporting includes those policies and procedures that:

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

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions or that the degree of compliance with the policies or procedures may deteriorate.

#### Item 9B. Other Information

None



Part III

Item 10. Directors, Executive Officers and Corporate Governance

The information required by this Item regarding our directors, executive officers and corporate governance is incorporated into this section by reference to the sections captioned “Election of Directors” and “Executive Officers” in the proxy statement for our 2016 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 2016 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” in the proxy statement for our 2016 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 2016 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 section captioned “Transactions with Related Persons, Promoters and Certain Control Persons” in the proxy statement for our 2016 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 2016 annual meeting of stockholders.

Part IV

Item 15. Exhibits, Financial Statement Schedules

Financial Statements

	Page
<u>Reports of Independent Registered Public Accounting Firm</u>	F-2
Consolidated Financial Statements as of December 31, 2015 and 2014 and for the three years ended December 31, 2015:	
<u>Consolidated Balance Sheets</u>	F-4
<u>Consolidated Statements of Operations</u>	F-5
<u>Consolidated Statements of Comprehensive Loss</u>	F-6
<u>Consolidated Statements of Changes in Stockholders' Equity</u>	F-7
<u>Consolidated Statements of Cash Flows</u>	F-8
<u>Notes to Consolidated Financial Statements</u>	F-9



Exhibit Index

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 by reference herein.

2.2 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.

2.3\* Amended and Restated Termination and Transition Agreement, dated as of December 23, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A. 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 Agreement, dated as of October 1, 2015, between

BioMarin  
Pharmaceutical  
Inc. and Ares  
Trading S.A.  
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\* Termination  
and Transition  
Agreement,  
dated as of  
October 1,  
2015, between  
BioMarin  
Pharmaceutical  
Inc. and Ares  
Trading S.A.  
Portions of this  
exhibit  
(indicated by  
asterisks) have  
been omitted  
pursuant to a  
request for  
confidential  
treatment.  
Omitted  
portions have  
been filed  
separately with  
the SEC.

3.1 Amended and  
Restated  
Certificate of  
Incorporation  
of BioMarin  
Pharmaceutical  
Inc., as

amended  
June 12, 2003,  
previously filed  
with the SEC  
on June 23,  
2003 as Exhibit  
3.1 to the  
Company's  
Current Report  
on Form 8-K  
(File No.  
000-26727),  
which is  
incorporated  
herein by  
reference.

3.2 Certificate of  
Correction to  
Certificate of  
Amendment to  
the Amended  
and Restated  
Certificate of  
Incorporation  
of BioMarin  
Pharmaceutical  
Inc., dated  
April 4, 2005,  
previously filed  
with the SEC  
on April 5,  
2005 as Exhibit  
3.2 to the  
Company's  
Current Report  
on Form 8-K  
(File No.  
000-26727),  
which is  
incorporated  
herein by  
reference.

3.3 Certificate of  
Amendment to  
the Amended  
and Restated  
Certificate of  
Incorporation  
of BioMarin

Pharmaceutical  
Inc. as filed  
with the  
Delaware  
Secretary of  
State on  
October 12,  
2007,  
previously filed  
with the SEC  
on February 22,  
2012 as Exhibit  
3.3 to the  
Company's  
Annual Report  
on Form 10-K  
(File No.  
000-26727),  
which is  
incorporated  
herein by  
reference.

3.4 Amended and  
Restated  
Bylaws of  
BioMarin  
Pharmaceutical  
Inc., previously  
filed with the  
SEC on June  
15, 2015 as  
Exhibit 3.1 to  
the Company's  
Current Report  
on Form 8-K  
(File No.  
000-26727),  
which is  
incorporated  
herein by  
reference.

4.1 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, 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,  
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 Form of 0.75%  
Senior  
Subordinated  
Convertible  
Notes due 2018,  
previously filed  
with the SEC on  
October 15, 2013  
as included in  
Exhibit 4.2 to the  
Company's  
Current Report  
on Form 8-K  
(File No.  
000-26727),  
which is  
incorporated  
herein by  
reference.

4.8 Form of 1.50%  
Senior  
Subordinated  
Convertible  
Notes due 2020,  
previously filed  
with the SEC on  
October 15, 2013  
as included in  
Exhibit 4.3 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  
October 19, 2010  
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† Amended and  
Restated  
Severance Plan  
and Summary  
Plan Description  
as originally  
adopted on  
January 27, 2004  
and amended  
and restated on  
May 12, 2009  
and further  
amended and  
restated on July  
29, 2013 and  
October 7, 2014,  
previously filed  
with the SEC on  
October 14, 2014  
as Exhibit 10.1  
to the Company's  
Current Report  
on Form 8-K  
(File No.  
000-26727),  
which is  
incorporated by  
reference herein.

10.3† Amendment to  
BioMarin  
Pharmaceutical  
Inc. 1997 Stock  
Plan, as  
amended, as  
adopted March  
20, 2002,  
previously filed  
with the SEC on  
March 21, 2002  
as Exhibit 99.1

to the Company's  
Current Report  
on Form 8-K  
(File No.  
000-26727),  
which is  
incorporated  
herein by  
reference.

10.4† Amendment No.  
2 to BioMarin  
Pharmaceutical  
Inc. 1997 Stock  
Plan, as  
amended, as  
adopted May 5,  
2004, previously  
filed with the  
SEC on  
August 9, 2004  
as Exhibit 10.1  
to the Company's  
Quarterly Report  
on Form 10-Q  
(File No.  
000-26727),  
which is  
incorporated  
herein by  
reference.

10.5† BioMarin  
Pharmaceutical  
Inc. 1998  
Director Option  
Plan and forms  
of agreements  
thereunder,  
previously filed  
with the SEC on  
May 4, 1999 as  
Exhibit 10.3 to  
the Company's  
Registration  
Statement on  
Form S-1 (File  
No. 333-77701),  
which is  
incorporated  
herein by

reference.

10.6† Amendment No.  
1 to BioMarin  
Pharmaceutical  
Inc. 1998  
Director Plan as  
adopted  
March 26, 2003  
previously filed  
with the SEC on  
May 15, 2003 as  
Exhibit 10.1 to  
the Company's  
Quarterly Report  
on Form 10-Q  
(File No.  
000-26727),  
which is  
incorporated  
herein by  
reference.

10.7† Amendment No.  
2 to BioMarin  
Pharmaceutical  
Inc. 1998  
Director Option  
Plan, effective as  
of June 12, 2003  
and July 21,  
2003, previously  
filed with the  
SEC on August  
12, 2003 as  
Exhibit 10.1 to  
the Company's  
Quarterly report  
on Form 10-Q  
(File No.  
000-26727),  
which is  
incorporated  
herein by  
reference.

10.8† Amendment No.  
3 to BioMarin  
Pharmaceutical  
Inc. 1998  
Director Option

Plan, as amended, as adopted May 5, 2004, previously filed with the SEC on August 9, 2004 as Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference.

10.9† 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.

10.10† 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..

10.11†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.

10.12†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.

10.13†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.

10.14†Amended and  
Restated  
Employment  
Agreement with  
Jean-Jacques  
Bienaimé  
effective  
January 1, 2009  
previously filed  
with the SEC on  
December 23,  
2008 as Exhibit

10.1 to the  
Company's  
Current Report  
on Form 8-K  
(File No.  
000-26727),  
which is  
incorporated  
herein by  
reference.



10.15 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.

10.16 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.

10.17 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.

10.18 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.

10.19 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.

10.20 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.

10.21†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.

10.22†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.

10.23†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.

10.24†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.

10.25†Amendment No. 1

to the Amended and  
Restated  
Employment  
Agreement with  
Jean-Jacques  
Bienaimé dated as  
of December 17,  
2012, previously  
filed with the SEC

on December 18,  
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.

10.26 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.

10.27 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.

10.28 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.

10.29 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.

10.30 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.

10.31 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.

10.32 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.

10.33

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.

10.34

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.

10.35

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.

10.36

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.

10.37

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.

10.38

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.

10.39 Asset Purchase Agreement, between BioMarin Pharmaceutical Inc., BioMarin GALNS Ltd. and Regeneron Ireland dated July 29, 2014, previously filed with the SEC on October 28, 2014 as Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q (File No. 000-26727), which is incorporated herein by reference.

10.40 Form of Tender and Support Agreement by and among BioMarin Pharmaceutical Inc.,

BioMarin Falcons B.V.  
and shareholders of  
Prosensa Holding N.V.,  
previously filed with  
the SEC on November  
26, 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.

10.41 Convertible Promissory  
Note, dated as of  
November 26, 2014,  
between Prosensa  
Holding N.V. and  
BioMarin Falcons B.V.,  
previously filed as  
Exhibit 10.3 to the  
Company's Current  
Report on Form 8-K  
(File No. 000-26727),  
which is incorporated  
herein by reference.

10.42 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.

10.43 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' Current Report on Form 8-K (File No. 000-26727), which is incorporated by reference herein.

10.44 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.

- 10.45† 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.
- 10.46† 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.
- 10.47 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.

- |       |   |
|-------|---|
| 21.1* | Subsidiaries of<br>BioMarin<br>Pharmaceutical Inc.  |
| 23.1* | Consent of KPMG<br>LLP, Independent<br>Registered Public<br>Accounting Firm for<br>BioMarin<br>Pharmaceutical Inc.  |
| 24.1* | Power of Attorney<br>(Included in<br>Signature Page to<br>this Report)  |
| 31.1* | Certification of<br>Chief Executive<br>Officer pursuant to<br>Rules<br>13a-14(a)/15d-14(a)<br>of the Securities<br>Exchange Act of<br>1934, as amended.   |
| 31.2* | Certification of<br>Chief Financial<br>Officer pursuant to<br>Rules<br>13a-14(a)/15d-14(a)<br>of the Securities<br>Exchange Act of<br>1934, as amended.   |
| 32.1* | Certification of<br>Chief Executive<br>Officer and Chief<br>Financial Officer<br>pursuant to 18<br>U.S.C. Section 1350,<br>as adopted pursuant<br>to Section 906 of the<br>Sarbanes-Oxley Act<br>of 2002. This<br>Certification<br>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

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 29, 2016 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 29, 2016
/S/ DANIEL SPIEGELMAN Daniel Spiegelman	Executive Vice President and Chief Financial Officer (Principal Financial Officer)	February 29, 2016
/S/ BRIAN R. MUELLER Brian R. Mueller	Group Vice President, Corporate Controller and Chief Accounting Officer (Principal Accounting Officer)	February 29, 2016
/S/ PIERRE LAPALME Pierre Lapalme	Director	February 29, 2016
/S/ MICHAEL G. GREY Michael G. Grey	Director	February 29, 2016
/S/ ELAINE HERON Elaine Heron	Director	February 29, 2016
/S/ V. BRYAN LAWLIS V. Bryan Lawlis	Director	February 29, 2016
/S/ ALAN J. LEWIS Alan J. Lewis	Director	February 29, 2016
/S/ RICHARD A. MEIER Richard A. Meier	Lead Independent Director	February 29, 2016
/S/ DAVID PYOTT David Pyott	Director	February 29, 2016
/S/ DENNIS J. SLAMON	Director	

Dennis J. Slamon

February 29,  
2016

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BIOMARIN PHARMACEUTICAL INC.

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Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders

BioMarin Pharmaceutical Inc.:

We have audited the accompanying consolidated balance sheets of BioMarin Pharmaceutical Inc. and subsidiaries as of December 31, 2015 and 2014, and 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, 2015. 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 conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the financial position of BioMarin Pharmaceutical Inc. and subsidiaries as of December 31, 2015 and 2014, and the results of their operations and their cash flows for each of the years in the three year period ended December 31, 2015, 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), BioMarin Pharmaceutical Inc.'s internal control over financial reporting as of December 31, 2015, based on criteria established in Internal Control – Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO), and our report dated February 29, 2016 expressed an unqualified opinion on the effectiveness of the Company's internal control over financial reporting.

/s/ KPMG LLP

San Francisco, California  
February 29, 2016

Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders

BioMarin Pharmaceutical Inc.:

We have audited BioMarin Pharmaceutical Inc. and subsidiaries' (the Company) internal control over financial reporting as of December 31, 2015, based on criteria established in Internal Control – Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). 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 conducted our audit in accordance with the standards of the Public Company Accounting Oversight Board (United States). 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 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.

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.

In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2015, based on criteria established in Internal Control – Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO).

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the consolidated balance sheets of BioMarin Pharmaceutical Inc. and subsidiaries as of December 31, 2015 and 2014, and 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, 2015, and our report dated February 29, 2016 expressed an unqualified opinion on those consolidated financial statements.



/s/ KPMG LLP

San Francisco, California  
February 29, 2016

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## BIOMARIN PHARMACEUTICAL INC.

