

BIOMARIN PHARMACEUTICAL INC
Form 10-K
March 02, 2015
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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, 2014

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 stock held by non-affiliates of the registrant as of June 30, 2014 was \$5.5 billion, based on the closing price reported for such date on the NASDAQ Global Select Market.

As of February 13, 2015, the registrant had 159,135,697 shares 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 9, 2015, are incorporated by reference into Part III.

BIOMARIN PHARMACEUTICAL INC.

2014 FORM 10-K ANNUAL REPORT

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Vimizim™ is our trademark. BioMarin®, 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 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,” 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 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 issue 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 is comprised 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 several 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, the European Medicines Agency (the EMA) granted marketing approval for Firdapse, which was launched in the EU beginning in April 2010.

We are conducting clinical trials on several investigational product candidates for the treatment of various diseases including: drisapersen, an exon-51 skipping compound for the potential treatment of Duchenne muscular dystrophy (DMD); 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; talazoparib (formerly referred to as BMN 673), an orally available poly-ADP ribose polymerase (PARP) inhibitor for the treatment of patients with certain cancers; 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); and 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. We are conducting or planning to conduct preclinical development of several other product candidates for genetic and other metabolic diseases including BMN 270 and BMN 250. BMN 270 is a Factor VIII gene therapy drug development candidate, an AAV VIII vector, for the treatment of hemophilia A. We expect to initiate a Phase 1 study for BMN 270 in the first half of 2015. BMN 250 is a novel fusion of alpha-N-acetylglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo B syndrome, or Mucopolysaccharidosis type IIIB (MPS IIIB). We expect to initiate a Phase 1 study for BMN 250 in the second half of 2015.

Recent Developments

Equity Offering

On January 27, 2015 we completed an underwritten public offering of 9,775,000 shares of our common stock at the public offering price of \$93.25 per share pursuant to an effective registration statement previously filed with the SEC. Our net proceeds from the offering were approximately \$888.2 million after deducting commissions and estimated offering expenses payable by us.

Paragraph IV Notice Letter from Dr. Reddy's Laboratories

As previously disclosed, we have 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 has filed an abbreviated new drug application (ANDA) seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the U.S. Food and Drug Administration's (the FDA) Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book).

On November 17, 2014, we, together with Merck & Cie (Merck), filed a lawsuit against DRL in the United States District Court for the District of New Jersey alleging patent infringement for our patents relating to Kuvan. On January 16, 2015, we, together with Merck, filed an Amended Complaint requesting a declaratory judgment that DRL has no legitimate basis to trigger the ANDA process, alleging that DRL did not have a proper ANDA because, upon information and belief, it did not submit proper bioequivalence data in support of its purported ANDA.

Paragraph IV Notice Letter from Par Pharmaceutical

We have 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 (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book.

Declaratory Judgment Action

On January 16, 2015, we filed a lawsuit in the U.S. District Court for the Southern District of New York, seeking a declaratory judgment that we are under no legal obligation to sell or otherwise provide samples of Kuvan (sapropterin dihydrochloride) to DRL. DRL seeks such samples to conduct bioequivalence testing in support of its proposed generic Kuvan (sapropterin dihydrochloride) product.

Acquisition of Prosensa Holding N.V.

On December 12, 2014, we commenced a tender offer (the Offer) to acquire all of the ordinary shares (the Prosensa Shares) of Prosensa Holding N.V. (Prosensa), a public limited liability company (NASDAQ: RNA) organized under the laws of The Netherlands in an all cash transaction for \$17.75 per Prosensa Share for an upfront purchase price of approximately \$680.0 million. In addition, for each Prosensa Share purchased, we have 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 approximately \$160.0 million, upon the achievement of certain product approval milestones.

On January 15, 2015, we closed the initial offering period relating to the Offer and purchased approximately 93.4% of the Prosensa Shares. We immediately launched a subsequent offering period that expired on January 29, 2015. As of the expiration of this subsequent offering period, we paid approximately \$620.7 million for 34,970,514 Prosensa Shares, representing approximately 96.8% of all 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 the asset transfer and paid an additional \$20.8 million to the remaining Prosensa shareholders that did not tender their shares under the Offer. We funded the acquisition with our available cash balances.

Effective February 12, 2015, Prosensa has been dissolved and is in liquidation under Dutch law.

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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. Prosensa's lead product, drisapersen, is currently under a rolling review as part of a rolling new drug application (NDA) with the FDA. As previously announced by Prosensa we expect to complete the filing of this application in April 2015. We expect to file a marketing authorization application (MAA) for drisapersen with the EMA in the summer of 2015.

The transaction will be accounted for as a business combination. We will maintain operations at Prosensa's headquarters, based in Leiden, The Netherlands and integrate Prosensa personnel from that office.

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, 2014, is provided below:

Commercial Products	Indication	Orphan Drug Expiry U.S.	Orphan Drug Expiry EU	2014 Total Net Product Revenues (in millions)	2014 Research & Development Expense (in millions)
Vimizim	MPS IV A ⁽¹⁾	2021	2024	\$ 77.3	\$ 63.6
Naglazyme	MPS VI ⁽²⁾	Expired	September 2015	\$ 334.4	\$ 12.1
Kuvan	PKU ⁽³⁾	June 2015	NA ⁽⁴⁾	\$ 203.0	\$ 13.5
Aldurazyme ⁽⁵⁾	MPS I ⁽⁶⁾	Expired	Expired	\$ 105.6	\$ 1.6
Firdapse	LEMS ⁽⁷⁾	NA ⁽⁸⁾	2019	\$ 18.1	\$ 4.6

Products in Development	Target Indication	Orphan Designation US	Orphan Designation EU	Stage	2014 Research & Development Expense (in millions)
Drisapersen	DMD ⁽⁹⁾	Yes	Yes	Clinical Phase 3	N/A
BMN 044 (PRO 044)	DMD ⁽⁹⁾	Yes	Yes	Clinical Phase 2	N/A
BMN 045 (PRO 045)	DMD ⁽⁹⁾	Yes	Yes	Clinical Phase 2	N/A
BMN 053 (PRO 053)	DMD ⁽⁹⁾	Yes	Yes	Clinical Phase 1/2	N/A
Pegvaliase (PEG PAL)	PKU	Yes	Yes	Clinical Phase 3	\$ 70.5
Reveglucosidase alfa (BMN 701)	Pompe ⁽¹⁰⁾	Yes	Yes	Clinical Phase 2/3	\$ 51.1
Talazoparib (BMN 673) ⁽¹¹⁾	BRCA breast cancer	No	No	Clinical Phase 3	\$ 59.8
BMN 111	Achondroplasia	Yes	Yes	Clinical Phase 2	\$ 22.5
Cerliponase alfa (BMN 190)	CLN2 ⁽¹²⁾	Yes	Yes	Clinical Phase 1/2	\$ 39.5

- (1) Mucopolysaccharidosis IV Type A, or MPS IVA
- (2) Mucopolysaccharidosis VI, or MPS VI
- (3) Phenylketonuria, or PKU
- (4) Merck Serono S.A. markets Kuvan in the EU.
- (5) The Aldurazyme total product revenue noted above is the total product revenue 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. and we have licensed the North American rights to develop and market Firdapse to a third party.
- (9) Duchenne muscular dystrophy, or DMD, acquired from Prosensa in January 2015
- (10) Pompe disease, a glycogen storage disorder

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(11) Talazoparib is an orally available poly (ADP-ribose) polymerase, or PARP inhibitor for the treatment of patients with certain cancers.

(12) CLN2, or late infantile neuronal ceroid lipofuscinosis, is a lysosomal storage disorder primarily affecting the brain.

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 1,650 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 immediately began marketing Vimizim in the U.S. using our own existing sales force and commercial organization and have completed our first commercial sales in the U.S. and the EU as well as several other countries. We plan to pursue registration and/or market Vimizim on a named patient basis in other regions. Many countries allow for named patient or other early access sales based on the FDA approval. We plan to institute sales in these countries where appropriate.

Vimizim net product revenues for the years ended December 31, 2014 and 2013 totaled \$77.3 million and \$0.1 million, respectively; there were no sales of Vimizim prior to 2013.

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, 2014, 2013 and 2012 totaled \$334.4 million, \$271.2 million and \$257.0 million, respectively.

Kuvan

Kuvan is a proprietary synthetic oral form of 6R-BH4, a naturally occurring enzyme co-factor for phenylalanine hydroxylase (PAH), indicated for patients with 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 which will be 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 1-7 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. We market Kuvan in the U.S. and Canada using our own sales force and commercial organization. Kuvan has been granted orphan drug status in the U.S., which confers market exclusivity in the U.S. for the treatment of PKU, expiring in June 2015. 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, 2014, 2013 and 2012 totaled \$203.0 million, \$167.4 million, and \$143.1 million, respectively.

In 2005, we entered into an agreement with Merck Serono S.A. (Merck Serono) for the further development and commercialization of Kuvan and any other product containing 6R-BH4, and pegvaliase for PKU. Through the 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. Merck Serono markets Kuvan in the EU and several other countries outside the U.S., Canada and Japan. Under the agreement with Merck Serono, we are 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. Over the next several years, we expect a royalty of approximately four percent on net sales of Kuvan by Merck Serono. We also sell Kuvan to Merck Serono at or near cost, and Merck Serono resells 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 2014, 2013 and 2012 we earned \$2.2 million, \$2.0 million and \$1.9 million, respectively, in net royalties on net sales of \$55.5 million, \$51.0 million and \$46.8 million of Kuvan by Merck Serono, respectively. We recorded collaborative agreement revenue associated with shared Kuvan development costs in the amounts of \$0.9 million, \$1.0 million, and \$1.8 million in 2014, 2013 and 2012, respectively.

Aldurazyme

Aldurazyme has been approved for marketing in the U.S., the EU and 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. 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 (the LLC), a 50/50 limited liability company 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 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, 2014, 2013 and 2012 totaled \$105.6 million, \$83.6 million and \$82.2 million, respectively. The net product revenues for each of the years ended December 31, 2014, 2013 and 2012 include \$97.0 million, \$88.5 million and \$80.4 million, respectively, of royalty revenue on net Aldurazyme sales by Genzyme. Net sales of Aldurazyme by Genzyme totaled \$228.8 million, \$212.4 million and \$193.1 million for the years ended December 31, 2014, 2013 and 2012, respectively. Aldurazyme net product revenue included incremental Aldurazyme net product transfer revenue of \$8.6 million in the year ended December 31, 2014, previously recognized product transfer revenue of \$4.9 million in the year ended December 31, 2013 and incremental product transfer revenue of \$1.8 million in the year ended December 31, 2012. Incremental/previously recognized product transfer revenue 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.

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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, 2014, 2013 and 2012 totaled \$18.1 million, \$16.1 million and \$14.2 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, 2014 and 2013 we recognized collaborative revenue of \$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

Drisapersen

We acquired drisapersen, Prosensa's lead candidate for a subset of DMD, on January 15, 2015. See Item 1, Business—Recent Developments—Acquisition of Prosensa Holding N.V.

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. Drisapersen aims to address a specific mutation in the dystrophin gene that represents approximately 13% of all DMD patients, or approximately 10,000 patients worldwide. In clinical trials, drisapersen has been shown to induce dystrophin expression and has shown a treatment effect on DMD patients.