## CONSOLIDATED BALANCE SHEETS

December 31, 2015 and 2014

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

	December 31, 2015	December 31, 2014
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$397,040	\$875,486
Short-term investments	195,579	69,706
Accounts receivable, net (allowance for doubtful accounts: \$93 and \$490, at December 31, 2015 and 2014, respectively)	164,959	144,472
Inventory	271,683	199,452
Other current assets	60,378	108,524
<b>Total current assets</b>	<b>1,089,639</b>	<b>1,397,640</b>
Noncurrent assets:		
Long-term investments	425,652	97,856
Property, plant and equipment, net	704,207	523,516
Intangible assets, net	683,996	156,578
Goodwill	197,039	54,258
Deferred tax assets	220,191	190,974
Other assets	408,644	54,557
<b>Total assets</b>	<b>\$3,729,368</b>	<b>\$2,475,379</b>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable and accrued liabilities	\$392,511	\$231,844
Short-term contingent acquisition consideration payable	52,946	3,895
<b>Total current liabilities</b>	<b>445,457</b>	<b>235,739</b>
Noncurrent liabilities:		
Long-term convertible debt, net	662,286	642,902
Long-term contingent acquisition consideration payable	32,663	38,767
Long-term deferred tax liabilities	143,527	—
<b>Other long-term liabilities</b>	<b>44,588</b>	<b>30,077</b>
<b>Total liabilities</b>	<b>1,328,521</b>	<b>947,485</b>
Stockholders' equity:		
Common stock, \$0.001 par value: 250,000,000 shares authorized at December 31, 2015 and 2014: 161,526,044 and 149,093,647 shares issued and outstanding at December 31, 2015 and 2014, respectively.		
	162	149

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Additional paid-in capital	3,414,837	2,359,744
Company common stock held by Nonqualified Deferred Compensation Plan	(13,616 )	(9,695 )
Accumulated other comprehensive income	21,033	27,466
Accumulated deficit	(1,021,569)	(849,770 )
Total stockholders' equity	2,400,847	1,527,894
Total liabilities and stockholders' equity	\$3,729,368	\$2,475,379

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, 2015, 2014 and 2013

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

	2015	2014	2013
<b>REVENUES:</b>			
Net product revenues	\$884,522	\$738,416	\$538,360
Collaborative agreement revenues	1,018	1,592	3,918
Royalty, license and other revenues	4,355	9,276	6,207
Total revenues	889,895	749,284	548,485
<b>OPERATING EXPENSES:</b>			
Cost of sales (excludes amortization of intangible assets)	152,008	122,267	88,391
Research and development	634,806	461,543	354,780
Selling, general and administrative	402,271	302,156	235,356
Intangible asset amortization and contingent consideration	(17,690 )	23,709	25,026
Impairment of intangible asset	198,700	—	939
Gain on sale of intangible asset	(369,498 )	(67,500 )	—
Total operating expenses	1,000,597	842,175	704,492
<b>LOSS FROM OPERATIONS</b>	<b>(110,702 )</b>	<b>(92,891 )</b>	<b>(156,007 )</b>
Equity in the loss of BioMarin/Genzyme LLC	(817 )	(877 )	(1,149 )
Interest income	4,501	5,937	3,083
Interest expense	(38,244 )	(36,642 )	(10,447 )
Debt conversion expense	(163 )	(674 )	(12,965 )
Other income (expense)	(9,299 )	279	982
<b>LOSS BEFORE INCOME TAXES</b>	<b>(154,724 )</b>	<b>(124,868 )</b>	<b>(176,503 )</b>
Provision for (benefit from) income taxes	17,075	9,101	(150 )
<b>NET LOSS</b>	<b>\$(171,799 )</b>	<b>\$(133,969 )</b>	<b>\$(176,353 )</b>
<b>NET LOSS PER SHARE, BASIC AND DILUTED</b>	<b>\$(1.07 )</b>	<b>\$(0.92 )</b>	<b>\$(1.28 )</b>
Weighted average common shares outstanding, basic and diluted	160,025	146,349	137,755

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

## BIOMARIN PHARMACEUTICAL INC.

## CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

Years Ended December 31, 2015, 2014 and 2013

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

	2015	2014	2013
NET LOSS	\$(171,799)	\$(133,969)	\$(176,353)
OTHER COMPREHENSIVE INCOME (LOSS):			
Net foreign currency gain (loss)	(59 )	(75 )	361
Available-for-sale securities:			
Unrealized holding gain (loss) arising during the period,			
net of tax impact of \$1,581, \$(2,931) and \$(3,535) for the years			
ended December 31, 2015, 2014 and 2013, respectively.	(2,878 )	5,088	6,275
Less reclassifications to net loss, net of tax impact of \$(681), \$0			
and \$0 for the years ended December 31, 2015, 2014			
and 2013, respectively.	1,192	—	1
Net change in unrealized holding gains, net of tax	(4,070 )	5,088	6,274
Cash flow hedges:			
Unrealized holding gain (loss) arising during the period, net of tax			
impact of \$0, \$(1,214) and \$789 for the years ended			
December 31, 2015, 2014 and 2013, respectively.	17,300	18,078	(1,366 )
Less reclassifications to net loss, net of tax impact of \$0, \$(365)			
and \$(28) for the years ended December 31, 2015,			
2014 and 2013, respectively.	19,604	643	49
Net change in unrealized holding gains (loss), net of tax	(2,304 )	17,435	(1,415 )
OTHER COMPREHENSIVE INCOME (LOSS), NET OF TAX	(6,433 )	22,448	5,220
COMPREHENSIVE LOSS	\$(178,232)	\$(111,521)	\$(171,133)

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



## BIOMARIN PHARMACEUTICAL INC.

## CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY

Years Ended December 31, 2015, 2014 and 2013

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

	Common stock		Additional	Company Common Stock Held by Nonqualified Deferred Compensation Plan	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Capital	Plan	(Loss)	Deficit	Equity
Balance at December 31, 2012	125,809	\$ 126	\$ 1,561,890	\$ (6,603 )	\$ (202 )	\$ (539,448 )	\$ 1,015,763
Net loss						(176,353 )	(176,353 )
Other comprehensive income					5,220		5,220
Purchase of capped call share options, net of tax			(19,065 )				(19,065 )
Issuance of convertible debt, net of tax and offering costs			99,879				99,879
Issuance of common stock under the 2006 Employee Stock Purchase Plan (the ESPP)	254		6,839				6,839
Exercise of common stock options	2,885	4	65,736				65,740
Excess tax benefit from stock option exercises			733				733
Conversion of convertible notes, net	14,313	14	283,305				283,319
Restricted stock vested during the period, net	203		(6,397 )				(6,397 )
Common stock held by Nonqualified Deferred Compensation Plan				(818 )			(818 )
Stock-based compensation			66,181				66,181

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Balance at December 31, 2013	143,464	\$ 144	\$2,059,101	\$ (7,421 )	\$ 5,018	\$ (715,801 )	\$ 1,341,041
Net loss						(133,969 )	(133,969 )
Other comprehensive income					22,448		22,448
Issuance of common stock, net of offering costs	1,500	1	117,463				117,464
Issuance of common stock under ESPP	258		8,714				8,714
Exercise of common stock options	2,564	3	71,187				71,190
Excess tax benefit from stock option exercises			1,491				1,491
Conversion of convertible notes, net	1,055	1	21,323				21,324
Restricted stock vested during the period, net	253		(7,768 )				(7,768 )
Common stock held by Nonqualified							
Deferred Compensation Plan				(2,274 )			(2,274 )
Stock-based compensation			88,233				88,233
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
Issuance of common stock under ESPP	185		9,957				9,957
Exercise of common stock options	1,723	2	53,086				53,088
Excess tax benefit from stock option exercises			2,190				2,190
Conversion of convertible notes, net	449	1	9,111				9,112
Restricted stock vested during the period, net	300		(22,989 )				(22,989 )
Common stock held by Nonqualified							
Deferred Compensation Plan				(3,921 )			(3,921 )
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

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



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## BIOMARIN PHARMACEUTICAL INC.

## CONSOLIDATED STATEMENTS OF CASH FLOWS

Years Ended December 31, 2015, 2014 and 2013

(In thousands of U.S. dollars)

	2015	2014	2013
<b>CASH FLOWS FROM OPERATING ACTIVITIES:</b>			
Net loss	\$(171,799 )	\$(133,969 )	\$(176,353 )
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization expense	47,187	45,871	36,014
Non-cash interest expense	28,493	27,225	5,875
Accretion of discount on investments	2,177	7,211	5,899
Stock-based compensation expense	111,525	86,410	64,376
Gain on sale of intangible asset	(369,498 )	(67,500 )	—
Gain on termination of leases	—	(10,092 )	—
Gain on sale of equity investment	(3,022 )	—	—
Impairment of assets	211,502	—	939
Deferred income taxes	(76,827 )	(25,617 )	(8,905 )
Excess tax benefit from stock option exercises	(2,190 )	(1,491 )	(733 )
Unrealized foreign exchange gain on forward contracts	(19,575 )	(832 )	(658 )
Non-cash changes in the fair value of contingent acquisition consideration payable	(28,457 )	11,567	10,197
Debt conversion expense	163	674	12,965
Other	2,300	4,514	1,149
Changes in operating assets and liabilities:			
Accounts receivable, net	(16,367 )	(25,951 )	(8,740 )
Inventory	(50,989 )	(22,339 )	(21,102 )
Other current assets	25,800	(2,211 )	(12,073 )
Other assets	(3,157 )	(6,516 )	3,952
Accounts payable and accrued liabilities	90,298	38,040	20,304
Other long-term liabilities	747	3,093	9,559
Net cash used in operating activities	(221,689 )	(71,913 )	(57,335 )
<b>CASH FLOWS FROM INVESTING ACTIVITIES:</b>			
Purchases of property, plant and equipment	(227,653 )	(117,062 )	(65,193 )
Funds held in escrow for the purchase of real property	—	—	(116,500 )
Deposit on purchase of PKU rights	(371,756 )	—	—
Maturities and sales of investments	424,713	808,313	288,643
Purchase of available-for-sale investments	(873,184 )	(507,036 )	(395,042 )
Proceeds from sale of intangible asset	410,000	67,500	—
Business acquisitions, net of cash acquired	(538,392 )	—	(9,875 )
Investment in convertible promissory note	(3,326 )	(52,288 )	—
Other	—	(3,100 )	(885 )
Net cash provided by (used in) investing activities	(1,179,598 )	196,327	(298,852 )
<b>CASH FLOWS FROM FINANCING ACTIVITIES:</b>			

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Proceeds from exercises of stock options and the ESPP	63,045	79,904	72,579
Taxes paid related to net share settlement of equity awards	(22,989 )	(7,768 )	(6,397 )
Proceeds from convertible senior note offering, net	—	—	726,202
Purchase of capped call share options	—	—	(29,813 )
Proceeds from public offering of common stock, net	888,257	117,464	—
Excess tax benefit from stock option exercises	2,190	1,491	733
Payments for debt conversion	(163 )	(674 )	(12,965 )
Payment of contingent acquisition consideration payable	—	(4,691 )	(3,061 )
Other	(2,427 )	(37 )	(607 )
Net cash provided by financing activities	927,913	185,689	746,671
Effect of exchange rate changes on cash	(5,072 )	(3,398 )	(2,230 )
<b>NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS</b>	<b>(478,446 )</b>	<b>306,705</b>	<b>388,254</b>
Cash and cash equivalents:			
Beginning of period	\$875,486	\$568,781	\$180,527
End of period	\$397,040	\$875,486	\$568,781
<b>SUPPLEMENTAL CASH FLOW DISCLOSURES:</b>			
Cash paid for interest, net of interest capitalized into fixed assets	9,307	9,324	2,159
Cash paid for income taxes	16,084	34,986	14,897
Stock-based compensation capitalized into inventory	11,140	8,166	6,121
Depreciation capitalized into inventory	14,627	10,952	11,016
<b>SUPPLEMENTAL CASH FLOW DISCLOSURES FROM</b>			
<b>INVESTING AND FINANCING ACTIVITIES:</b>			
Increase (decrease) in accounts payable and accrued liabilities related to fixed assets	(4,651 )	16,766	5,001
Conversion of convertible debt, net	9,112	21,324	283,319

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), a Delaware corporation, develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. BioMarin 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 product portfolio consists of five approved products and multiple clinical and pre-clinical product candidates. The Company's approved products are Vimizim (elosulfase alfa), Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (laronidase) and Firdapse (amifampridine phosphate).

The Company expects to continue to finance future cash needs that exceed its operating activities primarily through its current cash, cash equivalents, short-term and long-term investments, and to the extent necessary, through proceeds from equity or debt financings, loans and 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 Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse; the potential need for additional financings; the Company's ability to successfully commercialize its approved product candidates; 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 health care industry.

(2) BASIS OF PRESENTATION

Basis of Presentation

These Consolidated Financial Statements have been prepared in accordance with accounting principles generally accepted in the United States (U.S. GAAP) 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 except for the transactions disclosed in Note 25 to these 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. Actual results could differ from those estimates.

### (3) SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

#### Cash and Cash Equivalents

The Company treats liquid investments with original maturities of three months or less when purchased as cash and cash equivalents.

#### Investments

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. All of the Company's securities are classified as available-for-sale and reported in short-term investments, other assets or long-term investments. Available-for-sale investments are recorded at fair market value, with unrealized gains or losses included in Accumulated Other Comprehensive Income on the Company's Consolidated Balance Sheets, exclusive of other-than-temporary impairment losses, if any. Investments consist of corporate securities, commercial paper, U.S. federal government agency securities and certificates of deposit.

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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)

### Inventory

The Company values inventory at the lower of cost or net realizable value and determines the cost of inventory using the average-cost method. Inventories consist of currently marketed products and may contain certain products awaiting regulatory approval. In evaluating the recoverability of inventories produced in preparation for product launches, the Company considers the likelihood that revenue will be obtained from the future sale of the related inventory together with the status of the product within the regulatory approval process.

The Company analyzes its inventory levels quarterly and writes down inventory that has become obsolete, or has a cost basis in excess of its expected net realizable value and inventory quantities in excess of expected requirements. Expired inventory is disposed of and the related costs are recognized as Cost of Sales in the Company's Consolidated Statements of Operations.

### Inventories Produced in Preparation for Product Launches

The Company capitalizes inventories produced in preparation for product launches based upon the probability of regulatory approval and earning future revenues. Typically, capitalization of such inventory begins when positive results have been obtained for the clinical trials that the Company believes are necessary to support regulatory approval, uncertainties regarding ultimate regulatory approval have been significantly reduced and the Company has determined it is probable that these capitalized costs will provide some future economic benefit in excess of capitalized costs. The material factors considered by the Company in evaluating these uncertainties include the receipt and analysis of positive pivotal clinical trial results for the underlying product candidate, results from meetings with the relevant regulatory authorities prior to the filing of regulatory applications, and the compilation of the regulatory application. The Company closely monitors the status of each respective product within the regulatory approval process, including all relevant communication with regulatory authorities. The Company also considers its historical experience with manufacturing and commercializing similar products and the relevant product candidate. If the Company is aware of any specific material risks or contingencies other than the normal regulatory review and approval process, or if there are any specific issues identified relating to safety, efficacy, manufacturing, marketing or labeling, the related inventory would generally not be capitalized.

For inventories that are capitalized in preparation of product launch, anticipated future sales, expected approval date and shelf lives are evaluated in assessing realizability. The shelf life of a product is determined as part of the regulatory approval process; however, in evaluating whether to capitalize pre-launch inventory production costs, the Company considers the product stability data of all of the pre-approval production to date to determine whether there is adequate expected shelf life for the capitalized pre-launch production costs. In applying the lower of cost or net realizable value to pre-launch inventory, the Company estimates a range of likely commercial prices based on its comparable commercial products.

### 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. Property and

equipment purchased for specific research and development (R&D) projects with no alternative uses are expensed as incurred.

Leasehold improvements	Shorter of life of asset or lease term
Building and improvements	20 to 50 years
Manufacturing and laboratory equipment	5 to 15 years
Computer hardware and software	3 to 8 years
Office furniture and equipment	5 years
Vehicles	5 years
Land improvements	10 years
Land	Not applicable
Construction-in-progress	Not applicable

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 accompanying 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.

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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)

Impairment of Long-Lived 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. Goodwill and intangible assets with indefinite lives are not amortized but subject to an annual impairment analysis. Intangible assets with finite lives are amortized over their estimated useful lives on a straight-line basis.

The Company performs its annual impairment review of goodwill and indefinite lived intangibles during the fourth quarter and whenever events or circumstances indicate that the carrying amount of an asset may not be recoverable. If it is determined that the full carrying amount of an asset is not recoverable, an impairment loss is recorded in the amount by which the carrying amount of the asset exceeds its fair value. The Company currently operates in one business segment, the biopharmaceutical development and commercialization segment. When reviewing goodwill for impairment, the Company assesses whether goodwill should be allocated to operating levels lower than its single operating segment for which discrete financial information is available and reviewed for decision making purposes. These lower levels are referred to as reporting units. As of December 31, 2015, the Company has only one reporting unit.

The Company tests finite-lived intangible assets for impairment when facts or circumstances suggest that the carrying value of the asset may not be recoverable. If the carrying value exceeds the projected undiscounted pre-tax cash flows of the intangible asset, an impairment loss equal to the excess of the carrying value over the estimated fair value (discounted after-tax cash flows) is recognized.

The recoverability of the carrying value of the Company's buildings, leasehold improvements for its facilities and equipment depends on the successful execution of the Company's business initiatives and its ability to earn sufficient returns on approved products and product candidates. The Company continually monitors events and changes in circumstances that could indicate carrying amounts of its fixed assets may not be recoverable. When such events or changes in circumstances occur, the Company assesses recoverability by determining whether the carrying value of such assets will be recovered through the undiscounted expected future cash flows. If the future undiscounted cash flows are less than the carrying amount of these assets, the Company recognizes an impairment loss based on the excess of the carrying amount over the fair value of the assets.

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. Upon recognition of revenue from product sales, provisions are made for government rebates such as Medicaid reimbursements, customer incentives such as cash discounts for prompt payment, distributor fees and expected returns of expired products, as appropriate. 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 Company's 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 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 39.5% to 50% royalty on worldwide net Aldurazyme sales by 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 product is 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 calculated royalty earned when the product is sold by Genzyme. The Company records the Aldurazyme royalty revenue based on net sales information provided by Genzyme and

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

records 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.

The Company records reserves for rebates payable under Medicaid and other government programs as a reduction of revenue at the time product revenues are recorded. The Company's reserve calculations require estimates, including estimates of customer mix, to determine which sales will be subject to rebates and the amount of such rebates. The Company updates its estimates and assumptions each quarter and records any necessary adjustments to its reserves. The Company records fees paid to distributors 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. 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.

However, 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. The Company relies on historical return rates to estimate returns. 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.

**Collaborative Agreement Revenues**—Collaborative agreement revenues include both license revenue and contract research revenue.

Activities under collaborative agreements are evaluated to determine if they represent a multiple element revenue arrangement. The Company identifies the deliverables included within the agreement and evaluates which deliverables represent separate units of accounting. The Company accounts for those components as separate units of accounting if the following two criteria are met:

- The delivered item or items have value to the customer on a stand-alone basis; and
- If there is a general right of return relative to the delivered items, delivery or performance of the undelivered items is considered probable and within the Company's control.

Factors considered in this determination include, among other things, whether any other vendors sell the items separately and if the licensee could use the delivered item for its intended purpose without the receipt of the remaining deliverables. If multiple deliverables included in an arrangement are separable into different units of accounting, the Company allocates the arrangement consideration to those units of accounting. The amount of allocable arrangement consideration is limited to amounts that are fixed or determinable. Arrangement consideration is allocated at the inception of the arrangement to the identified units of accounting based on their relative estimated selling price. Revenue is recognized for each unit of accounting when the appropriate revenue recognition criteria are met.

Nonrefundable up-front license fees where the Company has continuing involvement through R&D collaboration are initially deferred and recognized as collaborative agreement license revenue over the estimated period for which the Company continues to have a performance obligation.

Future milestone payments that are contingent upon the achievement of a substantive milestone are recognized in their entirety in the period in which the milestone is achieved. A milestone is substantive if:

- It can only be achieved based in whole or in part on either the Company's performance or on the occurrence of a specific outcome resulting from the Company's performance;
- There is substantive uncertainty at the date an arrangement is entered into that the event will be achieved; and
- It would result in additional payments being due to the entity.

Royalty, License and Other Revenues—Royalty revenues includes royalties on net sales of products with which the Company has no direct involvement and is recognized based on data reported by licensees or sublicensees and rental income associated with the tenants in the SRCC, which is recognized on a straight-line basis over the term of the respective lease. Royalties are recognized as

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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)

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

Due to the significant role the Company plays in the operations (primarily the manufacturing and regulatory activities) of Aldurazyme and Kuvan as well as the rights and responsibilities to deliver the products to Genzyme and Merck Serono, respectively, the Company elected not to classify these royalties earned as royalty, license and other revenues but instead to include them as a component of Net Product Revenues in the Company's Consolidated Statements of Operations.

#### Research and Development

R&D expenses include expenses associated with contract research and development provided by third parties, most product manufacturing prior to regulatory approval, clinical and regulatory costs, and internal R&D costs. 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. The Company accrues costs for clinical trial activities based upon the services received and estimates of related expenses incurred that have yet to be invoiced by the vendors that perform the activities.

#### Convertible Debt Transactions

The Company separately accounts for the liability and equity components of convertible debt instruments that can be settled in cash by allocating the proceeds from issuance between the liability component and the embedded conversion option, or equity component, in accordance with accounting for convertible debt instruments that may be settled in cash (including partial cash settlement) upon conversion. 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 Company recognizes the accretion of the resulting discount using the effective interest method as part of Interest Expense in its 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. The Company currently has no dilutive securities due to the net loss position and as such, basic and diluted net loss per share are the same for the periods presented.

#### Stock-Based Compensation

The Company uses the Black-Scholes option-pricing model to determine the fair value of stock options and the Company's ESPP awards. The determination of the fair value of stock-based payment awards using an option-pricing model is affected by the Company's stock price as well as assumptions regarding a number of complex and subjective variables. Stock-based compensation expense is recognized on a straight-line basis over the requisite service period for each award. Further, stock-based compensation expense recognized in the Company's Consolidated Statements of Operations is based on awards expected to vest and therefore the amount of expense has been reduced for estimated forfeitures, which are based on historical experience. If actual forfeitures differ from estimates at the time of grant they will be revised in subsequent periods.

The Company uses a lattice model with a Monte Carlo simulation to value restricted stock unit awards with performance and market conditions. This valuation methodology utilizes the closing price of the Company's common stock on grant date and several key assumptions, including expected volatility of the Company's stock price, risk-free rates of return, expected dividend yield and estimated total shareholder return.

If factors change and different assumptions are employed in determining the fair value of stock-based awards, the stock-based compensation expense recorded in future periods may differ significantly from what was recorded in the current period. See Note 17 to these Consolidated Financial Statements for further information.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

### Nonqualified Deferred Compensation Plan

The Company's Nonqualified Deferred Compensation Plan (the NQDC Plan) allows eligible employees, including members of the Company's Board of Directors (the Board), management and certain highly-compensated employees as designated by the NQDC Plan's administrative committee, to make voluntary deferrals of compensation to specified dates, retirement or death. Participants are permitted to defer portions of their salary, annual cash bonus and restricted stock. The Company is not allowed to make additional direct contributions to the NQDC Plan on behalf of the participants without further action by the Board.

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 liability for the NQDC Plan is included in Accounts Payable and Accrued Liabilities and Other Long-Term Liabilities in the Company's Consolidated Balance Sheets. The corresponding asset for the NQDC Plan is included in Other Current Assets and Other Assets in the Company's Consolidated Balance Sheets.

### 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 and Other Hedging Instruments

The Company engages in transactions denominated in foreign currencies and, as a result, is exposed to changes in foreign currency exchange rates. To manage the volatility resulting from fluctuating foreign currency exchange rates, the Company nets its exposures, where possible to take advantage of natural offsets and enters into forward foreign currency exchange contracts for the remaining exposures.

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 defined as hedging instruments are adjusted to fair value through earnings. Gains and losses resulting from changes in fair value are accounted for depending on the use of the derivative and whether it is designated and qualifies for hedge accounting.

The Company assesses, both at inception and on an ongoing basis, whether the derivatives that are used in hedging transactions are highly effective in offsetting the changes in cash flows of the hedged items. The Company also assesses hedge ineffectiveness on a monthly basis and records the gain or loss related to the ineffective portion to current earnings. If the Company determines that a forecasted transaction is no longer probable of occurring, it discontinues hedge accounting for the affected portion of the hedge instrument, and any related unrealized gain or loss on the contract is recognized in current earnings.

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

#### Fair Value of Financial Instruments

The Company discloses the fair value of financial instruments for assets and liabilities for which the value is practicable to estimate. The carrying amounts of all cash equivalents, short-term and long-term investments and forward exchange contracts approximate fair value based upon quoted market prices. The fair values of trade accounts receivables, accounts payable and other financial instruments approximate carrying value due to their short-term nature, and would be considered level 2 items in the fair value hierarchy.

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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)

Business Combinations

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 in-process research and development (IPR&D). In connection with the purchase price allocations for acquisitions, the Company estimates the fair value of contingent payments utilizing a probability-based income approach inclusive of an estimated discount rate.

Contingent Acquisition Consideration Payable

The Company determines the fair value of contingent acquisition consideration payable on the acquisition date using a probability-based income approach utilizing an appropriate 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 Company's Consolidated Statements of Operations. Changes in the fair value of the contingent acquisition consideration payable can result from adjustments to the estimated probability and assumed timing of achieving the underlying milestones, as well as from changes to the discount rates and periods.

Comprehensive Income (Loss) and Accumulated Other Comprehensive Income

Comprehensive income (loss) includes net income (loss) and certain changes in stockholders' equity that are excluded from net income (loss), such as changes in unrealized gains and losses on the Company's available-for-sale securities, unrealized gains (losses) on foreign currency hedges and changes in the Company's cumulative foreign currency translation account.

Reclassifications and Adjustments

Certain items in the prior year's Consolidated Financial Statements have been reclassified to conform to the current presentation.

(4) RECENT ACCOUNTING PRONOUNCEMENTS

In April 2015, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. ASU 2015-03, Interest–Imputation of Interest (Subtopic 835-30): Simplifying the Presentation of Debt Issuance Costs (ASU 2015-03), which changes the presentation of debt issuance costs in financial statements. ASU 2015-03 requires an entity to present such costs in the balance sheet as a direct deduction from the related debt liability rather than as an asset. Amortization of the costs will continue to be reported as interest expense. It is effective for annual reporting periods beginning after December 15, 2015, which for the Company is January 1, 2016. Early adoption is permitted, and the Company adopted the new standard as of December 31, 2015. The new guidance was applied retrospectively to each prior period presented. The early adoption of this standard did not have a material impact on the Company's



results of operations and resulted in the reclassification of \$11.7 million and \$15.1 million of deferred offering costs on the Company's Consolidated Balance Sheets for the years ended December 31, 2015 and 2014, respectively.

In July 2015, the FASB deferred the effective date for ASU No. 2014-09, Revenue from Contracts with Customers (ASU 2014-09), by one year. ASU 2014-09 will supersede the revenue recognition requirements in Revenue Recognition (Topic 605) and requires entities to recognize revenue in a way that depicts the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled to in exchange for those goods or services. ASU 2014-09 is effective for annual reporting periods beginning after December 15, 2017, including interim periods within that reporting period, which for the Company is January 1, 2018. Early adoption is not permitted. The new standard can be applied retrospectively to each prior reporting period presented or retrospectively with the cumulative effect of the change recognized at the date of the initial application in retained earnings. The Company is currently evaluating the potential impact the adoption of ASU 2014-09 will have on its consolidated financial statements and has not yet selected a transition method.