Two of the Phase 2 trials of drisapersen 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 drisapersen 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 drisapersen 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 drisapersen group compared to a mean 11-meter decline in the placebo group ($p=.003$). In the Phase 3 trial, the drisapersen 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 drisapersen 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 drisapersen 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 drisapersen to the FDA. Drisapersen 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. We intend to complete the submission of the NDA for drisapersen in the first quarter of 2015 and submit an MAA with the EMA in the second quarter of 2015.

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 have 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.

Talazoparib

Talazoparib is a PARP inhibitor, a class of molecules that has shown clinical activity against cancers involving defects in DNA repair that we are investigating for the treatment of certain cancers. In January 2011, we announced the initiation of a Phase 1/2 clinical trial for talazoparib for the treatment of patients with solid tumors. This clinical trial is an open-label study of once daily, orally administered talazoparib in approximately 105 patients ages 18 and older with advanced or recurrent solid tumors. The study established a preliminary dose that is generally well-tolerated and reaches steady state with repeated daily doses. The study has focused on patients with breast and ovarian cancers characterized by deleterious BRCA-1 and -2 mutations, Ewing's sarcoma and small cell lung cancer, and has been expanded to include patients with prostate and pancreatic cancers. In June 2014, we presented an update on the study at the 2014 annual meeting of the American Society for Clinical Oncology. As presented, among 14 enrolled germline BRCA (gBRCA) mutated breast cancer patients treated at the recommended Phase 3 dose of 1mg/day, the confirmed RECIST response rate was 50% (seven confirmed objective responses: one complete and six partial). In addition, there were five patients with stable disease lasting at least 24 weeks for an overall clinical benefit response (CBR) rate at this dose of 86% (12/14). In the complete cohort of 18 gBRCA mutated breast cancer patients, which included six patients from the dose-escalation cohort at doses ranging from 900 µg/day to 1100 µg/day and 12 patients from the dose expansion cohort at a dose of 1.0 mg/day, the RECIST response rate was 44% (8/18), with one complete and seven partial responses. The CBR rate was 72% (13/18), with five patients having stable disease in excess of 24 weeks. The median progression-free survival (PFS) was 32 weeks in this heavily pre-treated advanced breast cancer population. Safety data continue to show that talazoparib is generally well tolerated with the most common drug-related toxicities being myelosuppression (including thrombocytopenia, anemia and neutropenia), mild to moderate fatigue, nausea and alopecia.

Based on the results of this Phase 1/2 study, we initiated a Phase 3 trial in patients with gBRCA mutated breast cancer in October 2013. The Phase 3 trial is an open-label, 2:1 randomized, parallel, two-arm study of talazoparib as compared to the protocol-specified physicians' choice of chemotherapy in gBRCA mutated locally advanced and/or metastatic breast cancer patients who have received no more than two prior chemotherapy regimens for metastatic

disease. The study is enrolling approximately 429 patients and is being conducted at approximately 140 sites in sixteen countries. The primary objective of the study is to compare PFS of patients treated with talazoparib as a monotherapy relative to those treated with protocol-specified physicians' choice. The secondary objectives are to evaluate objective response rate (ORR), overall survival (OS), safety and the pharmacokinetics of talazoparib. We expect to complete enrollment of this Phase 3 trial in the second half of 2015.

Additionally, we initiated a Phase 2 trial in patients with gBRCA mutated breast cancer at the beginning of 2014. The purpose of this 2-stage, 2-cohort Phase 2 trial is to evaluate the safety and efficacy of talazoparib in patients with locally advanced or metastatic breast cancer with a deleterious gBRCA mutation. Patients are assigned to either cohort 1 or 2 based on prior chemotherapy for metastatic disease: cohort 1) Patients who have previously responded to a platinum-containing regimen for metastatic disease with disease progression > 8 weeks following the last dose of platinum; or cohort 2) Patients who have received > 2 chemotherapy regimens and who have had no prior platinum therapy for metastatic disease. The primary objective of the study is to determine the ORR for each cohort of patients. The secondary objectives are to evaluate CBR rate, the duration of response for objective responders, the PFS and the OS.

Talazoparib is also being studied as monotherapy and in combination with chemotherapy agents in collaboration with the U.S. National Cancer Institute under a cooperative research and development agreement in a series of clinical trials.

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 have completed enrollment of this study 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 are 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 are 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.

Health authorities, including the FDA and the EMA, have indicated that MIP is a potentially approvable primary endpoint for our Phase 2/3 switching trial with reveglucosidase alfa, assuming the results of the trial are compelling and clinically meaningful. This switching trial is designed to enroll late onset Pompe patients who have previously been treated with alglucosidase alfa. The trial has been initiated with the first patient enrolled in May of 2014. We are targeting to enroll approximately 20 patients in the first quarter of 2015 to establish proof-of-concept. We are currently working on improving the manufacturing process for reveglucosidase alfa, which we expect will be our commercial manufacturing process, and will target to enroll up to 50 additional patients in a trial to be administered the drug manufactured with this improved process.

The reveglucosidase alfa program now includes, in addition to the above studies, an observational study as well as another Phase 2 study which is designed to support MIP as a primary endpoint. Both of these studies are also currently active and enrolling.

BMN 111

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 BMN 111. The primary objective of the Phase 1 clinical trial was to assess the safety and tolerability of single and multiple doses of BMN 111 in normal healthy adult volunteers up to the maximum tolerated dose. BMN 111 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 BMN 111 for the treatment of children with achondroplasia. This international clinical trial is an open-label, sequential cohort, dose-escalation study of BMN 111 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 BMN 111 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. A total of 26 subjects have been enrolled in this study for a treatment duration of six months. The protocol was recently amended to allow subjects who completed six months of treatment to be enrolled in an 18-month extension study. We plan to report 6-month data for the first three cohorts in the second quarter of 2015.

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. It is estimated that 400-600 cases exist worldwide, but CLN2 is believed to be underdiagnosed. 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 are tolerating the therapeutic dose. These preliminary results have the potential to support the feasibility of a single-study filing with regulatory authorities. We expect to announce complete results in the fourth quarter of 2015. We may decide to enroll one or more additional cohorts in this study after we review the data for the first three cohorts and meet with regulatory authorities.

BMN 044, BMN 045, and BMN 053

We acquired BMN 044, BMN 045 and BMN 053, Prosensa's candidates for the treatment of subsets of DMD, on January 15, 2015. See Item 1, Business—Recent Developments—Acquisition of Prosensa Holding N.V.

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 the first quarter of 2013 in Europe, 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 September 2013 which is ongoing.

Manufacturing

We manufacture Naglazyme, Aldurazyme, Vimizim, pegvaliase, BMN 111 and cerliponase alfa in our approved Good Manufacturing Practices (GMP) production facilities located in Novato, California. Vialing and packaging are performed by contract manufacturers. We believe that we have ample operating capacity to support the commercial demand of both Naglazyme and Aldurazyme through at least the next five years as well as the commercial requirements for the initial launch of Vimizim.

In August 2011, we acquired a bulk biologics manufacturing plant located in Shanbally, County of Cork, Ireland. This 142,000-square-foot facility which was completed and validated in 2009 was approved by the Irish Medicines Board in 2010. We are not currently manufacturing any products in this facility. We currently intend to manufacture Vimizim in this facility. However, before we can manufacture any product in this facility, including Vimizim, we will need to requalify and validate certain systems in the facility. The addition of the Shanbally facility will increase our operating capacity to support the anticipated commercial demand of Vimizim.

Our Novato, California facilities have demonstrated compliance with GMPs to the satisfaction of the FDA, the European Commission (EC) and health agencies in other countries for the commercial production of Aldurazyme, Naglazyme and Vimizim. 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.

Both the Kuvan tablet and powder sachet are manufactured on a contract basis by a third-party. There are two approved manufacturers of the active pharmaceutical ingredient (API) for Kuvan. Firdapse, reveglucosidase alfa and talazoparib are each manufactured on a contract basis by a third-party. There is one approved manufacturer of the API for Firdapse.

In general, we expect to continue to contract with outside service providers for certain manufacturing services, including final product vialing and packaging operations for our recombinant enzymes and API production and tableting for Kuvan and Firdapse. 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 commercial demand for Kuvan and Firdapse. 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 Naglazyme. 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. We believe that with moderate additions in 2015, 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. We utilize third-party logistics companies to store and distribute our products.

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

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 European distributors and to certain larger pharmaceutical wholesalers, which act as intermediaries between us and end-users and generally do not stock significant quantities of Naglazyme and Vimizim. During 2014, 54% of our net Vimizim, Naglazyme, Kuvan and Firdapse product revenues were generated by four customers. Genzyme is our sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third-parties. In certain countries particularly in Latin America, governments place large periodic orders for Vimizim and Naglazyme. The timing of these orders can create significant quarter to quarter variation in our revenue.

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 (MPS) 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. 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

Talazoparib, faces competition from several other PARP inhibitors at a similar stage in development and from AstraZeneca’s approved product, Lynparza™ (olaparib), which is approved for patients with deleterious or suspected deleterious gBRCA mutated advanced ovarian cancer. 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. Drisapersen, has competition from Sarepta Therapeutics’ product eteplirsen, which is at a similar stage in development. 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.

The number of our worldwide issued patents now stands at approximately 500, including approximately 64 patents issued by the U.S. Patent and Trademark Office (the USPTO). Furthermore, our portfolio of pending patent applications totals approximately 458 applications, including approximately 78 pending U.S. applications.

With respect to Naglazyme, we have 24 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 75 issued patents including 13 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 2029.

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 11 issued patents including five 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 and are set to expire in 2024.

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 products 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 United States and other jurisdictions.

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

Approval Process in the United States and European Union

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.

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 GMP 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. The EMA currently has proposed regulations that would require substantially more disclosure regarding clinical trials, including individual patient level data.

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

Vimizim, Naglazyme, Aldurazyme, Kuvan and Firdapse have received orphan drug designations from the FDA and the EMA. Orphan drug designation is granted 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. 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 which 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, risk evaluation and mitigation strategies, 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 current Good Manufacturing Practices (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.

Patient Protection and Affordable Care Act of 2010

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 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 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. No biosimilar or interchangeable products have been approved under the BPCIA 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 twelve 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 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. The Centers for Medicare & Medicaid Services (CMS) issued regulations, which required manufacturers to begin collecting required information in 2013, with the first reports due in 2014. The reported data was posted in searchable form on a public website beginning September 30, 2014.

Approval Outside of the United States/European Union

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.

Other Regulatory Requirements

In addition to FDA restrictions on marketing of pharmaceutical products, several other types of state and federal laws have been applied to restrict certain marketing practices in the pharmaceutical industry in recent years. These laws include anti-kickback statutes and false claims statutes. The federal healthcare program 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. 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. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs. 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 majority of states also have statutes or regulations similar to the federal anti-kickback law 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, exclusion of a company's products from reimbursement under government programs, criminal fines 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, 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.

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 GMP.

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 issue warning or similar letters or may seek civil, criminal, or administrative sanctions against us.

Pricing and Reimbursement

Because the course of treatment for patients using our products is expensive, sales of our products depends, in part, on the availability and extent of coverage and reimbursement from third party payers, including governments 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. Reimbursement rates from private companies vary depending on the third-party payer, the insurance plan and other factors. 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

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 has increased since launch.