In July 2015, the FASB issued ASU No. 2015-11, Inventory (ASU 2015-11), which requires an entity to measure inventory within the scope at the lower of cost and net realizable value. Net realizable value is the estimated selling prices in the ordinary course of business, less reasonably predictable costs of completion, disposal, and transportation. The effective date for the standard is for fiscal years beginning after December 15, 2016, which for the Company is January 1, 2017. Early adoption is permitted. The new standard is to be applied prospectively. The Company does not expect ASU 2015-11 to have a material impact on its consolidated financial statements.

In September 2015, the FASB issued ASU No. 2015-16, Simplifying the Accounting for Measurement-Period Adjustments (ASU 2015-16). The amended guidance requires that an acquirer recognize adjustments to provisional amounts that are identified during the

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

measurement period in the reporting period in which the adjustment amounts are determined. The amendments are effective prospectively for the fiscal years, and the interim reporting periods within those years, beginning on or after December 15, 2015 and early adoption is permitted. The Company has elected not to early adopt ASU 2015-16.

In November 2015 the FASB issued ASU 2015-17, Balance Sheet Classification of Deferred Taxes (ASU 2015-17), which requires that deferred tax liabilities and assets be classified as noncurrent in a classified statement of financial position. ASU is effective for the Company in its first quarter of fiscal 2017, with early application permitted and, upon adoption, may be applied either prospectively or retrospectively. The Company has elected to adopt this standard early and apply it retrospectively. The early adoption of this standard did not have a material impact on the Company's results of operations and resulted in the reclassification of the 2014 current deferred tax asset of \$31.2 million to long-term.

In January 2016, the FASB issued ASU No. 2016-01, Financial Instruments - Overall (Subtopic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities (ASU 2016-01). ASU 2016-01 addresses certain aspects of recognition, measurement, presentation, and disclosure of financial instruments. ASU 2016-01 is effective for fiscal years, and interim periods within those years, beginning after December 15, 2017, which for the Company is January 1, 2018. The Company is currently evaluating the impact that the standard will have on its consolidated financial statements.

## (5) ACQUISITIONS

### Prosensa Holding N.V.

On January 29, 2015, the Company completed the acquisition of Prosensa Holding N.V. (Prosensa), a public limited liability company organized under the laws of the Netherlands, for a total purchase price of \$751.5 million. In connection with the acquisition of Prosensa, the Company recognized transaction costs of \$9.7 million, of which \$7.0 million and \$2.7 million, was recognized in the years ended December 31, 2015 and 2014, respectively.

Prosensa was an innovative biotechnology company engaged in the discovery and development of ribonucleic acid (RNA)-modulating therapeutics for the treatment of genetic disorders. Prosensa's primary focus was on rare neuromuscular and neurodegenerative disorders with a large unmet medical need, including subsets of patients with Duchenne muscular dystrophy (DMD), myotonic dystrophy and Huntington's disease. Prosensa's clinical portfolio of RNA-based product candidates was focused on the treatment of DMD. Each of Prosensa's DMD compounds has been granted orphan drug status in the United States (the U.S.) and the European Union (the EU). Prosensa's lead product, Kyndrisa (drisapersen), was under review during most of 2015 as part of a rolling new drug application (NDA) with the Food and Drug Administration (the FDA). In January 2016 the FDA issued a complete response letter to the Company's NDA for Kyndrisa concluding that the standard of substantial evidence of effectiveness for Kyndrisa had not been met. On June 8, 2015, the Company announced the submission of a marketing authorization application (MAA) for Kyndrisa with the European Medicines Agency (the EMA).

In connection with its acquisition of Prosensa, the Company made cash payments totaling \$680.1 million, which consisted of \$620.7 million for approximately 96.8% of Prosensa's ordinary shares (the Prosensa Shares), \$38.6 million for the options that vested pursuant to the Company's tender offer for the Prosensa Shares and \$20.8 million to the remaining Prosensa shareholders that did not tender their shares under the tender offer. Additionally, for each Prosensa Share, the Company issued one non-transferable contingent value right (the CVR), which represents the contractual right to receive a cash payment of up to \$4.14 per Prosensa Share, or an aggregate of approximately \$160.0 million (undiscounted), upon the achievement of certain product approval milestones. The fair value of the CVRs and acquired in-process research and development (IPR&D) on the acquisition date was \$71.4 million and \$772.8 million, respectively. The acquisition date fair value of the CVRs and IPR&D was estimated by applying a probability-based income approach utilizing an appropriate discount rate. Key assumptions include a discount rate and various probability factors. See Note 13 to these Consolidated Financial Statements for additional discussion regarding fair value measurements of the CVRs, which is included in contingent acquisition consideration payable.

## 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 allocation of the purchase consideration for the Prosensa acquisition, including the CVRs, based on fair value.

Cash and cash equivalents	\$ 141,669
Trade accounts receivable	3,086
Other current assets	1,537
Property, plant and equipment	2,683
Intangible assets	497
Other assets	104
Acquired IPR&D	772,808
Total identifiable assets acquired	922,384
Accounts payable and accrued expenses	(68,799 )
Debt assumed	(57,053 )
Deferred tax liability	(193,202)
Total liabilities assumed	(319,054)
Net identifiable assets acquired	603,330
Goodwill	148,134
Net assets acquired	\$ 751,464

A substantial portion of the assets acquired consisted of IPR&D related to Prosensa's product candidates Kyndrisa and exons PRO 044 and PRO 045, which are considered to be indefinite-lived assets until completion or abandonment of the associated research and development (R&D) efforts. The Company determined that the estimated acquisition-date fair value of the intangible assets related to Kyndrisa, and Prosensa's other primary product candidates, PRO 044 and PRO 045, was \$731.8 million, \$16.9 million and \$24.1 million, respectively.

The deferred tax liability relates to the tax impact of future amortization or possible impairments associated with the identified intangible assets acquired, which are not deductible for tax purposes.

Prosensa's results of operations prior to and since the acquisition date are insignificant to the Company's Consolidated Financial Statements.

See Note 8 to these Consolidated Financial Statements for further discussion of the indefinite-lived intangible assets.

## San Rafael Corporate Center

In March 2014, the Company completed the acquisition of the real estate commonly known as the San Rafael Corporate Center (SRCC), located in San Rafael, California. SRCC is a multi-building, commercial property where, prior to the transaction, the Company was leasing a certain portion of the space for its headquarters and related operating activities. The purpose of this acquisition is to allow for future expansion of the Company's corporate headquarters to accommodate anticipated headcount growth. The acquisition of SRCC has been accounted for as a business combination because the building and the in-place leases met the definition of a business in Accounting Standards Codification 805 (ASC 805), Business Combinations. The fair value of the consideration paid was \$116.5

million, all of which was paid in cash.

The following table summarizes the estimated fair values of assets acquired as of the date of acquisition:

	Estimated	Estimated
	Fair Value	Useful Lives
Building and improvements	\$ 94,414	50 years
Land	14,565	
Land improvements	3,616	10 years
		Remaining
Intangible assets	3,905	lease terms
Total identifiable net assets	\$ 116,500	

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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 values assigned to tangible and identifiable intangible assets acquired are based on management's estimates and assumptions using the information that was available as of the date of the acquisition. The Company believes that the information provides a reasonable basis for estimating the fair values of assets acquired.

The following table sets forth the fair value of the components of the identifiable intangible assets acquired by asset class as of the date of acquisition:

Above market leases	\$351
In-place leases	3,554
Total intangible assets subject to amortization	\$3,905

The value of any lease intangible assets (such as in-place and above-market leases) is estimated to be equal to the property owners' avoidance of costs necessary to release the property for a lease term equal to the remaining primary in-place lease term and the value of investment-grade tenancy, which is derived by estimating, based on a review of the market, the cost to be borne by a property owner to replicate a market lease for the remaining in-place term. These costs consist of: (i) rent lost during downtime (e.g., assumed periods of vacancy), (ii) estimated expenses that would be incurred by the property owner during periods of vacancy, (iii) rent concessions (e.g., free rent), (iv) leasing commissions and (v) tenant improvement allowances. The Company determined these values using management's estimates along with third-party appraisals. The Company will amortize the capitalized value of lease intangible assets over the remaining lives of the underlying leases. Lease intangible assets are amortized as a reduction in (addition to) third-party tenant revenue, which is included in Royalty, License and Other Revenues on the Company's Consolidated Statements of Operations for the years ended December 31, 2015 and 2014.

The amount of third-party tenant revenue (included in the line item Royalty, License and Other Revenues) included in the Company's Consolidated Statements of Operations for the years ended December 31, 2015 and 2014, was \$2.7 million and \$4.0 million, respectively. Amortization expense recorded as a reduction of third-party tenant revenues for the years December 31, 2015 and 2014 were \$1.2 million and \$1.8 million, respectively. The amount of net income/loss from third-party tenants for the years ended December 31, 2015 and 2014, was insignificant the to the Company's Consolidated Statement of Operations.

SRCC's results of operations prior to the acquisition were insignificant to the Company's Consolidated Financial Statements.

Included in Selling, General and Administrative (SG&A) expenses are transaction costs incurred in connection with the acquisition of SRCC of \$0.3 million during 2014. In connection with the purchase of SRCC, the Company recognized a gain of \$8.8 million in the year ended December 31, 2014, due to the early termination of the Company's pre-existing lease and the realization of the remaining balance in deferred rent and the reversal of the related asset retirement obligation upon acquisition of the SRCC. \$2.7 million and \$6.1 million of the gain were included in SG&A

and R&D expenses, respectively. The allocation of the gain to SG&A and R&D is consistent with the Company's allocation practices for facility costs for this previously leased space.

#### (6) GOODWILL

Goodwill is tested for impairment on an annual basis and between annual tests if the Company becomes aware of any events occurring or changes in the circumstances that would indicate a reduction in the fair value of the goodwill below its carrying amount.

The following table represents the changes in goodwill for the year ended December 31, 2015:

Balance at December 31, 2014	\$54,258
Addition of goodwill related to the acquisition of Prosensa	148,134
Reduction of goodwill related to the sale of talazoparib <sup>(1)</sup>	(5,353 )
Balance at December 31, 2015	\$197,039

(1) See Note 8 to these Consolidated Financial Statements for additional discussion related to the sale of talazoparib. During the fourth quarter of 2015, the Company performed its annual impairment review and determined no impairments of goodwill existed at December 31, 2015.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

## (7) INVESTMENTS

All investments were classified as available-for-sale at December 31, 2015 and 2014. The amortized cost, gross unrealized holding gains or losses, and fair value of the Company's available-for-sale securities by major security type at December 31, 2015 and 2014 are summarized in the tables below:

		Gross	Gross	Aggregate
		Unrealized	Unrealized	Fair
	Amortized	Holding	Holding	Value at
	Cost	Gains	Losses	December
Certificates of deposit	\$ 63,919	\$ 1	\$ —	\$ 63,920
Corporate debt securities	358,625	20	(732 )	357,913
Commercial paper	12,733	—	—	12,733
U.S. government agency securities	186,882	—	(344 )	186,538
Greek government-issued bonds	48	79	—	127
Total	\$ 622,207	\$ 100	\$ (1,076 )	\$ 621,231

		Gross	Gross	Aggregate
		Unrealized	Unrealized	Fair
	Amortized	Holding	Holding	Value at
	Cost	Gains	Losses	December
Certificates of deposit	\$ 72,302	\$ 1	\$ —	\$ 72,303
Corporate debt securities	95,478	—	(342 )	95,136
Greek government-issued bonds	50	73	—	123
Total	\$ 167,830	\$ 74	\$ (342 )	\$ 167,562

The Company has two investments in marketable equity securities measured using quoted prices in their respective active markets that are collectively considered strategic investments. As of December 31, 2015, the fair value of the Company's strategic investments of \$18.1 million included an unrealized gain of \$12.7 million. As of December 31, 2014, the fair value of the Company's strategic investments of \$30.8 million includes an unrealized gain of \$18.3 million. These investments are recorded in Other Assets in the Company's Consolidated Balance Sheets.

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



	December 31,	
	2015	2014
Maturing in one year or less	\$195,579	\$69,706
Maturing after one year through five years	425,652	97,856
Total	\$621,231	\$167,562

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, 2015, some of the Company's investments were in an unrealized loss position. The Company determined that it did not have the ability and intent to hold all investments that have been in a continuous loss position until maturity or recovery, thus recognizing an other-than-temporary impairment of \$1.2 million in the year ended December 31, 2015.

See Note 13 to these Consolidated Financial Statements for additional discussion regarding the fair value of the Company's available-for-sale securities.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

## (8) INTANGIBLE ASSETS

Intangible assets consisted of the following:

	December 31,	
	2015	2014
<b>Intangible assets:</b>		
Finite-lived intangible assets	\$ 129,572	\$ 123,365
Indefinite-lived intangible assets	607,548	74,430
Gross intangible assets:	737,120	197,795
Less: Accumulated amortization	(53,124 )	(41,217 )
Net carrying value	\$ 683,996	\$ 156,578

## Finite-Lived Intangible Assets

The following table summarizes the net-book-value and estimated remaining life of the Company's finite-lived intangible assets at December 31, 2015:

	Net Balance at	
	December 31,	
	2015	Average Remaining Life
Repurchased royalty rights	\$ 53,438	7.9 years
Acquired intellectual property	19,652	8.6 years
License payments for marketing approvals	2,380	5.7 years
SRCC in-place and above market tenant leases	978	Remaining lease terms
Total	\$ 76,448	

As of December 31, 2015, the estimated future amortization expense associated with the Company's finite-lived intangible assets for each of the five succeeding fiscal years is as follows:

Fiscal Year	Amount
2016	\$ 11,371
2017	10,904

2018	10,874
2019	10,605
2020	8,188
Thereafter	24,506
	\$76,448

## Indefinite-Lived Intangible Assets

Indefinite-lived intangible assets consisted of the following:

	December 31,	
	2015	2014
In-Process Research and Development:		
Talazoparib	\$—	\$35,150
Kyndrisa	533,064	—
Other exons	41,044	—
Reveglucosidase alfa	25,010	25,010
Other acquired pre-clinical compounds	8,430	14,270
Net carrying value	\$607,548	\$74,430

Intangible assets related to IPR&D assets are considered to be indefinite-lived until the completion or abandonment of the associated R&D efforts. During the period the assets are considered indefinite-lived, they will not be amortized but will be tested for impairment on an annual basis and between annual tests if the Company becomes aware of any events occurring or changes in

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

circumstances that would indicate a reduction in the fair value of the IPR&D assets below their respective carrying amounts. If and when development is complete, which generally occurs if and when regulatory approval to market a product is obtained, the associated assets would be deemed finite-lived and would then be amortized based on their respective estimated useful lives at that point in time.

On October 6, 2015, the Company completed the sale of talazoparib to Medivation Inc. (Medivation). Pursuant to the 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, Medivation 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. See Note 6 to these Consolidated Financial Statements for additional discussion.

Based on the current U.S. development efforts the Company recorded an impairment charge of \$198.7 million during the fourth quarter of 2015. The Marketing Authorization Application (MAA) for Kyndrisa remains under review in the EU. The Company anticipates that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) will provide an opinion for its MAA for Kyndrisa in the second quarter of 2016. If the CHMP opinion is positive, the MAA will be referred to the European Commission (EC). If the MAA is approved by the EC, the Company would receive marketing authorization for Kyndrisa in all EU Member States.

Additionally, during the fourth quarter of 2015, the Company performed its annual impairment review and determined that no other intangible assets were impaired as of December 31, 2015.

## (9) PROPERTY, PLANT AND EQUIPMENT

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

	December 31,	
	2015	2014
Building and improvements	\$442,100	\$335,991
Manufacturing and laboratory equipment	145,313	124,564
Computer hardware and software	113,442	97,032
Leasehold improvements	44,247	39,297
Furniture and equipment	22,817	13,717
Land improvements	4,881	4,106
Land	45,727	29,358
Construction-in-progress	164,283	108,340

	982,810	752,405
Less: Accumulated depreciation	(278,603)	(228,889)
Total property, plant and equipment, net	\$704,207	\$523,516

The construction-in-process balance primarily consists of costs related to active construction projects including the expansion of the Company's corporate headquarters in San Rafael, California and the build-out of the manufacturing facility in Shanbally, Ireland.

Depreciation for the years ended December 31, 2015, 2014 and 2013 was \$50.1 million, \$44.3 million and \$36.5 million, respectively, of which \$14.6 million, \$11.0 million and \$11.0 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, 2015 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)

## (10) INVENTORY

Inventory consisted of the following:

	December 31,	
	2015	2014
Raw materials	\$46,115	\$22,488
Work-in-process	150,289	114,393
Finished goods	75,279	62,571
Total inventory	\$271,683	\$199,452

The inventory balances at December 31, 2015 and 2014 do not contain any pre-launch product costs.

## (11) SUPPLEMENTAL BALANCE SHEET INFORMATION

Other current assets consisted of the following:

	December 31,	
	2015	2014
Prepaid expenses	\$27,957	\$35,390
Short-term forward currency exchange contract assets	10,478	10,513
Promissory notes receivable, net	—	46,946
Restricted investments	7,348	2,354
Convertible promissory note conversion option	—	2,386
Other receivables	14,150	9,733
Other	445	1,202
Total other current assets	\$60,378	\$108,524

Other assets consisted of the following:

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	December 31,	
	2015	2014
Deposit for business acquisition	\$371,756	\$—
Deposits	8,606	12,021
Strategic investments	18,056	30,811
Long-term forward foreign currency exchange contract assets	3,533	5,387
Other	6,693	6,338
Total other assets	\$408,644	\$54,557

Accounts payable and accrued liabilities consisted of the following:

	December 31,	
	2015	2014
Accounts payable and accrued operating expenses	\$179,294	\$139,513
Accrued income taxes	59,572	—
Accrued compensation expense	78,424	45,479
Accrued vacation expense	16,921	12,540
Accrued rebates payable	32,553	14,859
Accrued royalties payable	10,412	9,050
Value added taxes payable	6,377	5,479
Other	8,958	4,924
Total accounts payable and accrued liabilities	\$392,511	\$231,844

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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 roll forward of significant estimated accrued rebates, reserve for cash discounts and allowance for doubtful accounts for the years ended December 31, 2015, 2014 and 2013 were as follows:

	Balance at Beginning of Period	Provision/ for Current Period Sales	Provision/ (Reversals) for Prior Period Sales	Actual Charges Related to Current Period Sales	Actual Charges Related to Prior Period Sales	Balance at End of Period
<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
Sales return reserve	—	40	—	—	—	40
Allowance for doubtful accounts	490	—	(397 )	—	—	93
<b>Year ended December 31, 2014:</b>						
Accrued rebates	\$ 10,429	\$ 24,431	\$ (1,159 )	\$ (12,768 )	\$ (6,074 )	\$ 14,859
Reserve for cash discounts	388	6,435	—	(5,747 )	(388 )	688
Sales return reserve	907	—	(907 )	—	—	—
Allowance for doubtful accounts	529	410	(319 )	—	(130 )	490
<b>Year ended December 31, 2013:</b>						
Accrued rebates	\$ 9,625	\$ 18,872	\$ (1,169 )	\$ (12,025 )	\$ (4,874 )	\$ 10,429
Reserve for cash discounts	372	4,549	—	(4,191 )	(342 )	388
Sales return reserve	—	907	—	—	—	907
Allowance for doubtful accounts	348	138	43	—	—	529

## (12) 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, the British Pound and the Brazilian Real.



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.

At December 31, 2015, the Company had 221 forward foreign currency exchange contracts outstanding to sell a total of 309.0 million Euros, 150 forward foreign currency exchange contracts outstanding to purchase 143.1 million Euros, 24 forward foreign currency exchange contract to sell 12.5 million Canadian dollars and 12 foreign currency exchange contracts to sell 39.2 billion Columbian Pesos with expiration dates ranging from January 2016 through November 2018. These hedges were entered into in order to protect against the fluctuations in revenue and operating expenses associated with Euro-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 revenues and operating expenses denominated in Euros related to changes in foreign currency exchange rates.

The Company also enters into forward foreign currency exchange contracts that are not designated as hedges for accounting purposes. The changes in fair value of these forward foreign currency exchange contracts are included as a part of SG&A expense in the Company's Consolidated Statements of Operations. At December 31, 2015, the Company had one outstanding forward foreign currency exchange contract to sell 40.1 million Euros and one outstanding forward foreign currency exchange contract to sell 3.9 million British Pounds, which were not designated as hedges for accounting purposes and matured on January 31, 2016.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

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 November 2018. Over the next twelve months, the Company expects to reclassify \$13.1 million from accumulated other comprehensive income to earnings as the forecasted revenue transactions occur.

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

	Asset Derivatives December 31, 2015		Liability Derivatives December 31, 2015	
	Balance Sheet Location	Fair Value	Balance Sheet Location	Fair Value
Derivatives designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	\$ 10,478	Accounts payable & accrued liabilities	\$ 1,986
Forward foreign currency exchange contracts	Other assets	3,533	Other long-term liabilities	3,057
Total		\$ 14,011		\$ 5,043
Derivatives not designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	\$ —	Accounts payable & accrued liabilities	\$ 22
Total		—		22
Total value of derivative contracts		\$ 14,011		\$ 5,065

	Asset Derivatives December 31, 2014		Liability Derivatives December 31, 2014	
	Balance Sheet Location	Fair Value	Balance Sheet Location	Fair Value
Derivatives designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	\$ 10,206	Accounts payable & accrued liabilities	\$ —
	Other assets	5,387		—

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Forward foreign currency exchange contracts			Other long-term liabilities	
Total		\$ 15,593		\$ —
Derivatives not designated as hedging instruments:				
Forward foreign currency exchange contracts			Accounts payable & accrued liabilities	
	Other current assets	\$ 307		\$ 12
Total		307		12
Total value of derivative contracts		\$ 15,900		\$ 12

The effect of the Company's derivative instruments on the Consolidated Financial Statements for the years ended December 31, 2015, 2014 and 2013 was as follows:

	Forward Foreign Currency Exchange Contracts		
	2015	2014	2013
<b>Derivatives Designated as Hedging Instruments:</b>			
Net gain (loss) recognized in Other Comprehensive Income (OCI) <sup>(1)</sup>	\$ 17,300	\$ 18,078	\$ (1,366 )
Net gain (loss) reclassified from accumulated OCI into income <sup>(2)</sup>	19,604	643	49
Net gain (loss) recognized in net loss <sup>(3)</sup>	(727 )	(294 )	310
<b>Derivatives Not Designated as Hedging Instruments:</b>			
Net gain (loss) recognized in net loss <sup>(4)</sup>	\$ 4,493	\$ 8,010	\$ (2,041 )

(1) Net change in the fair value of the effective portion classified as OCI.

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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)

(2) Effective portion classified as net product revenues.

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

(4) Classified as selling, general and administrative expense.

At December 31, 2015, 2014 and 2013, accumulated other comprehensive income before taxes associated with forward foreign currency exchange contracts qualifying for hedge accounting treatment was a gain of \$13.6 million, gain of \$15.9 million and a loss of \$2.4 million, respectively.

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.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

## (13) 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, 2015			Total
	Quoted Price in			
	Active	Significant Other	Significant	
	For	Observable	Unobservable	
Identical	Inputs	Inputs		
Assets	(Level 2)	(Level 3)		
(Level 1)	(Level 2)	(Level 3)	Total	
<b>Assets:</b>				
<b>Cash and cash equivalents:</b>				
Overnight deposits	\$ 290,731	\$ —	\$ —	\$ 290,731
Money market instruments	—	106,309	—	106,309
Total cash and cash equivalents	290,731	106,309	—	397,040
<b>Available-for-sale securities:</b>				
<b>Short-term:</b>				
Certificates of deposit	—	56,951	—	56,951
Corporate debt securities	—	42,673	—	42,673
Commercial paper	—	12,733	—	12,733
U.S. government agency securities	—	83,222	—	83,222
<b>Long-term:</b>				
Certificates of deposit	—	6,969	—	6,969
Corporate debt securities	—	315,240	—	315,240
U.S. government agency securities	—	103,316	—	103,316
Greek government-issued bonds	—	127	—	127
Total available-for-sale securities	—	621,231	—	621,231
<b>Other Current Assets:</b>				
Nonqualified Deferred Compensation Plan assets	—	440	—	440
Forward foreign currency exchange contract <sup>(1)</sup>	—	10,478	—	10,478
Restricted investments <sup>(2)</sup>	—	7,348	—	7,348

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Total other current assets	—	18,266	—	18,266
<b>Other Assets:</b>				
Nonqualified Deferred Compensation Plan assets	—	6,362	—	6,362
Forward foreign currency exchange contract <sup>(1)</sup>	—	3,533	—	3,533
Strategic investment <sup>(4)</sup>	18,056	—	—	18,056
Total other assets	18,056	9,895	—	27,951
Total assets	\$ 308,787	\$ 755,701	\$ —	\$ 1,064,488
<b>Liabilities:</b>				
<b>Current Liabilities:</b>				
Nonqualified Deferred Compensation Plan liability	\$ 1,151	\$ 440	\$ —	\$ 1,591
Forward foreign currency exchange contract <sup>(1)</sup>	—	2,008	—	2,008
Contingent acquisition consideration payable	—	—	52,946	52,946
Total current liabilities	1,151	2,448	52,946	56,545
<b>Other long-term liabilities:</b>				
Nonqualified Deferred Compensation Plan liability	24,341	6,362	—	30,703
Forward foreign currency exchange contract <sup>(1)</sup>	—	3,057	—	3,057
Contingent acquisition consideration payable	—	—	32,663	32,663
Total other long-term liabilities	24,341	9,419	32,663	66,423
Total liabilities	\$ 25,492	\$ 11,867	\$ 85,609	\$ 122,968

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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, 2014				
Quoted Price in				
Active Markets	Significant Other		Significant	Total
	For Identical Assets	Observable Inputs	Unobservable Inputs	
	(Level 1)	(Level 2)	(Level 3)	
<b>Assets:</b>				
<b>Cash and cash equivalents:</b>				
Overnight deposits	\$225,159	\$ —	\$ —	\$225,159
Money market instruments	—	650,327	—	650,327
<b>Total cash and cash equivalents</b>	<b>225,159</b>	<b>650,327</b>	<b>—</b>	<b>875,486</b>
<b>Available-for-sale securities:</b>				
<b>Short-term:</b>				
Certificates of deposit	—	54,174	—	54,174
Corporate debt securities	—	15,532	—	15,532
<b>Long-term:</b>				
Certificates of deposit	—	18,129	—	18,129
Corporate debt securities	—	79,604	—	79,604
Greek government-issued bonds	—	123	—	123
<b>Total available-for-sale securities</b>	<b>—</b>	<b>167,562</b>	<b>—</b>	<b>167,562</b>
<b>Other Current Assets:</b>				
Nonqualified Deferred Compensation Plan assets	—	514	—	514
Forward foreign currency exchange contract <sup>(1)</sup>	—	10,513	—	10,513
Restricted investments <sup>(2)</sup>	—	2,354	—	2,354
Embedded derivative <sup>(3)</sup>	—	—	2,386	2,386
<b>Total other current assets</b>	<b>—</b>	<b>13,381</b>	<b>2,386</b>	<b>15,767</b>
<b>Other Assets:</b>				
Nonqualified Deferred Compensation Plan assets	—	5,112	—	5,112
Restricted investments <sup>(2)</sup>	—	5,387	—	5,387
Strategic investment <sup>(4)</sup>	30,811	—	—	30,811
<b>Total other assets</b>	<b>30,811</b>	<b>10,499</b>	<b>—</b>	<b>41,310</b>
<b>Total assets</b>	<b>\$255,970</b>	<b>\$ 841,769</b>	<b>\$ 2,386</b>	<b>\$1,100,125</b>
<b>Liabilities:</b>				
<b>Current Liabilities:</b>				
Nonqualified Deferred Compensation Plan liability	\$1,790	\$ 514	\$ —	\$2,304

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Forward foreign currency exchange contract <sup>(1)</sup>	—	12	—	12
Contingent acquisition consideration payable	—	—	3,895	3,895
Total current liabilities	1,790	526	3,895	6,211
Other long-term liabilities:				
Nonqualified Deferred Compensation Plan liability	18,453	5,112	—	23,565
Contingent acquisition consideration payable	—	—	38,767	38,767
Total other long-term liabilities	18,453	5,112	38,767	62,332
Total liabilities	\$20,243	\$ 5,638	\$ 42,662	\$68,543

- (1) See Note 12 to these Consolidated Financial Statements for further information regarding the derivative instruments.
- (2) The restricted investments at December 31, 2015 and 2014 secure the Company's irrevocable standby letter of credit obtained in connection with certain commercial agreements.
- (3) The embedded derivative represents the fair value of the conversion feature of a promissory note which may be settled in the issuer's underlying shares.
- (4) The Company has investments in marketable equity securities measured using quoted prices in an active market that are considered strategic investments. See Note 7 to these Consolidated Financial Statements for additional discussion regarding the Company's strategic investments.