The statutory definition of AMP was recently amended, and there are many ambiguities in the revised provision. In February 2012, CMS published a proposed rule further defining AMP and providing clarification on other parts of the rebate program. Until the rule is finalized, manufacturers are required to make reasonable assumptions when interpreting the statute and calculating AMP.

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 21, 2015, we had 1,681 full-time employees, 676 of whom are in operations, 540 of whom are in research and development, 206 of whom are in sales and marketing and 259 of whom are 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 2014, 2013 and 2012, see Item 7, Management 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.

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Net product revenues by geography are based on patients' locations for Vimizim, Naglazyme, Kuvan and Firdapse, and are based on Genzyme's U.S. location for Aldurazyme. Although Genzyme sells Aldurazyme worldwide, the royalties we earned on Genzyme's net sales are included in the U.S. net product revenues as our transactions are with Genzyme.

The following table outlines net product revenues by geographic area (in thousands):

	Years Ended December 31,		
	2014	2013	2012
Net product revenues:			
United States	\$375,710	\$277,495	\$249,745
Europe	139,940	116,896	108,138
Latin America	118,562	67,338	74,390
Rest of the world	104,204	76,631	64,224
Total net product revenues	\$738,416	\$538,360	\$496,497

Total revenue generated outside the U.S. was \$371.0 million, \$264.2 million and \$251.0 million, in the years ended December 31, 2014, 2013 and 2012, respectively.

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

	December 31,		
	2014	2013	2012
Non-monetary long-lived assets:			
United States	\$820,356	\$651,815	\$574,522
International	104,081	82,130	80,067
Total long-lived assets	\$924,437	\$733,945	\$654,589

The increase in non-monetary long-lived assets in 2014 compared to 2013 was primarily attributed to increases in property, plant and equipment and deferred tax assets. The increase in non-monetary long-lived assets in 2013 compared to 2012 was attributed to increases in property, plant and equipment and long-term deferred offering costs.

Other Information

We were incorporated in Delaware in October 1996 and began operations on March 21, 1997. 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 Exchange Act are available free of charge at 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 or maintain 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. 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. Naglazyme, Aldurazyme and Kuvan 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. Vimizim received regulatory approval in the U.S. on February 14, 2014, in the EU on April 28, 2014, in Canada on July 7, 2014 and in Australia on December 2, 2014 but has not been approved in any other jurisdiction and may never receive additional regulatory approvals for any other jurisdiction.

As part of the recent reauthorization of the Prescription Drug User Fee Act, new biologics are included in a new product review program intended to enhance FDA-sponsor communications to lead to greater first-cycle approval decisions. As part of this program, applications for new biologics are subject to either a 12-month standard or 8-month priority review period that begins from the date of application submission. However, since this is a new product review program and few products have completed this new review process, the priority review period may take longer than eight months and the standard review period may take longer than 12 months. Similarly, although the EMA has an accelerated approval process, the timelines mandated by the regulations are subject to the possibility of substantial delays.

In addition, the FDA and its international equivalents have substantial discretion over the approval process for pharmaceutical products. As such, these regulatory agencies may in the end 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 ex-U.S. and ex-EU 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.

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 and, to date, we have generally been able to reach reasonable accommodations and resolutions regarding the underlying issues. 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.

After any of our products receive regulatory approval, they remain subject to ongoing regulation, which can impact, among other things product labeling, manufacturing practices, adverse event reporting, storage, expiration, distribution, advertising and promotion, record keeping and import and export. If we do not comply with the applicable regulations, the range of possible sanctions includes issuance of warning or untitled letters or adverse publicity, product recalls or seizures, fines, total or partial suspensions of production and/or distribution, suspension of marketing applications, and other enforcement actions, including injunctions and civil or criminal prosecution. The FDA and comparable international regulatory agencies can withdraw a product's approval under some circumstances, such as the failure to comply with regulatory requirements or unexpected safety issues. Further, the FDA often requires post-marketing testing and surveillance to monitor the effects of approved products. The FDA and 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. If data we collect from post-marketing studies suggest that one of our approved products may present a risk to safety, the government authorities could withdraw our product approval, suspend production or place other marketing restrictions on our products. If regulatory sanctions are applied or if

regulatory approval is delayed or withdrawn, the value of our company and our operating results will be adversely affected. Additionally, 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 fail to obtain or maintain orphan drug exclusivity for some of our products, our competitors may sell products to treat the same conditions and our revenues will be reduced.

As part of our business strategy, we intend to 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. 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 products and 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 until the first 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. 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, 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 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 PPACA created a regulatory pathway under the PHS Act for the abbreviated approval for 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 law 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. Our products approved under BLAs, as well as products in development that may be approved under BLAs, could be reference products for such biosimilar marketing applications.

To obtain regulatory approval to market our products, preclinical studies and costly and lengthy preclinical and 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 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;

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- 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 GCP, invalid or inadequate, our own clinical data and results and related regulatory approvals could adversely be impacted.

If we continue to incur operating losses 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 operated at a net loss until 2008. Although we were profitable in 2008, 2010 and the third quarter of 2014, we operated at a net loss in 2009, 2011, 2012, 2013 and 2014. Based upon our current plan for investments in research and development for existing and new programs, we expect to operate at a net loss for at least the next 12 months. Our future profitability depends 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 and the impact of any possible future business development transactions. The extent of our future losses and the timing of profitability are highly uncertain. If we fail to become profitable or are unable to sustain profitability 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 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 facilities in the U.S. have been approved by the FDA, the EC, and health agencies in other countries for the manufacture of Aldurazyme and Naglazyme. 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. The manufacturing facility located in Shanbally, Cork, Ireland that we purchased in 2011 has not yet been approved by the FDA or the EMA to manufacture any of our products. We intend to make a substantial investment in the build-out of the Shanbally facility in order to manufacture Vimizim and other products. If the facility is not ultimately approved by the FDA or the EMA to manufacture any of our products, we will not be able to manufacture Vimizim or other products at this facility and we may not be able to meet the anticipated commercial demand for Vimizim which would have an adverse effect on our financial results.

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 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, 2014, we had cash, cash equivalents and short and long-term investments totaling \$1,043.1 million and long-term debt obligations of \$790.6 million (undiscounted). In January 2015, we paid \$620.7 million for 34,970,514 Prosensa Shares, 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 \$160.0 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 and 1.50% senior subordinated convertible notes due in 2020 (collectively, the Notes) but also the ongoing interest due on the Notes during their term. 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 \$888.2 million. We may require additional financing to fund 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, if needed, 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. that trigger related milestone payments;

- 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.

We may 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 may 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.

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 and Firdapse, if those manufacturers are unwilling or unable to fulfill their contractual obligations, we may be unable to meet demand for these products or sell these products at all and we may lose potential revenue. We have contracts for the production of final product for Kuvan and Firdapse. We also rely on third-parties for portions of the manufacture of Naglazyme and Aldurazyme. 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.

Our manufacturing facility for Naglazyme, Aldurazyme and Vimizim is 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 manufacturer's ability to manufacture Kuvan or Firdapse.

Our Galli Drive facility located in Novato, California is currently our only manufacturing facility for Naglazyme, Aldurazyme and Vimizim. It is located in the San Francisco Bay Area near known earthquake fault zones and is vulnerable to significant damage from earthquakes. We, the third-party manufacturers with whom we contract and our single-source suppliers of raw materials, which include many of our critical raw materials, are also vulnerable to damage from other types of disasters, including fires, 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.

Numerous factors could cause interruptions in the supply 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 manufacturers as needed on a timely basis; and
- conditions affecting the cost and availability of raw materials.

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 we must market worldwide to achieve significant market penetration of the product. In addition, because the number of potential patients in the disease populations are 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.

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 through special access or “named patient” programs, which do not require full product approval. We expect to also utilize these programs for Vimizim. 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. 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. 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 could restrict the amount that we are able to charge for our current and future products, which would adversely affect our revenue and results of operations.

Government 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 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, which have varying effective dates, may 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 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.

In addition, other legislative changes have been proposed and 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 will remain in effect through 2024 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. 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 “fraud and abuse” 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 fraud and abuse laws, including anti-kickback laws, false claims laws and laws related to ensuring compliance. The federal health care program anti-kickback statute makes it illegal for any person, 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. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs.

Federal and state 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. 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, 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. Sanctions under these federal and state laws may include civil monetary penalties, exclusion of a manufacturer's products from reimbursement under government programs, criminal fines and imprisonment.

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.

Substantial new provisions affecting compliance have also been adopted, which may require us to modify our business practices with health care practitioners. The PPACA, among other things, requires drug manufacturers to collect and report 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. Manufacturers were required to begin collecting required information on August 1, 2013 and the CMS made public the reported data in a searchable form on September 30, 2014. Manufacturers are required to submit reports to CMS by the 90th day of each subsequent calendar year.

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 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.

While we believe we have structured our business arrangements to comply with these laws, because of 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, it is possible that some of 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 and criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. 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 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 and Naglazyme and all of the sales of Firdapse are generated from countries other than the U.S. Additionally, we have operations in several European countries, Brazil, other Latin American countries, Turkey and other Asian 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:

- changes in international regulatory and compliance requirements that could restrict our ability to manufacture, market and sell our products;
- political and economic instability;
- diminished protection of intellectual property in some countries outside of the U.S.;
- trade protection measures and import or export licensing requirements;
- difficulty in staffing and managing international operations;
- differing labor regulations and business practices;
- potentially negative consequences from changes in or interpretations of tax laws;
- changes in international medical reimbursement policies and programs;
- financial risks such as longer payment cycles, difficulty collecting accounts receivable and exposure to fluctuations in foreign currency exchange rates; and
- 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 FCPA.

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. Moreover, when we do implement currency hedges, we only hedge our net exposure, or the difference between our revenues in a currency and the offsetting expenses in that currency. Since we do not generally hedge the portion of our revenues that has offsetting expenses in

that currency, our revenues can be particularly affected by changes in exchange rates. 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 proprietary technology, we may not be able to compete as 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:

- 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.
- 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 USPTO after grant. It is also unclear whether our trade secrets are adequately protected. Our 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 the outcome is unpredictable. 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.

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 talazoparib, reveglucosidase alfa, BMN 111 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 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 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 LLC at a specified buyout amount. If such option is not exercised, all rights to Aldurazyme will be sold and the 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 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 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 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 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.

Based on our strategic alliance with Merck Serono, unless Merck Serono "opts in" to the pegvaliase program, we will not realize any cost sharing for the development expenses, development milestones, or royalties for ex-U.S. sales.

In May 2005, we entered into an agreement with Merck Serono for the further development and commercialization of Kuvan (and any other product containing 6R-BH4) and pegvaliase for PKU. Pursuant to that agreement, we received development milestones on Kuvan and receive royalties on sales by Merck Serono. Additionally, we may be entitled to development milestones and royalties related to pegvaliase. However, Merck Serono has "opted out" of the pegvaliase development program. Unless and until it elects to opt in, it is not obligated to pay any of the milestones related to the program or to reimburse us for any of the development costs. Additionally, even though Merck Serono has opted out of the pegvaliase development program, we do not have any right to commercialize pegvaliase outside of the U.S. and Japan or to grant anyone else such rights.