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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)

There were no transfers between levels during the year ended December 31, 2015.

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 7 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 Operations. 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, 2014	\$42,662
Addition of contingent consideration payable related to	
the Prosensa acquisition	71,402
Reversal of contingent liability related to no early FDA approval for Kyndrisa	(39,726)
Changes in the fair value of other acquisition contingent	
consideration payable	11,271
Contingent acquisition consideration payable at December 31, 2015	\$85,609

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.

Asset retirement obligations at December 31, 2014	\$3,765
Accretion expense	148
Additions	820
Settlements	(29 )
Asset retirement obligations at December 31, 2015	\$4,704

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.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

(14) CONVERTIBLE DEBT

2018/2020 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 due in October 2018 (the 2018 Notes) and \$375.0 million in aggregate principal amount of 1.50% senior subordinated convertible notes due in October 2020 (the 2020 Notes and, together with the 2018 Notes, the 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 1.5% per year, respectively, which is payable semiannually in arrears on April 15 and October 15 of each year, beginning on April 15, 2014.

The Notes are senior unsecured obligations, and rank (i) equally to any of the Company's existing and future unsecured senior debt, (ii) senior to any of the Company's future indebtedness that is expressly subordinated to the Notes, and (iii) 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 Notes for cash at 100% of the principal amount of the Notes being purchased, plus any accrued and unpaid interest.

The Notes are convertible into 7,965,975 shares of the Company's common stock under certain circumstances prior to maturity at a conversion rate of 10.6213 shares per \$1,000 principal amount of the Notes, which represents a conversion price of \$94.15 per share, subject to adjustment under certain conditions. Holders may convert their 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.

Upon conversion, 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 Notes by allocating the proceeds from issuance of the Notes between the liability component and the embedded conversion option, or equity component. This allocation was done by first 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 Notes. Additionally, in connection with the

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issuance of the 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. The effective interest rate on the liability component of the Notes for the years ended December 31, 2015 and 2014 was 7.3% and 7.5% for the year ended December 31, 2013.

The following table summarizes the additional interest expense recognized for the accretion of the debt discount and amortization of the deferred offering costs.

	Years Ended December 31,		
	2015	2014	2013
Convertible Notes due 2018			
Amortization of issuance costs	\$1,921	\$1,910	\$397
Accretion of debt discount	13,633	12,963	2,620
Convertible Notes due 2020			
Amortization of issuance costs	1,283	1,279	264
Accretion of debt discount	11,567	10,930	2,201
Total	\$28,404	\$27,082	\$5,482

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

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

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 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), of which \$31.4 million remained outstanding at December 31, 2015. The 2017 Notes were issued at face value and bear interest at the rate of 1.875% per annum, payable semi-annually in cash. The 2017 Notes are convertible, at the option of the holder, at any time prior to maturity or redemption, into shares of the Company's common stock at a conversion price of \$20.36 per share, subject to adjustment in certain circumstances. The 2017 Notes do not include a call provision and the Company is unable to unilaterally redeem the 2017 Notes prior to maturity on April 23, 2017. The Company also must repay the 2017 Notes if there is a qualifying change in control or termination of trading of its common stock. If a change of control occurs, the Company will pay a make whole premium by increasing the conversion rate applicable to the 2017 Notes.

In connection with the placement of the 2017 Notes, the Company paid \$8.5 million in offering costs, which have been deferred and are presented as a direct reduction of the outstanding 2017 Notes. The deferred offering costs are being amortized as interest expense over the life of the debt. For the year ended December 31, 2015, the Company recognized amortization expense of \$0.1 million, compared to \$0.1 million and \$0.4 million for the years ended December 31, 2014 and 2013, respectively.

During 2015, the Company entered into separate agreements with three existing holders of its senior subordinated convertible notes due in 2017 pursuant to which such holders converted \$8.1 million in aggregate principal amount of the 2017 Notes into 399,469 share of the Company's common stock. In addition to issuing the requisite number of the Company's common stock, the Company also made varying cash payments to the holders totaling \$0.2 million in the aggregate, which was recognized as Debt Conversion Expense on the Consolidated Statement of Operations for the year ended December 31, 2015.

During 2014, the Company entered into two separate agreements with an existing holder of its senior subordinated convertible notes due in 2017 pursuant to which such holder converted \$16.5 million in aggregate principal amount of the 2017 Notes into 809,351 shares of the Company's common stock. In addition to issuing the requisite number of shares of the Company's common stock, the Company also made varying cash payments to the holder totaling \$0.7 million in aggregate, of which \$0.7 million was recognized in total as Debt Conversion Expense on the Consolidated Statement of Operations for the year ended December 31, 2014.

During 2013, the Company entered into separate agreements with 18 of the existing holders of the 2017 Notes pursuant to which such holders converted \$262.8 million in aggregate principal amount of the 2017 Notes into 12,906,780 shares of the Company's common stock. In addition to issuing the requisite number of shares of the

Company's common stock pursuant to the 2017 Notes, the Company also made varying cash payments to each of the holders, totaling \$14.8 million in the aggregate, of which \$13.0 million was recognized in total as Debt Conversion Expense in the Company's Consolidated Statement of Operations for the year ended December 31, 2013 and \$1.8 million was for accrued interest. Additionally, the Company reclassified \$2.8 million of deferred offering costs to additional paid-in capital in connection with the conversion of the 2017 Notes.

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

	2015	2014
Convertible Notes due 2020	\$374,993	\$375,000
Unamortized discount	(65,478 )	(77,045 )
Unamortized deferred offering costs	(6,210 )	(7,493 )
Convertible Notes due 2020, net	303,305	290,462
Convertible Notes due 2018	374,980	375,000
Unamortized discount	(41,904 )	(55,537 )
Unamortized deferred offering costs	(5,415 )	(7,335 )
Convertible Notes due 2018, net	327,661	312,128
Convertible Notes due 2017	31,430	40,558
Unamortized deferred offering costs	(110 )	(246 )
Convertible Notes due 2017, net	31,320	40,312
Total convertible debt, net	\$662,286	\$642,902
Fair value of fixed rate convertible debt		
Convertible Notes due in 2020 <sup>(1)</sup>	\$502,701	\$456,360
Convertible Notes due in 2018 <sup>(1)</sup>	482,584	442,448
Convertible Notes due in 2017 <sup>(1)</sup>	162,016	180,984
Total	\$1,147,301	\$1,079,792

(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.

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

	Years Ended December 31,		
	2015	2014	2013
Coupon interest	\$9,750	\$9,417	\$4,550
Amortization of issuance costs	3,294	3,332	1,053
Accretion of debt discount	25,200	23,893	4,821
Total interest expense on convertible debt	\$38,244	\$36,642	\$10,424

See Note 15 to these Consolidated Financial Statements for further discussion of the effect of conversion on net loss per common share.

## (15) 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 ESPP, unvested restricted stock, common stock held by the NQDC Plan and contingent issuances of common stock related to convertible debt. The table below presents potential shares of common stock that were excluded from the computation as they were anti-dilutive using the treasury stock method (in thousands):

	Years Ended December		
	31, 2015	2014	2013
Options to purchase common stock	10,323	11,477	13,157
Common stock issuable under the 2017 Notes	1,544	1,992	3,047
Common stock issuable under the 2018 and 2020 Notes	7,966	7,966	7,966
Unvested restricted stock units	1,743	1,244	1,159
Potentially issuable common stock for the ESPP purchases	180	195	197
Common stock held by the NQDC	243	224	193
Total number of potentially issuable shares	21,999	23,098	25,719



## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

The effect of the Notes was excluded from the diluted net loss per common share since they may be settled in cash or shares at the Company's option and the Company's current intention is to settle up to the principal amount of the converted notes in cash and any excess conversion value (conversion spread) in shares of the Company's common stock. Although the Company's stock price exceeded the conversion price \$94.15 at December 31, 2015, the potential shares issuable under the Notes were excluded from the calculation of diluted loss per share as they were anti-dilutive using the if-converted method. During the years ended December 31, 2014 and 2013 the Notes had no effect on diluted net loss per share as the Company's stock price did not exceed the conversion price of \$94.15 per share for the Notes.

## (16) INCOME TAXES

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

	Years Ended December 31,		
	2015	2014	2013
U.S. Source	\$182,215	\$49,411	\$46,675
Non-U.S. Source	(336,939)	(174,279)	(223,178)
Loss before income taxes	\$(154,724)	\$(124,868)	\$(176,503)

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

	Years Ended December 31,		
	2015	2014	2013
Provision for current income tax expense:			
Federal	84,743	28,093	5,060
State and local	5,323	3,011	1,496
Foreign	3,836	3,614	2,199
	93,902	34,718	8,755
Provision for (benefit from) deferred income tax expense:			
Federal	(17,741)	(20,367)	(6,084)
State and local	(8,770 )	(4,982 )	(2,658 )
Foreign	(50,316)	(268 )	(163 )
	(76,827)	(25,617)	(8,905)
Provision for (benefit from) income taxes	\$17,075	\$9,101	\$(150 )

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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 income/(loss) before income taxes:

	Years Ended December		
	31, 2015	2014	2013
Federal statutory income tax rate	35.0 %	35.0 %	35.0 %
State and local taxes	(2.2 )%	(1.6 )%	(0.3 )%
Orphan Drug & General Business Credit	34.8 %	29.3 %	14.7 %
Stock compensation expense	(2.8 )%	(2.4 )%	(1.7 )%
Changes in the fair value of contingent acquisition consideration payable	0.2 %	(3.6 )%	(2.9 )%
Subpart F income	(8.4 )%	(9.2 )%	—
Foreign tax rate differential	(46.2)%	(51.5)%	(45.4)%
Other	(2.9 )%	(3.6 )%	1.6 %
Valuation allowance/Deferred benefit	(18.5)%	0.3 %	(0.9 )%
Effective income tax rate	(11.0)%	(7.3 )%	0.1 %

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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,	
	2015	2014
Net deferred tax assets:		
Net operating loss carryforwards	\$44,942	\$16,208
Tax credit carryforwards	143,987	171,840
Accrued expenses, reserves, and prepaids	79,029	30,626
Intangible assets	16,177	14,550
Stock-based compensation	49,322	34,133
Inventory	18,942	14,108
Impairment and capital loss carryforwards	5,005	1,997
Other	1,155	928
Valuation allowance	(67,708 )	(7,812 )
Total deferred tax assets	290,851	276,578
Joint venture basis difference	(1,888 )	(1,781 )
Acquired intangibles	(162,689)	(33,049)
Convertible notes discount	(32,162 )	(39,317 )
Property, plant and equipment	(13,192 )	(4,932 )
Unrealized gains	(4,256 )	(6,525 )
Total deferred tax liabilities	(214,187)	(85,604)
Net deferred tax assets	\$76,664	\$190,974

As of December 31, 2015, the Company had federal net operating loss carryforwards of \$22.3 million, state net operating loss carryforwards of \$184.1 million and Dutch net operating loss carryforwards of \$121.0 million. The Company also had federal research and development and orphan drug credit carryforwards of \$299.7 million and state research credit carryovers of \$61.0 million. The Company has elected to recognize the excess benefits related to the exercise of employee stock options under a with and without approach, which will be accounted for as an increase to additional paid-in-capital if and when realized. As of December 31, 2015, the Company had unrecognized federal and state stock option benefits of \$387.8 million and \$79.9 million, respectively.

The federal net operating loss carryforwards will expire at various dates beginning in 2027 through 2033 if not utilized. The federal credit carryforward will expire at various dates beginning in 2021 through 2035 if not utilized. The state net operating loss carryforwards will expire at various dates beginning in 2016 through 2035 if not utilized. The Dutch net operating loss carryforwards will expire at various dates beginning in 2016 through 2024 if not utilized. Certain state research credit carryovers will begin to expire in 2019 if not utilized, with others carrying forward indefinitely.

The Company's net operating losses and credits could be subject to annual limitations due to ownership change limitations provided by Internal Revenue Code Section 382 and similar state provisions. An annual limitation could result in the expiration of net operating losses and tax credit carryforward before utilization. There are limitations on the tax attributes of acquired entities however, the Company does not believe the limitations will have a material impact on the utilization of the net operating losses or tax credits.

In 2015, the Company established deferred tax assets related to the future contingent consideration on the sale of talazoparib and the net operating loss carryforwards acquired with Prosensa. Due to the uncertainty of the Company's ability to realize the benefits from these deferred tax assets, the Company has recorded a full valuation allowance on these assets resulting in a \$59.9 million increase in the valuation allowance. In 2014, the valuation allowance decreased by \$0.5 million primarily due to the expiration of fully capital loss carryforwards.

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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, 2015 is as follows:

	December 31,	
	2015	2014
Balance at beginning of period	\$71,663	\$50,815
Additions based on tax positions related to the current year	13,614	20,303
Additions for tax positions of prior years	1,454	545
Balance at end of period	\$86,731	\$71,663

Included in the balance of unrecognized tax benefits at December 31, 2015 are potential benefits of \$86.7 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, 2015.

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 2012 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 2012.

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 \$1.7 million as of December 31, 2015, which will be indefinitely reinvested; therefore, deferred income taxes of approximately \$0.2 million have not been provided on such foreign earnings.

## (17) EQUITY COMPENSATION PLANS

## 2014 Inducement Plan

In December 2014, the Board approved the BioMarin Pharmaceutical Inc. 2014 Inducement Plan (the 2014 Inducement Plan), which provides for grants of up to 1.7 million share-based awards to new employees, including grants of restricted stock units (RSUs) and grants of options to purchase common stock at a price equal to the fair market value of such shares on the date of grant. The awards are substantially similar to those granted under the

BioMarin Pharmaceutical Inc. 2006 Share Incentive Plan, as amended and restated on March 22, 2010 (as further amended, the 2006 Share Incentive Plan), and the BioMarin Pharmaceutical Inc. 2012 Inducement Plan (the 2012 Inducement Plan). The 2014 Inducement Plan expired in June 2015.

#### Share Incentive Plan

The 2006 Share Incentive Plan, which replaced the Company's previous stock option plans (the 1997 Stock Plan and the 1998 Directors Options Plan), provides for grants of options to employees to purchase common stock at the fair market value of such shares on the grant date, as well as other forms of equity compensation. As of December 31, 2015, awards issued under the 2006 Share Incentive Plan include both stock options and RSUs. Stock option awards granted to employees generally vest over a four-year period on a cliff basis six months after the grant date and then monthly thereafter. The term of the outstanding options is generally ten years. RSUs granted to employees generally vest annually over a straight-line four-year period after the grant date. Restricted stock units granted to directors generally vest in full one year after the grant date.

As of December 31, 2015, options to purchase approximately 10.0 million and 0.3 million shares were outstanding under the Share Incentive Plan and the Company's previous stock option plans, respectively.

As of December 31, 2015, an aggregate of approximately 27.7 million unissued shares were authorized for future issuance under the Share Incentive Plan.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

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 payroll deductions at the lower of 85% of the fair market value of the stock at the commencement of the offering period or each purchase date of the offering period. Each offering period will span up to two-years. The ESPP permits eligible employees to purchase common stock through payroll deductions for up to 10% of qualified compensation, up to an annual limit of \$25,000. The ESPP is intended to qualify as an "employee stock purchase plan" under Section 423 of the Internal Revenue Code. During 2015, the Company issued 185,091 shares under the ESPP.

As of December 31, 2015 there were approximately 1.0 million shares reserved for future issuance under the ESPP.

Board of Director Grants

Each Independent Director is automatically granted an initial stock option grant to purchase 10,000 share of common stock and 4,000 RSUs on the date that such person first becomes an Independent Director. The shares of common stock 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 shareholders, each re-elected director is granted an additional stock option grant to purchase 8,500 shares of common stock and 3,400 RSUs. The shares of common stock subject to the annual option 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 option grant or RSU grant for a director that has served for less than a year is prorated to the nearest quarter. These options and RSUs continue to vest only while the director serves on the Board. The exercise price per share of each of these options is 100% of the fair market value of a share of the Company's common stock on the date of the grant. These options have a term of 10 years.

Shares Available Under Equity Compensation Plans

At December 31, 2015, an aggregate of approximately 30.2 million unissued shares was authorized for future issuance under the Company's stock plans, which includes shares issuable under the 2006 Share Incentive Plan, the 2014 Inducement Plan, the 2012 Inducement Plan and the ESPP. Under the 2006 Share Incentive Plan awards that expire or are cancelled without delivery of shares generally become available for issuance under the respective plan. Awards that expire or are cancelled under the Company's suspended 1997 Stock Plan, 1998 Director Option Plan, 2012 Inducement Plan or 2014 Inducement Plan may not be reissued.

(18) STOCK-BASED COMPENSATION

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 2006 Share Incentive Plan for the year ended

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December 31, 2015. 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:

		Weighted Average Exercise	Weighted Average Remaining	Aggregate Intrinsic Value <sup>(1)</sup>
	Shares	Price	Years	Value <sup>(1)</sup>
Options outstanding as of December 31, 2014	11,477,164	\$ 38.11	6.2	\$ 600,112
Granted	728,770	\$ 116.94		
Exercised	(1,723,345 )	\$ 30.80		
Expired and forfeited	(159,686 )	\$ 63.79		
Options outstanding as of December 31, 2015	10,322,903	\$ 44.50	5.6	\$ 630,949
Options expected to vest at December 31, 2015	1,563,790	\$ 62.18		\$ 66,579
Exercisable at December 31, 2015	8,102,226	\$ 35.62	4.9	\$ 561,554

(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 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 \$104.76, the closing price of the Company's common stock on December 31, 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 weighted-average fair value per option granted in the years ended December 31, 2015, 2014 and 2013 was \$56.76, \$30.93 and \$30.77, respectively. The total intrinsic value of options exercised during the years ended December 31, 2015, 2014 and 2013 was \$146.6 million, \$130.1 million and \$119.2 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 9.7 million options that were in-the-money at December 31, 2015.

## Determining the Fair Value of Stock Options and Stock Purchase Rights

The fair value of each option award is estimated on the date of grant using the Black-Scholes valuation model and the assumptions noted in the tables below. The expected life of options is based on observed historical exercise patterns. Groups of employees that have similar historical exercise patterns were considered separately for valuation purposes, and as of December 31, 2015 the Company has identified two groups with distinctly different exercise patterns. The two groups identified are executive and non-executive employees. The executive employee group has a history of holding options for longer periods than non-executive employees. The expected volatility of stock options is based upon the weighted average of the historical volatility of the Company's common stock and the implied volatility of traded options on the Company's common stock for fiscal periods in which there is sufficient trading volume in options on the Company's common stock. The risk-free interest rate is based on the implied yield on a U.S. Treasury zero-coupon issue with a remaining term equal to the expected term of the option. 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 assumptions used to estimate the per share fair value of stock options granted under the 2012 Inducement Plan, the 2014 Inducement Plan and the Share Incentive Plan were as follows:

	Years Ended December 31,		
	2015	2014	2013
	36 –		
Expected volatility	45%	44 – 45%	44 – 47%
Dividend yield	0.00%	0.00%	0.00%
	6.4 -		
Expected life	8.0 years	6.9 years	6.6 – 6.8 years
	1.5 –	1.8 –	
Risk-free interest rate	2.2%	2.3%	1.0 – 2.4%

The Company recorded \$41.5 million, \$41.1 million and \$37.0 million of compensation costs related to current period vesting of stock options for the years ended December 31, 2015, 2014 and 2013, respectively. As of December 31, 2015, the total unrecognized compensation cost related to unvested stock options was \$74.2 million. These costs are expected to be recognized over a weighted average period of 2.3 years.

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

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	Years Ended December 31,		
	2015	2014	2013
	36 -		
Expected volatility	38%	38 - 39%	37%
Dividend yield	0.00%	0.00%	0.00%
Expected life	6-24 months	6-24 months	6-24 months
	0.1 -		
Risk-free interest rate	0.8%	0.1- 0.5%	0.1- 0.3%

The Company recorded \$7.1 million, \$4.8 million and \$3.6 million of compensation costs related to options granted under the ESPP for the years ended December 31, 2015, 2014 and 2013, respectively. As of December 31, 2015, there was \$9.8 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 1.3 years.

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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)

## 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. The Company expenses the cost of the RSUs, which is determined to be the fair market value of the shares of common stock underlying the RSUs at the date of grant, ratably over the period during which the vesting restrictions lapse.

A summary of non-vested RSU activity under the plan for the year ended December 31, 2015 as follows:

		Weighted		
		Average	Weighted	
		Grant	Average	Aggregate
		Date	Remaining	Intrinsic
		Fair	Years	Value
	Shares	Value		
Non-vested units as of December 31, 2014	1,540,703	\$ 59.46	2.9	\$ 139,280
Granted	1,234,650	\$ 119.86		
Vested	(489,301 )	\$ 54.48		
Forfeited	(138,843 )	\$ 81.67		
Non-vested units as of December 31, 2015	2,147,209	\$ 93.89	2.8	\$ 224,942
Non-vested units expected to vest at December 31, 2015	1,895,918	\$ 92.56		\$ 199,917

The weighted-average grant date fair value per share of RSUs granted during the years ended December 31, 2015, 2014 and 2013, was \$119.86, \$64.37 and \$66.81, respectively. The total intrinsic value of restricted stock that vested and was released in the years ended December 31, 2015, 2014 and 2013 was \$59.5 million, \$22.9 million and \$19.7 million, respectively.

The Company recorded \$47.9 million, \$21.3 million and \$13.0 million of compensation costs related to RSUs with service-based vesting conditions for the years ended December 31, 2015, 2014 and 2013, respectively. As of December 31, 2015, there was \$143.2 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 2.8 years.

## Restricted Stock Unit Awards with Performance and Market-Based Vesting Conditions

Pursuant to the approval of the Board the Company granted RSU awards with performance and market-based vesting conditions to certain executive officers that provide for a base award of 860,000 RSUs in total (Base RSUs) that may be adjusted to 75% to 125% depending on the performance of the Company's stock as discussed further below. A summary of non-vested Base RSU activity under the plans for the year ended December 31, 2015 is as follows:

	Base Awards	Weighted Average Grant Date Fair Value	Weighted Average Remaining Years	Aggregate Intrinsic Value
Non-vested units with performance and market vesting				
conditions as of December 31, 2014	860,000	\$ 34.66	1.2	\$ 77,744
Granted	—			
Vested	—			
Forfeited	—			
Non-vested units with performance and market vesting				
conditions as of December 31, 2015	860,000	\$ 34.66	0.2	\$ 90,094

The number of RSUs that could potentially vest from the Base RSUs granted is contingent upon achievement of specific performance goals and will be multiplied by the Total Shareholder Return (the TSR) multiplier which could range from 75% to 125% to determine the number of earned RSUs. The TSR multiplier is determined based on the Company's TSR percentile ranking relative to the TSR of the NASDAQ Biotechnology Index on December 31, 2015. TSR is calculated based on the 20-trading day average prices before the beginning and end of the performance period of the Company's common stock and each comparator company in the NASDAQ Biotechnology Index. The measurement period for the performance and TSR conditions is from the grant date through December 31, 2015 (the Performance Period), subject to certain change of control provisions.

## 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 contingent performance awards, which ones were achieved and the number of RSUs earned based on the final TSR multiplier of 124% at December 31, 2015.

	Percentage of	Base Number		Number of RSUs Earned After TSR Multiplier
	Base RSUs to Vest Upon Achievement of Goal	of RSUs Granted Before TSR Multiplier	Performance Goal Attained	1.24
Strategic Performance Goals				
Product Goals				
Approval of Vimizim in the U.S. or EU prior to December 31, 2015	35	% 301,000	Yes	373,240
Approval of pegvaliase (PEG PAL) or any other non-Vimizim product in the U.S. or EU prior to December 31, 2015	25	% 215,000	No	—
Financial Goal				
Total revenues of at least \$775.0 million in fiscal 2015	40	% 344,000 860,000	Yes	426,560 799,800

Stock-based compensation expense for this award was recognized over the remaining service period beginning in the period the Company determines the strategic performance goal or goals was probable of achievement. During 2014, management concluded that the revenue performance goal was probable and began recognizing compensation expense related to the performance awards allocated to the revenue performance goal. During 2013, management concluded that regulatory approval of Vimizim was probable and began recognizing compensation expense related to the performance based RSUs allocated to the Vimizim performance goal. For the years ended December 31, 2015, 2014 and 2013, the Company recorded \$5.8 million, \$12.9 million and \$6.5 million, respectively, of compensation expense related to performance awards. As of December 31, 2015, there was \$0.3 million of total unrecognized compensation cost related to the unvested awards allocated to the Vimizim and revenue performance goals. These costs are expected to be recognized over a weighted average period of 0.2 years.

## Restricted Stock Unit Awards with Performance Conditions

On March 3, 2015, pursuant to Board approval, the Company granted 58,300 RSU awards with performance-vesting conditions (the 2015 Base RSUs) under the Share Incentive Plan to certain executive officers. The vesting of the 2015 Base RSUs under this specific grant is contingent upon the achievement of a 2015 revenue target and a three-year

service period. The number of RSUs that will be awarded from the 2015 Base RSUs upon achievement of the performance condition will be calculated by multiplying the 2015 Base RSUs by a revenue multiplier (determined based on the Company's performance against the revenue target) which could range between 80% to 120%. Based on the Company's performance against the revenue target, the Company applied a multiplier of 111% and will issue 64,713 shares.