Merck Serono may elect to opt in at any time. If Merck Serono opts in to the pegvaliase development program before the unblinding of the first Phase 3 trial for pegvaliase, it must pay 75% of the Phase 3 costs incurred prior to the opt-in and the \$7,000,000 Phase 3 initiation milestone. If it opts in after unblinding of the first Phase 3 trial for pegvaliase, it must pay 100% of the Phase 3 costs incurred prior to the opt-in and the \$7,000,000 Phase 3 initiation milestone. Additionally, in all cases after it opts in to the pegvaliase development program, Merck Serono would be obligated to pay one half of future development costs under the agreement and any further milestones due under the agreement. If Merck Serono does not opt in, it will not have the right to use any of the clinical or other independently developed data.

We cannot determine when or if Merck Serono will opt in to the pegvaliase development program. If Merck Serono does not opt in, we will not receive any milestones under the agreement nor will there be any sales outside of the U.S. or Japan generating revenue from royalties or otherwise.

If we fail to compete successfully with respect to acquisitions, joint ventures or other collaboration opportunities, we may be limited in our ability to develop new products and to continue to expand our product pipeline.

Our competitors compete with us to attract organizations for acquisitions, joint ventures, licensing arrangements or other collaborations. To date, several of our product programs have been acquired through acquisitions, such as reveglucosidase alfa and talazoparib and several of our 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 (sapropterin hydrochloride) at any time after December 2011.

BioMarin owns several patents that cover Kuvan (sapropterin dihydrochloride), and we have listed those patents in conjunction with that product in the Orange Book. The Hatch-Waxman Act requires an ANDA applicant seeking FDA approval of its proposed generic product prior to the expiration of our Orange Book-listed patents to certify that the applicant believes that our patents are invalid or will not be infringed by the manufacture, use or sale of the drug for which the application has been submitted (a paragraph IV certification) and notify us of such certification (a paragraph IV notice). Upon receipt of a paragraph IV notice, the Hatch-Waxman Act allows us, with proper basis, to bring an action for patent infringement against the ANDA filer, asking that the proposed generic product not be approved until after our patents expire. If we commence a lawsuit within 45 days from receipt of the paragraph IV notice, the Hatch-Waxman Act provides a 30-month stay, during which time the FDA cannot finally approve the generic's application. If the litigation is resolved in favor of the ANDA applicant during the 30-month stay period, the stay is lifted and the FDA's review of the application may be completed. 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. Regardless of any litigation results, generic versions of Kuvan (sapropterin dihydrochloride) would be prohibited until the expiration of orphan drug exclusivity in June 2015, including pediatric exclusivity, at the earliest. We have also received three-year Hatch-Waxman exclusivity for a New Patient Population for Kuvan (sapropterin dihydrochloride) that expires in October 2017, including pediatric exclusivity. Thus, depending on the proposed labeling of a generic product, generic versions of Kuvan (sapropterin dihydrochloride) 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 have received a paragraph IV notice letter, dated October 3, 2014, from DRL, notifying us that DRL has filed an ANDA seeking approval of a proposed generic version Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our Orange Book-listed patents. On November 17, 2014, we, together with Merck, filed a lawsuit against DRL in the United States District Court for the District of New Jersey alleging patent infringement for our patents relating to Kuvan. On January 16, 2015, we, together with Merck, filed an Amended Complaint requesting a declaratory judgment that DRL has no legitimate basis to trigger the ANDA process, alleging that DRL did not have a proper ANDA because, upon information and belief, it did not submit proper bioequivalence data in support of its purported ANDA. We also have 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 (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the Orange Book.

The filing of DRL's and Par's purported ANDAs in respect to Kuvan (sapropterin dihydrochloride) 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 (sapropterin dihydrochloride) and its use are not upheld in litigation, or if DRL and/or 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.

If we do not achieve our projected development goals in the timeframes we announce and expect, 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.

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 MPS I, MPS VI, PKU or 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 Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse, or our clinical trials for pegvaliase, reveglucosidase alfa, talazoparib, BMN 111, cerliponase alfa or BMN 270 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 through 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, 2014 approximately 4% of our net product revenues were from Italy, Spain, Portugal, Greece and Russia. Approximately 8% of our total accounts receivable as of December 31, 2014 related to such 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 Prosensa Holding N.V.

If we do not successfully integrate Prosensa into our business operations, our business could be adversely affected.

We will need to successfully integrate the operations of Prosensa with our business operations. Integrating the operations of Prosensa with that of our own will be a complex and time-consuming process. Prior to the acquisition, Prosensa operated independently, with its own business, corporate culture, locations, employees and systems. There may be substantial difficulties, costs and delays involved in any integration of the business of Prosensa with that of our own. These may include:

- distracting management from day-to-day operations;
- potential incompatibility of corporate cultures;
- an inability to achieve synergies as planned;
- changes in the combined business due to potential divestitures or other requirements imposed by antitrust regulators;
- costs and delays in implementing common systems and procedures; and
- increased difficulties in managing our business due to the addition of international locations.

Many of these risks may be accentuated because the majority of Prosensa's operations, employees and customers are located 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 businesses. The failure to integrate the business operations of Prosensa successfully would have a material adverse effect on our business, financial condition and results of operations.

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

Even if the integration is successful, 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;
- certain other purchase accounting adjustments that we expect will be recorded in our financial statements in connection with the acquisition;

· 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.

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 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 will operate;

- complying with additional employment regulations in the new jurisdictions in which we will 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.

We will incur significant transaction, integration and restructuring costs in connection with the acquisition.

We have incurred significant transaction costs related to the acquisition. In addition, the combined business will incur integration and restructuring costs following the completion of the acquisition as we integrate Prosensa's businesses with our businesses. Although we expect that the realization of benefits and efficiencies related to the integration of the businesses may offset over time these transaction and integration and restructuring costs, no assurances can be made that this net benefit will be achieved in the near term, or at all, which could adversely affect our financial condition and results of operations.

Prosensa depends heavily on the success of drisapersen. Drisapersen is still in clinical development. If we are unable to commercialize drisapersen 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 Prosensa will depend heavily on the successful development and eventual commercialization of drisapersen.

In September 2013, Prosensa announced that the Phase 3 clinical trial of drisapersen 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 drisapersen, including retrospective and subgroup analyses, provide strong support for concluding that drisapersen showed clinically meaningful improvements over placebo in these trials, we cannot be sure that Prosensa's data will be sufficient to satisfy the EMA or the FDA. We may need to conduct additional clinical trials at significant delay and cost or abandon development of drisapersen altogether.

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

- we may not achieve market acceptance of drisapersen by physicians, patients and third-party payers;
- drisapersen may not have an acceptable safety profile following approval;
- we may not be able to manufacture drisapersen 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 drisapersen 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 drisapersen 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 drisapersen, Prosensa performed retrospective and subgroup analyses of the Phase 3 clinical trial and prior Phase 2 clinical trials of drisapersen that we believe provide strong support for concluding that drisapersen 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 an additional clinical trial or trials of drisapersen before we can apply for marketing approval.

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 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 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 drisapersen and our other product candidates acquired from Prosensa. If we are required to conduct additional clinical trials of drisapersen, 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 since the beginning of trading after our initial public offering have had 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;
 - manufacture, supply or distribution of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
 - progress of our integration of Prosensa;
 - 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 lawsuit against DRL to protect our patents relating to Kuvan;
 - 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; and
 - changes in company assessments or financial estimates by securities analysts.
- 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.

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.

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, 2014:

Location	Approximate Square Feet	Use	Lease Expiration Date
Several locations in Novato, California	228,000	Office, laboratory and warehouse	2016-2020
San Rafael facility, San Rafael, California	120,400	Corporate headquarters, office	NA: owned property
Galli Drive facility, Novato, California	91,500	Clinical and commercial manufacturing and laboratory	NA: owned property
Bel Marin Keys facility, Novato, California	83,900	Technical operations, finance, administration, and laboratory	NA: owned property
Digital Drive facility, Novato, California	45,000	Office and laboratory	NA: owned property
Leveroni Drive facility, Novato, California	38,400	Warehouse	NA: owned property
Dublin, Ireland	9,100	Office	2024
Shanbally facility, Cork, Ireland	142,000	Manufacturing	NA: owned property

Our administrative office space and plans to develop additional space are expected to be adequate for the foreseeable future. In addition to the above, we also maintain small offices in a variety of locations around the world. 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 Notice Letter from Dr. Reddy's Laboratories

As previously disclosed, we have received a paragraph IV notice letter, dated October 3, 2014, from DRL, notifying us that DRL has filed an ANDA seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book.

On November 17, 2014, we, together with Merck, filed a lawsuit against DRL in the United States District Court for the District of New Jersey alleging patent infringement for our patents relating to Kuvan.

On January 16, 2015, we, together with Merck, filed an Amended Complaint requesting a declaratory judgment that DRL has no legitimate basis to trigger the ANDA process, alleging that DRL did not have a proper ANDA because, upon information and belief, it did not submit proper bioequivalence data in support of its purported ANDA.

Paragraph IV Notice Letter from Par Pharmaceutical

We have 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.

Declaratory Judgment Action

On January 16, 2015, we filed a lawsuit in the United States District Court for the Southern District of New York, seeking a declaratory judgment that we are under no legal obligation to sell or otherwise provide samples of Kuvan (sapropterin dihydrochloride) to DRL. DRL seeks such samples to conduct bioequivalence testing in support of its proposed generic Kuvan (sapropterin dihydrochloride) product.

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
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
2013	Fourth Quarter	\$76.02	\$58.65
2013	Third Quarter	\$80.67	\$56.31
2013	Second Quarter	\$71.56	\$53.53
2013	First Quarter	\$62.96	\$49.71

On February 13, 2015, the last reported sale price on the NASDAQ Global Select Market for our common stock was \$100.79. 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, 2014.

Issuer Purchases of Equity Securities

We did not make any purchases of our common stock during the year ended December 31, 2014.

Holders

As of February 13, 2015, there were 53 holders of record of 159,135,697 outstanding shares of our common stock. Additionally, on such date, options to acquire 11.2 million shares of our common stock were outstanding.

Equity Compensation Plans

Plan Category	Number of securities to be issued	Weighted average exercise	Number of securities remaining for
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	upon exercise of outstanding options, warrants and rights	price of outstanding options, warrants and rights	future issuance under equity compensation plans (excluding securities reflected in column (a))
	(a)	(b)	(c)
Equity compensation plans			
approved by security holders	13,416,983	\$ 31.41	7,069,555
Equity compensation plans			
not approved by any security			
holders	460,884	\$ 34.66	1,866,197
Total	13,877,867		8,935,752

2012 Inducement Plan

On May 8, 2012, the Board of Directors approved the 2012 Inducement Plan (the 2012 Inducement Plan), which provides for grants of up to 750,000 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 Company's 2006 Share Incentive Plan as amended and restated on March 22, 2010 (as further amended, the Share Incentive Plan). The 2012 Inducement Plan expired in March 2013.

2014 Inducement Plan

On December 17 2014, the Compensation Committee of the Board of Directors 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 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 Share Incentive Plan and the 2012 Inducement Plan. The 2014 Inducement Plan expires on the date of the Company's 2015 annual meeting of stockholders.

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, 2009 in BioMarin common stock, the NASDAQ Composite Index (U.S.) and the NASDAQ Biotechnology Index. 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, 2009 in stock or index, including reinvestment of dividends.