Stock-based compensation for these awards will be recognized over the service period beginning in the period the Company determines it is probable that the revenue target will be achieved. The cost of the 2015 Base RSUs was determined to be \$108.36 per RSU, based on the fair value of the common stock underlying the 2015 Base RSUs on the grant date. Accordingly, because the Company's management determined that attainment of the revenue target is probable, the Company recognized \$1.8 million of compensation expense related to these awards during the year ended December 31, 2015. As December 31, 2015, there was \$5.0 million of total unrecognized compensation related to the unvested awards. These costs are expected to be recognized over a weighted average period of 2.2 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,		
	2015	2014	2013
Cost of sales	\$6,836	\$6,076	\$4,860
Research and development	49,399	33,835	27,763
Selling, general and administrative	55,290	46,499	31,753
Total stock-based compensation expense	\$111,525	\$86,410	\$64,376

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

Stock-based compensation of \$11.1 million, \$8.2 million and \$6.1 million was capitalized into inventory, for the years ended December 31, 2015, 2014 and 2013, 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)

## (19) COMPREHENSIVE INCOME

The following table summarizes amounts reclassified out of Accumulated Other Comprehensive Income/(Loss) (AOCI) and their effect on the Company's Consolidated Statements of Operations for the years ended December 31, 2015 and 2014.

Details about AOCI Components	Amount Reclassified from AOCI (Gain) Loss Years Ended		Consolidated Statement of Operations Classification
	December 31, 2015	2014	
Gains on cash flow hedges:			
Forward foreign currency exchange contracts	\$ 17,715	\$ 1,008	Net product revenues
Forward foreign currency exchange contracts	1,889	—	Selling, general and administrative
Other-than-temporary impairment on available-for-sale securities	(1,160 )	—	Other income (expense)
Gain on sale of available-for-sale investment	3,033	—	Other income (expense)
Less income tax effect of the above	681	365	Provision for (benefit from) income taxes
	\$ 20,796	\$ 643	Net loss

The following table summarizes changes in the accumulated balances for each component, of other comprehensive income/(loss), including current period other comprehensive income and reclassifications out of AOCI, for the years ended December 31, 2015 and 2014.

	Year Ended December 31, 2015			
	Gains and Losses on Cash Flow Hedges	Unrealized Gains on Available-for-Sale Securities	Foreign Currency Items	Total
AOCI balance at December 31, 2014	15,906	11,511	49	27,466
Other comprehensive income (loss) before	17,300	(2,878 )	(59 )	14,363



reclassifications				
Less gain (loss) reclassified from AOCI	19,604	1,192	—	20,796
Net current-period other comprehensive income (loss)	(2,304 )	(4,070 )	(59 )	(6,433 )
AOCI balance at December 31, 2015	13,602	7,441	(10 )	21,033
		Year Ended December 31, 2014		
		Gains		
		and		
		Losses	Unrealized Gains	Foreign
		on	on	Currency
		Cash	Available-for-Sale	Items
		Flow	Securities	Total
		Hedges		
AOCI balance at December 31, 2013	(1,529 )	6,423	124	5,018
Other comprehensive income (loss) before				
reclassifications	18,078	5,088	(75 )	23,091
Less gain (loss) reclassified from AOCI	643	—	—	643
Net current-period other comprehensive income (loss)	17,435	5,088	(75 )	22,448
AOCI balance at December 31, 2014	15,906	11,511	49	27,466

## (20) REVENUE AND CREDIT CONCENTRATIONS

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

## 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 summarizes consolidated net product revenues concentrations based on patient location for Vimizim, Naglazyme, Kuvan and Firdapse 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. Net product revenues from Genzyme consists of royalties on worldwide net Aldurazyme sales and incremental product transfer revenues.

Region:	Years Ended December 31,		
	2015	2014	2013
United States	39 %	37 %	36 %
Europe	20 %	20 %	22 %
Latin America	16 %	16 %	14 %
Rest of world	14 %	13 %	13 %
Total net product revenues marketed by the Company	89 %	86 %	85 %
Aldurazyme net product revenues marketed by Genzyme	11 %	14 %	15 %
Total net product revenue	100 %	100 %	100 %

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

	For the Years Ended December 31,					
	2015		2014		2013	
Customer A	15 %	15 %	15 %	15 %	15 %	15 %
Customer B <sup>(1)</sup>	11 %	14 %	14 %	16 %	16 %	16 %
Customer C	10 %	12 %	12 %	9 %	9 %	9 %
Customer D	13 %	11 %	11 %	11 %	11 %	11 %
Total	49 %	52 %	52 %	51 %	51 %	51 %

(1) Genzyme is the Company's sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third-parties. Net product revenues from Genzyme consists of royalties on worldwide net Aldurazyme sales and incremental product transfer revenue.

On a consolidated basis, the Company's two largest customers accounted for 37% and 18% of the December 31, 2015 accounts receivable balance, respectively, compared to December 31, 2014 when the two largest customers accounted for 42% and 18% of the accounts receivable balance, respectively. As of December 31, 2015 and 2014, accounts receivable for the Company's largest customer balance included \$36.1 million and \$34.5 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 is subject to credit risk from accounts receivable related to product sales. The majority of the Company's trade accounts receivable arises from product sales in the U.S. and the European Union (the EU). The Company's product sales to government-owned or government-funded customers in certain European countries, including Italy, Spain, Portugal, Greece and Russia, are subject to payment terms that are statutorily determined. Because these customers are government-owned or government-funded, the Company may be impacted by declines in sovereign credit ratings or sovereign defaults in these countries. A significant or further decline in sovereign credit ratings or a default in these countries may decrease the likelihood that the Company will collect accounts receivable or may increase the discount rates and the length of time until receivables are collected, which could result in a negative impact to the Company's operating results. In the year ended December 31, 2015 the Company's net product revenues for these countries was 5%. Additionally, approximately 9% of the Company's outstanding accounts receivable at December 31, 2015 related to such countries.

As of December 31, 2015, the Company's accounts receivable in certain European countries, specifically Greece, Italy, Portugal, Spain and Russia, totaled approximately \$14.5 million, of which \$0.5 million were greater than 90 days past due.

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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 also sells its products in other countries that face economic crises and local currency devaluation. Although the Company has 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 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. The Company believes that the allowances for doubtful accounts related to these countries is adequate based on its analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries.

## (21) SEGMENT INFORMATION

The Company operates in one business segment, which primarily focuses on the development and commercialization of innovative biopharmaceuticals for serious diseases and medical conditions. 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.

	Years Ended December 31,		
	2015	2014	2013
Net product revenues by product:			
Vimizim	\$228,147	\$77,319	\$85
Naglazyme	303,090	334,447	271,244
Kuvan	239,336	202,987	167,422
Aldurazyme	97,912	105,616	83,545
Firdapse	16,037	18,047	16,064
Total net product revenues	\$884,522	\$738,416	\$538,360

The following table summarizes total revenues from external customers and collaborative partners by geographic region. Net product revenues are based on patient location for Vimizim, Naglazyme, Kuvan and Firdapse and Genzyme's headquarters for Aldurazyme. Although Genzyme sells Aldurazyme worldwide, the royalties earned by the Company 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,		
	2015	2014	2013

Total revenues by geographic region:			
United States	\$444,075	\$378,288	\$284,303
Europe	178,746	139,940	115,729
Latin America	142,305	118,562	67,339
Rest of world	124,769	112,494	81,114
Total revenues	\$889,895	\$749,284	\$548,485

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

	December 31,	
	2015	2014
Long-lived assets by geography:		
United States	\$940,512	\$827,884
Europe	865,233	102,451
Rest of world	2,253	1,630
Total long-lived assets	\$1,807,998	\$931,965

## (22) COLLABORATIVE AGREEMENTS

### Merck Serono

In May 2005, the Company entered into an agreement with Merck Serono for the further development and commercialization of 6R-BH4, both in Kuvan for PKU and for other indications, and pegvaliase (phenylalanine ammonia lyase, formerly referred to as PEG

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

PAL). Through the agreement and subsequent amendment, Merck Serono acquired exclusive rights to market these products in all territories outside the U.S., Canada and Japan, and the Company retained exclusive rights to market these products in the U.S. and Canada. Merck Serono had the right to collaborate on the development of Kuvan and pegvaliase if it “opted-in”, which it did not do for pegvaliase.

The Company and Merck Serono were individually responsible for the costs of commercializing the products within their respective territories through December 31, 2015. Merck Serono will also pay the Company royalties on its net sales of these products. As of December 31, 2015 and 2014, amounts due from Merck Serono for reimbursable development costs for Kuvan totaled \$0.2 million and \$0.2 million, respectively.

As previously reported, 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 granted in the License Agreement to Merck Serono related to Kuvan, which became effective on January 1, 2016. Also on October 1, 2015, the Company and Merck Serono entered into a Termination Agreement (the Pegvaliase Agreement) to terminate the license granted in the License Agreement from to Merck Serono related to pegvaliase, which also became effective on January 1, 2016.

As of January 1, 2016, the Company and Merck Serono have no further rights or obligations under the License Agreement with respect to pegvaliase. The License Agreement will continue in effect in order to complete the transfer of certain assets related to Kuvan on a country-by-country basis. See Note 25 to these Consolidated Financial Statements for additional discussion.

#### Other Agreements

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.

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.

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, 2015 and 2014, amounts due from Catalyst for reimbursable development costs totaled \$0.1 million and \$0.1 million, respectively.

(23) 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 3% of the employee's annual compensation or \$6,000 per year through December 31, 2013. The Company's matching contribution vests over four years from employment commencement and was approximately \$15.1 million, \$8.3 million and \$3.4 million for the

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## NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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years ended December 31, 2015, 2014 and 2013, respectively. Employer contributions not vested upon employee termination are forfeited.

## Deferred Compensation Plan

In December 2005, the Company adopted the Deferred Compensation Plan. The Deferred Compensation Plan allows eligible employees, including members of the Board, management and certain highly-compensated employees as designated by the Deferred Compensation Plan's Administrative Committee, the opportunity to make voluntary deferrals of compensation to specified future dates, retirement or death. Participants are permitted to defer portions of their salary, annual cash bonus and restricted stock. The Company may not make additional direct contributions to the Deferred Compensation Plan on behalf of the participants, without further action by the Board. Deferred compensation is held in trust and generally invested to match the investment benchmarks selected by participants. The recorded cost of any investments will approximate fair value. Company stock issued into the Deferred Compensation Plan is recorded and 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 Deferred Compensation Plan. The Company stock issued into the Deferred Compensation Plan upon vesting is recorded in stockholders' equity. As of December 31, 2015 and 2014, the fair value of Company stock held by the Deferred Compensation Plan, was \$25.5 million and \$20.2 million, respectively, which is included in current and non-current liabilities. The change in market value amounted to a loss of \$2.5 million in 2015, compared to losses of \$4.8 million and \$4.2 million in 2014 and 2013, respectively. See Note 13 to these Consolidated Financial Statements for additional discussion regarding the fair value of the Deferred Compensation Plan assets and liabilities.

## (24) 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 2025. 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:

2016	\$7,209
2017	6,527
2018	5,444
2019	2,990
2020	2,352



Thereafter	9,953
Total	\$34,475

Rent expense for the years ended December 31, 2015, 2014 and 2013 was \$9.3 million, \$7.9 million and \$10.4 million, respectively. Deferred rent accruals at December 31, 2015 totaled \$1.7 million, of which \$1.2 million was current. Deferred rent accruals at December 31, 2014 totaled \$1.3 million, of which \$0.7 million was current.

See Note 5 to these Consolidated Financial Statements for additional discussion regarding the purchase of SRCC.

#### 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, 2015, these commitments for the next five years

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NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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were approximately \$51.0 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 January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying it that Par had filed an ANDA seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of the Company's patents listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). Together with Merck & Cie, on March 6, 2015, the Company filed a lawsuit against Par in the U.S. District Court for the District of New Jersey alleging infringement of its patents relating to Kuvan tablets and seeking an injunction to prevent Par from introducing a generic version of Kuvan tablets that would infringe its patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2017. In response, Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

The parties submitted opening claim construction briefs on January 14, 2016, and responsive claim construction briefs are due on March 4, 2016. The Court has not yet set a date for trial in this litigation.

The Company also received a paragraph IV notice letter, dated January 14, 2016, from Par, notifying us that Par has filed a separate ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral powder prior to the expiration of the Company's patents listed in the FDA's Orange Book. On February 22, 2016, the Company filed a lawsuit against Par in the U.S. District Court for the District of New Jersey alleging infringement of its patents relating to Kuvan powder and seeking an injunction to prevent Par from introducing a generic version of Kuvan powder that would infringe its patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2018.

#### Contingent Payments

As of December 31, 2015 the Company is also subject to contingent payments totaling approximately \$675.4 million upon achievement of certain regulatory and licensing milestones if they occur before certain dates in the future. Of this amount, \$80.0 million relates to Prosensa and \$22.7 million relates to programs that are no longer being developed.

As of December 31, 2015, the Company has recorded \$85.6 million of contingent acquisition consideration payable on its Consolidated Balance Sheet, of which \$52.9 million is expected to be paid in the next twelve months.

(25) SUBSEQUENT EVENT

On January 1, 2016, the Company acquired all global rights to Kuvan and pegvaliase from Ares Trading, S.A. (Merck Serono), with the exception of Kuvan in Japan, in exchange for an upfront payment of \$371.8 million and up to and additional €185.0 million if certain sales and regulatory milestones are attained. Previously, the Company had exclusive rights to Kuvan in the U.S. and Canada and pegvaliase in the U.S. and Japan. Under the terms of the agreement, the Company acquired exclusive worldwide rights to Kuvan and pegvaliase with the exception of Kuvan in Japan.

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