	Fiscal Year Ending December 31,					
	2009	2010	2011	2012	2013	2014
BioMarin Pharmaceutical Inc.	\$100.00	\$143.17	\$182.78	\$261.56	\$374.00	\$480.60
NASDAQ Composite Index	100.00	117.61	118.70	139.00	196.83	223.74
NASDAQ Biotechnology Index	100.00	106.73	122.40	166.72	286.55	379.71

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Item 6. Selected Consolidated Financial Data

The information set forth below for the five years ended December 31, 2014 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 of U.S. dollars, except for per share data)				
	2014	2013	2012	2011	2010
Consolidated statements of operations data:					
REVENUES:					
Net product revenues	\$738,416	\$538,360	\$496,497	\$437,647	\$369,701
Collaborative agreement revenues	1,592	3,918	1,955	468	682
Royalty, license and other revenues	11,032	6,207	2,271	3,243	5,884
Total revenues	751,040	548,485	500,723	441,358	376,267
OPERATING EXPENSES:					
Cost of sales	129,764	95,742	91,830	84,023	70,285
Research and development	461,543	354,780	302,218	214,374	147,309
Selling, general and administrative	302,156	235,356	198,173	175,423	151,723
Intangible asset amortization and contingent consideration	17,968	18,614	18,717	1,428	6,406
Gain on sale of intangible asset	(67,500)	—	—	—	—
Total operating expenses	843,931	704,492	610,938	475,248	375,723
INCOME (LOSS) FROM OPERATIONS	(92,891)	(156,007)	(110,215)	(33,890)	544
Equity in the loss of BioMarin/Genzyme LLC	(877)	(1,149)	(1,221)	(2,426)	(2,991)
Interest income	5,937	3,083	2,584	2,934	4,112
Interest expense	(36,642)	(10,447)	(7,639)	(8,409)	(10,818)
Debt conversion expense	(674)	(12,965)	—	(1,896)	(13,728)
Net gain from sale of investments	—	—	—	—	902
Other income (expense)	279	982	(1,787)	60	489
LOSS BEFORE INCOME TAXES	(124,868)	(176,503)	(118,278)	(43,627)	(21,490)
Provision for (benefit from) income taxes	9,101	(150)	(3,931)	10,209	(227,309)
NET INCOME (LOSS)	\$(133,969)	\$(176,353)	\$(114,347)	\$(53,836)	\$205,819
NET INCOME (LOSS) PER SHARE, BASIC	\$(0.92)	\$(1.28)	\$(0.95)	\$(0.48)	\$2.00
NET INCOME (LOSS) PER SHARE, DILUTED	\$(0.92)	\$(1.28)	\$(0.95)	\$(0.48)	\$1.73
Weighted average common shares outstanding, basic	146,349	137,755	120,271	112,122	103,093
Weighted average common shares outstanding, diluted	146,349	137,755	120,271	112,122	125,674

	December 31,				
	(in thousands)				
	2014	2013	2012	2011	2010
Consolidated balance sheet data:					
Cash, cash equivalents and investments	\$1,043,048	\$1,052,423	\$563,798	\$289,477	\$402,283
Total current assets	1,425,629	1,137,418	743,431	469,802	504,260
Total assets	2,490,453	2,244,060	1,568,347	1,268,554	1,226,106
Long-term convertible senior notes	657,976	655,566	324,859	348,329	377,521
Total stockholders' equity	1,527,894	1,341,041	1,015,763	773,048	717,257

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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,
2014:				
Total revenue	\$ 151,552	\$ 191,787	\$ 176,847	\$ 230,854
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)
2013:				
Total revenue	\$ 127,928	\$ 136,810	\$ 136,874	\$ 146,873
Net loss	(39,810)	(21,533)	(53,020)	(61,990)
Net loss per share, basic	(0.31)	(0.15)	(0.38)	(0.43)
Net loss per share, diluted	(0.31)	(0.16)	(0.38)	(0.44)

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 U.S. generally accepted accounting principles 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 is comprised 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 Highlights

During 2014 and January 2015, we continued to advance our product pipeline. We made significant progress, in our Phase 3 clinical studies for pegvaliase (PEG PAL), reveglucosidase alfa (BMN 701) and talazoparib (BMN 673). The combination of our internal research programs, acquisition and partnerships will allow us to continue develop and commercialize innovative biopharmaceuticals for serious diseases and medical conditions. Below is a summary of our recent key accomplishments:

- Received U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) approval of Vimizim, a treatment for mucopolysaccharidosis Type IVA or Morquio Syndrome Type A, a lysosomal storage disorder;
- Announced preliminary data from ongoing Phase 1/2 Pivotal study of cerliponase alfa (BMN 190) for the treatment of late infantile neuronal ceroid lipofuscinosis (CLN2), a lysosomal storage disorder primarily affecting the brain.
- Announced the selection of BMN 270, an AAV VIII vector, a Factor VIII gene therapy drug development candidate, for the treatment of hemophilia A. We expect to file an investigational new drug application (IND) or equivalent for BMN 270 in the first quarter of 2015;
- Continued to advance the Phase 3 trial which is an open-label, 2:1 randomized, parallel, two-arm study of talazoparib, an orally available poly-ADP ribose polymerase inhibitor for the treatment of patients with certain cancers, as compared to the protocol-specified physicians' choice of chemotherapy in germline BRCA (gBRCA) mutated locally advanced and/or metastatic breast cancer patients who have received no more than two prior chemotherapy regimens for metastatic disease. We expect to complete the enrolment of this Phase 3 trial in the second half of 2015;
- Dosed first patient in Phase 2/3 INSPIRE trial with reveglucosidase alfa, an enzyme replacement therapy for Pompe Disease, a glycogen storage disorder;

- Received orphan drug designation for BMN 250, a novel fusion of alpha-N-acetylglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo B syndrome, or Mucopolysaccharidosis type IIIB (MPS IIIB). We expect to file an investigational new drug application (IND) or equivalent for BMN 250 in mid-2015;
- Completed dosing of three dose cohorts in the Phase 2 trial of BMN 111, a peptide therapeutic for the treatment of achondroplasia, the leading cause of dwarfism;
- Continued to advance the Phase 3 clinical trial for the development of pegvaliase, an enzyme substitution therapy for the treatment of phenylketonuria (PKU). We expect to report results from the trial in the first quarter of 2016; and

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

On January 15, 2015, we closed the initial offering period related to our offer to purchase all of the ordinary shares (the Prosensa Shares) of Prosensa Holding N.V, a public limited liability company organized under the laws of the Netherlands (Prosensa), and purchased 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 approximately \$620.7 million for 34,970,514 Prosensa Shares, representing approximately 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 the asset transfer and paid an additional \$20.8 million to the remaining Prosensa shareholders.

Outlook 2015

In 2015, 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 will 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 will continue to build-out our commercial organization to support the commercialization of Vimizim.

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 2015 operating objectives.

2014 Financial Highlights

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

	Years Ended December 31,		
	2014	2013	2012
Total net product revenues	\$738.4	\$538.4	\$496.5
Cost of sales	129.8	95.7	91.8
Research & Development (R&D) expense	461.5	354.8	302.2
Selling, general and administrative (SG&A) expense	302.2	235.4	198.2
Net loss	(134.0)	(176.4)	(114.3)
Stock-based compensation expense	86.4	64.4	48.0

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

Net product revenues were as follows (in millions):

	Years Ended December		
	31,		
	2014	2013	2012
Vimizim	\$77.3	\$0.1	\$—
Naglazyme	334.4	271.2	257.0
Kuvan	203.0	167.4	143.1
Aldurazyme	105.6	83.6	82.2
Firdapse	18.1	16.1	14.2
Total net product revenues	\$738.4	\$538.4	\$496.5

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

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

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

Selling, general and administrative (SG&A) expense primarily includes expenses associated with the commercialization of approved products and general and administrative costs to support our operations. These expenses include: product marketing and sales operations personnel; corporate facility operating expenses; information technology expenses and depreciation; and core corporate support functions, including human resources, finance and legal, and other external corporate costs such as insurance, legal fees and other professional services.

Our cash, cash equivalents, short-term investments and long-term investments totaled \$1,043.1 million as of December 31, 2014, compared to \$1,052.4 million as of December 31, 2013. 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 revenue 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 accounting principles generally accepted 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 in-process research and development (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 which 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 which may affect the accuracy or validity of such assumptions, estimates or actual results.

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

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

Our Consolidated Balance Sheets reflect net deferred tax assets that 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. 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. If it is more likely than not that we would not realize the deferred tax benefits, then all or a portion of the valuation allowance may need to be established. 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.

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

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 2014 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, 2014.

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 are primarily comprised 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.

In the U.S., our commercial products are generally sold to specialty pharmacies or end-users, such as hospitals, which act as retailers. We also sell Kuvan to Merck Serono S.A. (Merck Serono) at a price near its manufacturing cost, and Merck Serono resells 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 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, 2014 and 2013, accounts receivable included \$34.5 million and \$26.3 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.

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

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, 2014 and 2013, our allowance for doubtful accounts was \$0.5 million.

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.

Revenue Dilution Item	Percentage of Gross Sales Years Ended December		Description
	31, 2014	2013	
			Rebates payable to state Medicaid, other government programs and certain managed care providers
Rebates	0.3-7.3%	1.0-4.3%	Fees paid to authorized distributors
Distributor Fees	0.2 - 3.3%	0.2-3.6%	Discounts offered to customers for prompt payment of accounts receivable
Cash Discounts	1.0 -1.8%	0.7-1.9%	
Total	1.5 - 12.4%	1.9-9.8%	

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 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 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.

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

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.

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. 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.

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.

Recent Accounting Pronouncements

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

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

Net loss for the year ended December 31, 2013	\$(176.4)
Gain on sale of intangible asset	67.5
Increased gross profit from product sales	166.0
Increased R&D expense	(106.8)
Increased SG&A expense	(66.8)
Increased interest expense	(26.2)
Increased provision for income taxes	(9.3)
Decreased debt conversion expense	12.3
Other individually insignificant fluctuations	5.7
Net loss for the year ended December 31, 2014	\$(134.0)

In July 2014, we sold the Rare Pediatric Disease Priority Review Voucher (the PRV) we received in connection with the approval of Vimizim. In exchange for the PRV we received \$67.5 million from Regeneron Ireland (Regeneron). The proceeds from the sale of PRV were recognized as a gain on the sale of intangible asset. The increase in gross profit from product sales during the year ended December 31, 2014 as compared to the year ended December 31, 2013 was primarily a result of the commercial launch of Vimizim, additional Kuvan patients initiating therapy in the U.S., and additional Naglazyme patients initiating therapy globally. The increase in R&D expense was primarily attributed to the clinical trials of our late-stage development programs, licensing fees paid to a third-party to secure licenses related to the development of talazoparib and increased research on earlier stage development programs. The increase in selling, general and administrative expense was primarily due to increased sales and marketing expenses related to our commercial products and increased expenses related to the commercial launch of Vimizim. The increase in interest expense was attributed to our October 2013 debt offering.

Our net loss for the year ended December 31, 2013 was \$176.4 million, compared to a net loss of \$114.3 million for the year ended December 31, 2012. The increase in net loss was primarily a result of the following (in millions):

Net loss for the year ended December 31, 2012	\$(114.3)
Increased R&D expense	(52.6)
Increased SG&A expense	(37.2)
Debt conversion expense	(13.0)
Decreased benefit from income taxes	(3.8)
Increased gross profit from product sales	38.0
Increased royalty and license revenues	3.9
Other individually insignificant fluctuations	2.6
Net loss for the year ended December 31, 2013	\$(176.4)

The increase in gross profit from product sales during the year ended December 31, 2013 as compared to the year ended December 31, 2012 was primarily a result of additional Naglazyme patients initiating therapy globally and additional Kuvan patients initiating therapy in the U.S. The increase in R&D expense was primarily attributed to increased development expenses for our reveglucosidase alfa, talazoparib and pegvaliase programs. The increase in selling, general and administrative expense was primarily due to increased sales and marketing expenses related to our commercial products and increased pre-commercial Vimizim expenses.

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

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

Net Product Revenues, Cost of Sales and Gross Profit

Net product revenues were as follows (in millions):

	Years Ended December 31,				
	2014	2013	2012	2014 v. 2013	2013 v. 2012
Vimizim	\$77.3	\$0.1	\$—	\$ 77.2	\$ 0.1
Naglazyme	334.4	271.2	257.0	63.2	14.2
Kuvan	203.0	167.4	143.1	35.6	24.3
Aldurazyme	105.6	83.6	82.2	22.0	1.4
Firdapse	18.1	16.1	14.2	2.0	1.9
Total net product revenues	\$738.4	\$538.4	\$496.5	\$ 200.0	\$ 41.9

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

	Years Ended December 31,				
	2014	2013	2012	2014 v. 2013	2013 v. 2012
Aldurazyme revenue reported by Genzyme	\$228.8	\$212.4	\$193.1	\$ 16.4	\$ 19.3

	Years Ended December 31,				
	2014	2013	2012	2014 v. 2013	2013 v. 2012
Royalties earned from Genzyme	\$97.0	\$88.5	\$80.4	\$ 8.5	\$ 8.1
Incremental (previously recognized) Aldurazyme					
product transfer revenue	8.6	(4.9)	1.8	13.5	(6.7)
Total Aldurazyme net product revenues	\$105.6	\$83.6	\$82.2	\$ 22.0	\$ 1.4

2014 compared to 2013

The FDA and the EMA granted marketing approval for Vimizim in February 2014 and April 2014, respectively, and subsequently in other countries. We began marketing the product immediately following approval in each of these markets. Net product revenues for Vimizim 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. Vimizim gross margins were 87% for the year ended December 31, 2014. In future periods, we expect Vimizim gross margins to decline and approximate Naglazyme gross margins as we deplete previously expensed product.

Net product revenues for Naglazyme 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 attributed to new patients initiating therapy. The impact of foreign currency exchange rates on Naglazyme sales denominated in currencies other than the U.S. dollar was positive by \$0.9 million for the year ended December 31, 2014. Naglazyme gross margins were 86% for the years ended December 31, 2014 and 2013. Naglazyme gross margins are not expected to fluctuate significantly in the future.

Net product revenue for Kuvan 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 attributed to new patients initiating therapy. Kuvan gross margins were 84% 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%. Kuvan gross margins are not expected to fluctuate significantly in the future. 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.

We own several patents that cover Kuvan and have listed those patents in conjunction with that product in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). The Hatch-Waxman Act permits the FDA to approve abbreviated new drug applications (ANDA), for the generic versions of branded drugs. The Hatch-Waxman Act requires an ANDA applicant seeking FDA approval of the applicant's proposed generic product prior to the expiration of our Orange Book-listed patents to notify us of the application. Upon receipt of such a notice (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

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

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's review of the application may be completed. 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. Regardless of any litigation results, generic versions of Kuvan would be prohibited until the expiration of orphan drug exclusivity in June 2015, including pediatric exclusivity, at the earliest. We have also received New Patient Population exclusivity for Kuvan (sapropterin dihydrochloride) that expires in October 2017, including pediatric exclusivity. Thus, depending on the label of a generic product, generic versions of Kuvan may be prohibited until October 2017.

We have 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 has filed an ANDA seeking approval of a proposed generic version Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our Orange Book-listed patents. On November 17, 2014, we, together with Merck & Cie (Merck), filed a lawsuit against DRL in the United States District Court for the District of New Jersey alleging patent infringement for our patents relating to Kuvan. On January 16, 2015, we, together with Merck, filed an Amended Complaint requesting a declaratory judgment that DRL has no legitimate basis to trigger the ANDA process, alleging that DRL did not have a proper ANDA because, upon information and belief, it did not submit proper bioequivalence data in support of its purported ANDA.

We also have 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.

The filing of DRL's and Par's purported ANDAs in respect to Kuvan (sapropterin dihydrochloride) 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 (sapropterin dihydrochloride) and its use are not upheld in litigation, or if DRL and/or 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.

Net product revenue for Aldurazyme 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 revenue was attributed to new patients initiating therapy and an increase in shipments to Genzyme. Aldurazyme gross margins were 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.

Net product revenue for Firdapse for the year ended December 31, 2014 totaled \$18.1 million, compared to \$16.1 million for the year ended December 31, 2013. The increase in Firdapse net product revenues was attributed to new patients initiating therapy. Firdapse gross margins for the years ended December 31, 2014 and 2013 were 77%. Cost of goods sold for the each of the years ended December 31, 2014 and 2013 reflect royalties paid to third-parties of approximately 8%. Firdapse gross margins for the year ended December 31, 2014 were consistent with expectations and are not expected to fluctuate significantly in the future.

Total cost of sales for the year ended December 31, 2014 were \$129.8 million, compared to \$95.7 million for the year ended December 31, 2013. The increase in cost of sales was primarily attributed to the increase in product sales.

2013 compared to 2012

Net product revenues for Naglazyme for the year ended December 31, 2013 totaled \$271.2 million, of which \$233.5 million was earned from customers based outside the U.S., compared to \$257.0 million for the year ended December 31, 2012, of which \$222.8 million was earned from customers based outside the U.S. The increase in Naglazyme net product revenues was attributed to new patients initiating therapy. The impact of foreign currency exchange rates on Naglazyme sales denominated in currencies other than the U.S. dollar was negative by \$1.2 million for the year ended December 31, 2013. Naglazyme gross margins for 2013 were 86%, compared to 2012 when gross margins were 85%. Naglazyme gross margins for the year ended December 31, 2013 were consistent with expectations and are not expected to fluctuate significantly in the future.

Net product revenue for Kuvan for the year ended December 31, 2013 was \$167.4 million, compared to \$143.1 million during 2012. The increase in Kuvan net product revenues in 2013 was attributed to new patients initiating therapy. Kuvan gross margins for 2013 were 84%, compared to 2012 when gross margins were 83%. Cost of goods sold for the years ended December 31, 2013 and 2012 reflect royalties paid to third-parties of approximately 10%. Kuvan gross margins for the year ended December 31, 2013 were consistent with expectations and are not expected to fluctuate significantly in the future. The 4% royalties earned from Merck Serono's net sales of Kuvan for the year ended December 31, 2013 were \$2.0 million, compared to \$1.9 million during 2012.

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Net product revenue for Firdapse for the year ended December 31, 2013 was \$16.1 million, compared to \$14.2 million during 2012. Firdapse gross margins for the year ended December 31, 2013 were 77%, compared to 2012 when gross margins were 80%. Cost of goods sold for the years ended December 31, 2013 and 2012 reflect royalties paid to third-parties of approximately 8%. Firdapse gross margins decreased during 2013 due to increased manufacturing costs and the depletion of manufactured product that was previously expensed as research and development expense. Firdapse gross margins for the year ended December 31, 2013 were consistent with expectations and are not expected to fluctuate significantly in the future.

Aldurazyme gross margins were 68% in each of the years ended December 31, 2013 and 2012. 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, 2013 was \$95.7 million, compared to \$91.8 million for the year ended December 31, 2012. The increase in cost of sales was primarily attributed to the increase in product sales.

Research and Development

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 \$461.5 million for the year ended December 31, 2014, from \$354.8 million for the year ended December 31, 2013. The increase in R&D expense was primarily a result of the following (in millions):

R&D expense for the year ended December 31, 2013	\$354.8
Increased development expense on early development stage programs	32.7
Increased talazoparib development expense	30.3
Increased cerliponase alfa development expense	25.7
Increased pegvaliase development expense	16.0
Increased BMN 111 development expense	7.5
Increased reveglucosidase alfa development expense	5.5
Increased stock-based compensation expense	6.1
Decreased Vimizim development expense	(18.4)
Decreased development expense related to mature commercial products	(5.5)
Increased non-allocated R&D expense and other net changes	6.8
R&D expense for the year ended December 31, 2014	\$461.5

The increase in development expense on early development stage programs was primarily attributed to the pre-clinical activity related to BMN 270 and BMN 250 and development costs related to the programs acquired from Zacharon Pharmaceuticals, Inc. (Zacharon). The increase in talazoparib development expense was attributed 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 attributed to increased clinical trial activities related to this product candidate. The increases in cerliponase alfa and

BMN 111 development expense were attributed to increased clinical activities related to these product candidates. The decrease in reveglucosidase alfa development expense was attributed to a decline in clinical manufacturing costs related to the product candidate, offset by an increase in clinical trial expense. The increase in stock-based compensation is attributed 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 and the recognition of approximately \$2.8 million of expense related to performance awards granted to certain executive officers. The increase in non-allocated R&D expense is primarily attributed to an increase in R&D personnel costs and facility costs that are not allocated to specific programs, which includes a \$7.2 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.

During 2015, we expect our R&D spending to increase over 2014 levels due to our drisapersen, pegvaliase, talazoparib, reveglucosidase alfa, BMN 111 and cerliponase alfa programs progressing, including a few of those programs progressing to more advanced phases of clinical studies. We acquired drisapersen, which is in Phase 3 clinical trials, in January 2015 from Prosensa. Phase

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

3 clinical trials for pegvaliase and talazoparib were initiated in the second and fourth quarters of 2013, respectively, and we initiated a Phase 3 trial of reveglucosidase alfa in the second quarter of 2014. We also expect increased spending on pre-clinical and clinical activities for our early development stage programs including BMN 270, programs acquired from Zacharon and BMN 250. 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.

R&D expense increased to \$354.8 million for the year ended December 31, 2013, from \$302.2 million for the year ended December 31, 2012. The increase in R&D expense was primarily a result of the following (in millions):

R&D expense for the year ended December 31, 2012	\$302.2
Increased pegvaliase development expense	27.8
Increased talazoparib development expense	18.1
Increased reveglucosidase alfa development expense	14.0
Increased development expense on early development stage programs	13.2
Increased stock-based compensation expense	7.0
Increased development expense related to commercial products	4.1
Increased BMN 111 development expense	2.9
Increased cerliponase alfa development expense	2.7
Decreased Vimizim development expense	(15.0)
Decrease in non-allocated R&D expense and other net changes	(22.2)
R&D expense for the year ended December 31, 2013	\$354.8

The increase in pegvaliase, talazoparib and reveglucosidase alfa development expense was attributed to increased clinical trial activities related to these product candidates. The increase in development expense on early stage programs was primarily attributed to the pre-clinical activity related to BMN 270, a Factor VIII gene therapy program for Hemophilia A, and development costs related to the programs acquired from Zacharon. The increase in stock-based compensation was primarily attributed to an increase in the number of options outstanding due to an increased number of employees and an increase in the weighted-average fair value of the equity awards granted during 2013. The increases in cerliponase alfa and BMN 111 development expense were attributed to increased pre-clinical activities related to these product candidates. During the first quarter of 2013, we evaluated the facts and circumstances supporting recoverability of pre-launch manufacturing costs related to Vimizim and concluded that recoverability was probable, resulting in the capitalization of approximately \$40.5 million pre-launch manufacturing costs during 2013. Pre-launch Vimizim manufacturing costs incurred during 2012 were expensed to R&D expense as significant uncertainty existed over the recoverability of the costs. The decrease in non-allocated R&D expense is primarily attributed to a decline in research and development personnel costs and facility costs that are not allocated to specific programs.

Selling, General and Administrative

SG&A expense increased to \$302.2 million for the year ended December 31, 2014, from \$235.4 million for the year ended December 31, 2013. The increase in SG&A expenses was primarily a result of the following (in millions):

SG&A expense for the year ended December 31, 2013	\$235.4
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Increased Vimizim commercial launch expenses	24.1
Increased stock-based compensation	14.7
Increased sales and marketing expenses related to mature commercial products	1.8
Net increase in corporate support and other administrative expenses	26.2
SG&A for the year ended December 31, 2014	\$302.2

We received regulatory approval to market Vimizim in the U.S. and the EU during 2014. The increase in commercial launch expense is consistent with the timing of these approvals. The increase in stock-based compensation is attributed 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 and the recognition of approximately \$10.1 million of expense related to performance awards granted to certain executive officers. We continue to incur sales and marketing expense for Naglazyme and Kuvan as a result of continued expansion of our international and U.S. activities, respectively. The increase in corporate support and other administrative expenses is

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

primarily attributed 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 \$2.8 million gain on the early termination of our SRCC lease and the recognition of the remaining deferred rent and asset retirement liabilities upon acquisition of the SRCC. We expect SG&A expense to increase in future periods as a result of the international expansion of Naglazyme and Vimizim, the U.S. commercialization activities for Kuvan, and the increase in administrative support required for our expanding operations.

SG&A expense increased to \$235.4 million for the year ended December 31, 2013, from \$198.2 million for the year ended December 31, 2012. The increase in SG&A was primarily a result of the following (in millions):

SG&A expense for the year ended December 31, 2012	\$198.2
Increased sales and marketing expense related to commercial products	10.7
Increased Vimizim pre-commercial expense	15.4
Increased stock-based compensation expense	9.5
Increased foreign exchange losses on unhedged transactions	1.3
Net increase in corporate support and other administrative expense	0.3
SG&A expense for the year ended December 31, 2013	\$235.4

We incurred sales and marketing expense for Naglazyme and Kuvan as a result of continued expansion of our international and U.S. activities, respectively. The increase in stock-based compensation was attributed 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 2013 and the recognition of approximately \$4.9 million of expense related to performance awards granted to certain executive officers.

Intangible Asset Amortization and Contingent Consideration

Intangible asset amortization and contingent consideration expense is comprised of changes in the fair value of contingent acquisition consideration payable to former stockholders of our acquired businesses, impairment loss (if any) on intangible assets and amortization of intangible assets. 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,		
	2014	2013	2012
Changes in the fair value of contingent acquisition consideration payable	\$13.0	\$14.5	\$8.8
Impairment loss on intangible assets	—	0.9	6.7
Amortization of intangible assets	5.0	3.2	3.2
Total intangible asset amortization and contingent consideration	\$18.0	\$18.6	\$18.7

The changes in the fair value of the contingent acquisition consideration payable were primarily attributed to changes in the estimated probability of achieving development milestones based on the current status of the related development programs as well as changes in the discount rate utilized in the fair value calculations. During the year ended December 31, 2014, the majority of the changes related to the development progress of reveglucosidase alfa.

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

In the second quarter of 2013, we recorded an impairment charge of \$0.9 million related to IPR&D assets related to acquired pre-clinical compounds based on the status of current development efforts and the related discounted cash flows that no longer supported the carrying-value of the IPR&D assets.

In the first quarter of 2012, we recorded an impairment charge of \$6.7 million related to the U.S. Firdapse IPR&D assets based on the status of business development efforts at the time and the related discounted cash flows that no longer supported the carrying-value of the IPR&D assets. The IPR&D assets impaired were associated with the marketing rights for Firdapse in the U.S. See Note 6 to our accompanying Consolidated Financial Statements for additional discussion.

Equity in the Loss of BioMarin/Genzyme LLC

Equity in the loss of BioMarin/Genzyme LLC (the LLC), our joint venture with Genzyme, includes our 50% share of the LLC's loss for the period. The LLC's operations consist primarily of certain R&D activities and the intellectual property that are managed by the LLC, with costs shared equally by BioMarin and Genzyme.

Equity in the loss of the LLC totaled \$0.9 million for the year ended December 31, 2014, compared to \$1.1 million and \$1.2 million for the years ended December 31, 2013 and 2012, respectively.

Interest Income

We invest our cash, short-term and long-term investments in government and other high credit quality securities in order to limit default and market risk. Interest income totaled \$5.9 million for the year ended December 31, 2014, compared to \$3.1 million and \$2.6 million for the years ended December 31, 2013 and 2012, respectively. The increase in interest income during the year ended December 31, 2014, as compared to the years ended December 31, 2013 and 2012 was primarily due to higher cash and investment balances. We do not expect future interest income to fluctuate significantly over the next twelve months.

Interest Expense and Debt Conversion Expense

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

	Years Ended		
	December 31,		
	2014	2013	2012
Coupon interest	\$9.4	\$4.5	\$6.6
Amortization of issuance costs	3.3	1.1	1.0
Accretion of discount on convertible notes	23.9	4.8	—
Total interest expense	\$36.6	\$10.4	\$7.6

The increased interest expense in the year ended December 31, 2014, compared to the years ended December 31, 2013 and 2012 was attributed to our October 2013 issuance of \$750.0 million in aggregate principal amount of senior subordinated convertible debt. We do not expect future interest expense to fluctuate significantly over the next twelve months. See Note 13 to the accompanying Consolidated Financial Statements for additional information regarding our Convertible Debt.

During 2014, we recognized Debt Conversion Expense of \$0.7 million in connection with the early conversion of \$16.5 million in aggregate principal amount of our senior subordinated convertible notes due in 2017 (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 \$262.8 million in aggregate principal amount of the 2017 Notes. The increase in interest expense in 2013 compared to 2012 was attributed to the October 2013 issuance of \$750.0 million of senior subordinated convertible notes

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

Provision for (Benefit from) Income Taxes

For the year ended December 31, 2014 we recognized an income tax expense of \$9.1 million, compared to an income tax benefit of \$0.2 million and \$3.9 million in the years ended December 31, 2013 and 2012, respectively. Provision for (benefit from) income taxes for 2014 and 2013 consisted of state, federal and foreign current tax expense which was offset by deferred tax benefits from federal orphan drug credits, federal R&D credits and California R&D credits. The 2014 provision was also reduced by a renewable energy investment tax credit under the flow-through method. The provisions for 2014 and 2013 were further reduced by the tax benefit related to stock option exercises during the years ended December 31, 2014 and 2013. In both 2014 and 2013, 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 of \$0.8 million and \$1.2 million related to R&D expenses incurred during 2014 and 2012, respectively, as a discrete item during the year ended December 31, 2014 and 2013, respectively, which further increased our income tax benefit for the current period provision. In 2013, these discrete 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. See Note 15 to our accompanying Consolidated Financial Statements for additional discussion of 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 \$177.5 million of foreign net losses. Other foreign operations generated U.S. GAAP income of approximately \$3.2 million with an effective tax rate of approximately 104%.

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 revenue 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 October 2013 debt offering and our March 2014 and January 2015 equity offerings.

We consider the unrepatriated cumulative earnings of certain of our foreign subsidiaries to be indefinitely invested outside the U.S. As of December 31, 2014, \$109.6 million of our \$1,043.1 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. In managing our liquidity needs in the U.S., we do not rely on the unrepatriated earnings as a source of funds and we have not provided for U.S. federal or state income taxes on these undistributed foreign earnings.

We 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 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.

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

Our financial condition as of December 31 was as follows (in millions):

	2014	2013	2012	2014 v. 2013	2013 v. 2012
Cash and cash equivalents	\$875.5	\$568.8	\$180.5	\$ 306.7	\$ 388.3
Short-term investments	69.7	215.9	267.3	(146.2)	(51.4)
Long-term investments	97.9	267.7	116.0	(169.8)	151.7
Cash, cash equivalents and investments	\$1,043.1	\$1,052.4	\$563.8	\$ (9.3)	\$ 488.6
Current assets	\$1,425.6	\$1,137.4	\$743.4	\$ 288.2	\$ 394.0
Current liabilities	235.7	183.3	170.4	52.4	12.9
Working capital	\$1,189.9	\$954.1	\$573.0	\$ 235.8	\$ 381.1
Convertible debt	\$658.0	\$655.6	\$348.2	\$ 2.4	\$ 307.4

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

	2014	2013	2012	2014 v. 2013	2013 v. 2012
Cash and cash equivalents at the beginning of the period	\$568.8	\$180.5	\$46.3	\$ 388.3	\$ 134.2
Net cash provided by (used in) operating activities	(73.5)	(59.6)	17.6	(13.9)	(77.2)
Net cash provided by (used in) investing activities	194.5	(298.8)	(195.6)	493.3	(103.2)
Net cash provided by financing activities	185.7	746.7	312.2	(561.0)	434.5
Cash and cash equivalents at the end of the period	\$875.5	\$568.8	\$180.5	\$ 306.7	\$ 388.3
Short-term and long-term investments	167.6	483.6	383.3	(316.0)	100.3
Cash, cash equivalents and investments	\$1,043.1	\$1,052.4	\$563.8	\$ (9.3)	\$ 488.6

Working Capital

Working capital increased by \$235.8 million, from \$954.1 million at December 31, 2013 to \$1,189.9 million at December 31, 2014. The increase in working capital was attributed to the following (in millions):

Working capital at December 31, 2013	\$954.1
Increased cash, cash equivalents and short-term investments	160.5
Increased accounts payable and accrued liabilities	(52.5)
Net increase in other current operating assets	127.8
Working capital at December 31, 2014	\$1,189.9

The increase in cash, cash equivalents and short-term investments at December 31, 2014 from December 31, 2013 was primarily attributed to the net proceeds of \$117.5 million from our March 2014 public offering of common stock, proceeds of \$79.9 million from employee stock option exercises and employee stock purchase plan contributions and the \$67.5 million proceeds from the sale of the PVR.

The net increase in other current operating assets is primarily comprised of a \$36.8 million increase in inventory, a \$26.7 million increase in accounts receivable and a \$63.6 million increase in other current assets. The increase in inventory was primarily attributed to building of inventories for all commercial products, including Vimizim (which was approved in 2014), to meet anticipated future sales demand. The increase in accounts receivable is attributed to increased revenues and the timing of net product revenues and cash receipts from customers.

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 authority. 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, 2014, approximately 8% of our outstanding accounts receivable relate to such countries. See Note 19 to our accompanying Consolidated Financial Statements for additional discussion. We also sell our products in

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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 Provided by (Used in) Operating Activities

Cash used in operating activities for the year ended December 31, 2014 was \$73.5 million, compared to cash used in operating activities of \$59.6 million for the year ended December 31, 2013. The increase in cash used in operating activities was primarily attributed to the \$42.4 million increase in our net loss and a \$17.9 million decrease in collection of accounts receivable. The increase in our net loss is primarily attributed 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.

Cash used in operating activities for the year ended December 31, 2013 was \$59.6 million, compared to cash provided by operating activities of \$17.6 million for the year ended December 31, 2012. The increase in cash used in operating activities was primarily related to the \$62.0 million increase in our net loss and a \$35.3 million inventory increase, offset by debt conversion expense of \$13.0 million. The increase in our net loss is primarily attributed to increased R&D expense related to increased clinical trial activities for our product candidates pegvaliase, talazoparib and reveglucosidase alfa, pre-commercial expense for Vimizim and increased sales and marketing expense related to continued expansion of our international and U.S activities for Naglazyme and Kuvan, respectively.

Cash Provided by (Used in) Investing Activities

Net cash provided by investing activities during the year ended December 31, 2014 was \$194.5 million compared to net cash used in investing activities of \$298.8 million and \$195.6 million during the years ended December 31, 2013 and 2012, respectively. Our investing activities have consisted primarily of purchases and sales and maturities of investments and capital expenditures, such as manufacturing equipment and facility construction and improvements. The increase in net cash provided by investing activities for the year ended December 31, 2014 compared to the year ended December 31, 2013 was primarily comprised 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 \$54.0 million increase in capital expenditures and a \$52.3 million increase in the investment in convertible notes. The increase in net cash used in investing activities for the year ended December 31, 2013 compared to the year ended December 31, 2012 was primarily comprised of a \$20.6 million increase in capital expenditures, a \$9.9 million increase in business acquisitions and the deposit of \$116.5 million in an escrow account for the purchase of the SRCC, offset by an increase in net maturities of investment securities of \$37.9 million. During 2015, we expect to make significant capital investments in our Shanbally, Ireland manufacturing facility to enable future commercial manufacturing of our products at the facility and our corporate headquarters to accommodate anticipated headcount growth.

Cash Provided by Financing Activities

Net cash provided by financing activities for the year ended December 31, 2014 was \$185.7 million, compared to net cash provided by financing activities of \$746.7 million and \$312.2 million for the years ended December 31, 2013 and 2012, respectively. Historically, our financing activities primarily included payments related to our contingent acquisition obligations, payments related to our convertible debt obligations and proceeds from employee stock purchases under the ESPP and employee stock option exercises. The decrease in net cash provided by financing

activities for the year ended December 31, 2014 was primarily attributed 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 proceeds from employee stock option exercises and ESPP contributions of \$7.3 million, offset by a \$1.6 million increase in payments of contingent acquisition consideration. The increase in net cash provided by financing activities for the year ended December 31, 2013 was primarily attributed to an increase of \$726.2 million in net proceeds from our October 2013 offering of senior subordinated convertible notes, offset by decreased proceeds from stock option exercises and ESPP contribution of \$10.5 million, increased taxes paid for net settlement of equity awards of \$4.7 million, increased debt conversion expense of \$13.0 million and \$29.8 million used to purchase capped calls in connection with our October 2013 offering of senior subordinated convertible notes.

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

Other Information

On January 15, 2015, we closed the initial offering period related to our offer to purchase all of the ordinary shares (Prosensa Shares) of Prosensa Holding N.V, a public limited liability company organized under the laws of the Netherlands (Prosensa), and purchased 93.4% of the Prosensa Shares and immediately launched a subsequent offering period that expired on January 29, 2015. On January 15, 2015, we paid approximately \$659.3 million for approximately 35 million Prosensa Shares, representing 96.8% of all of the outstanding Prosensa Shares and options that vested pursuant to the definitive purchase agreement. On February 12, 2015, we completed the asset transfer and paid \$20.8 million to the remaining Prosensa shareholders. Effective February 12, 2015, Prosensa has been dissolved and is in liquidation under Dutch law

On January 27, 2015, we sold approximately 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 \$888.2 million from this public offering.

On March 10, 2014, we sold 1.5 million shares of our common stock in an underwritten public offering pursuant to an effective registration statement previously filed with the SEC. We received net proceeds of approximately \$117.5 million from this public offering.

On October 15, 2013, we completed an offering of \$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 2018 (the 2018 Notes) and \$375.0 million in aggregate principal amount of the 1.50% senior subordinated convertible notes due in 2020 (the 2020 Notes and together with the 2018 Notes, the Notes). The net proceeds from the offering were \$696.4 million, after deducting commissions and offering expenses and the purchase of capped calls. The Notes were issued at face value and accrue interest at their stated annual rates which is payable semiannually in arrears on April 15 and October 15 of each year beginning on April 15, 2014. See Note 13 to our accompanying Consolidated Financial Statements for additional discussion.

Our \$790.6 million (undiscounted) of total convertible debt as of December 31, 2014 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 4, 2013, we acquired Zacharon, which focused on developing small molecules targeting pathways of glycan and glycolipid metabolism, for a net cash upfront payment of \$9.7 million. In connection with the acquisition, we agreed to pay the Zacharon stockholders additional consideration in future periods of up to \$134.0 million (undiscounted) in milestone payments if certain clinical, development and sales milestones are met.

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 or maintain 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; and

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 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 each of the three years ended December 31 and the period since inception of the major programs were as follows (in millions):

	2014	2013	2012	Since Program Inception
Vimizim	\$63.6	\$82.0	\$97.0	\$ 357.4
Talazoparib (BMN 673)	59.8	29.5	11.4	116.4
Reveglucosidase alfa (BMN 701)	51.1	45.6	31.6	148.3
BMN 111	22.5	15.0	12.1	69.4
Cerliponase alfa (BMN 190)	39.6	13.8	11.1	71.0
Pegvaliase (PEG PAL)	70.5	54.5	26.7	238.2
Mature approved products	31.8	37.3	33.2	403.3
Not allocated to specific major current projects	122.6	77.1	79.1	Not meaningful
Totals	\$461.5	\$354.8	\$302.2	

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;
- manufacture, supply or distribution of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- progress of our integration of Prosensa;
- 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 lawsuit against DRL to protect our patents relating to Kuvan;

- 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; and
- changes in company assessments or financial estimates by securities analysts.

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 for non-cancelable purchase commitments as of December 31, 2014 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	\$0.8	\$41.7	\$—	\$—	\$42.5
2018 Notes and related interest	2.8	5.6	377.8	—	386.2
2020 Notes and related interest	5.6	11.2	11.6	380.6	409.0
Operating leases	5.7	11.9	2.7	0.1	20.4
R&D and purchase commitments	44.8	14.0	2.2	—	61.0
Total	\$59.7	\$84.4	\$394.3	\$380.7	\$919.1

We are also subject to contingent payments totaling approximately \$1,398.7 million as of December 31, 2014, which are due upon achievement of certain regulatory and licensing milestones if they occur before certain dates in the future and the acquisition of Prosensa. Of this amount, \$52.8 million relates to programs that are no longer being developed.

In January 2015, we made payments totaling \$659.3 million in connection with the closing of the Prosensa tender offer to acquire the Prosensa Shares which were tendered and options which vested pursuant to the definitive purchase agreement. Additionally, in February 2015, we completed the asset transfer and paid \$20.8 million to the remaining Prosensa shareholders. The payments related to the acquisition of Prosensa were included in the total contingent payments of \$1,398.7 million as of December 31, 2014.

Item 7A. Quantitative and Qualitative Disclosure About Market Risk

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 \$40.6 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, 2014 the fair value of our convertible debt was \$1,079.8 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, 2014, 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, 2014, 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 \$2.6 million. Changes in interest rates may affect the fair value of our investment portfolio. However, we will not recognize such gains or losses in our Consolidated 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 presents the carrying value of our cash and investment portfolio, which approximates fair value at December 31, 2014 (in millions):

	Carrying Value	
Cash and cash equivalents	\$875,486	*
Short-term investments	69,706	**
Long-term investments	97,856	***
Total	\$1,043,048	

*74% of cash and cash equivalents are invested in money market instruments and 26% in cash.

**78% of short-term investments are invested in certificates of deposit and 22% in corporate debt securities.

***81% of long-term investments are invested in corporate debt securities and 19% in certificates of deposit

Foreign Currency Exchange Rate Risk

We transact business in various foreign currencies, primarily in Euros, British Pounds and Brazilian Real. Accordingly, we are subject to exposure from movements in foreign currency exchange rates of the Euro from sales of our products in Europe and operating expenses in the United Kingdom, other European countries and Brazil which are denominated in British Pounds, Euros and Real, respectively.

We hedge a portion of our net position in assets and liabilities denominated in Euros using forward foreign currency exchange contracts. We also use forward currency exchange contracts to hedge a percentage of our forecasted Euro-denominated revenue, as well as our operating expenses denominated in Brazilian Reais. Our hedging policy is designed to reduce the impact of foreign currency exchange rate movements. We mitigate short-term foreign currency exposure resulting from currency fluctuations by entering into forward foreign currency exchange contracts. These contracts have maturities ranging from one month to 26 months.

As of December 31, 2014, we had forward foreign currency exchange contracts to sell approximately 198.8 million Euros and 4.3 million British Pounds. As of December 31, 2014, our outstanding forward foreign currency exchange contracts had a net fair value of \$15.9 million, of which \$10.5 million was included in other current assets, \$5.4 million was included in other assets and \$12,000 was included in accounts payable and accrued expenses on our accompanying Consolidated Balance Sheets.

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 currently do not use financial instruments to hedge operating expenses denominated in local currencies in Europe. Instead, we believe that a natural hedge exists, in that local currency revenue substantially offsets the local currency operating expenses. We regularly review our hedging program and may, as part of this review, make changes to the program.

Based on our overall foreign currency exchange rate exposures at December 31, 2014, 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 and investments by approximately \$3.7 million. We expect to enter into new transactions based in foreign currencies that could be impacted by changes in exchange rates.

At December 31, 2014, we had cash of approximately \$56.0 million denominated in foreign currencies, which represented approximately 5% of our total cash and investment portfolio. As a result, our cash and investment portfolio is subject to limited amounts of foreign currency exchange rate risk.

Item 8. Financial Statements and Supplementary Data

The information required to be filed in this item appears on pages F-1 to F-45 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 in Rule 13a-15(f) under the Exchange Act as of December 31, 2014. 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, we believe our internal control over financial reporting as of December 31, 2014 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 from 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 generally accepted accounting principles. 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 generally accepted accounting principles, 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

We incorporate information regarding our directors, executive officers and corporate governance into this section by reference from sections captioned “Election of Directors” and “Executive Officers” in the proxy statement for our 2015 annual meeting of stockholders.

Item 11. Executive Compensation

We incorporate information regarding executive compensation into this section by reference from the section captioned “Executive Compensation” in the proxy statement for our 2015 annual meeting of stockholders.

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

We incorporate information regarding security ownership of our beneficial owners, management and related stockholder matters into this section by reference from the section captioned “Security Ownership of Certain Beneficial Owners” in the proxy statement for our 2015 annual meeting of stockholders. We incorporate information regarding the securities authorized for issuance under our equity compensation plans into this section by reference from the section captioned “Equity Compensation Plan Information” in the proxy statement for our 2015 annual meeting of stockholders.

Item 13. Certain Relationships and Related Transactions and Director Independence

We incorporate information regarding certain relationships, related transactions and director independence into this section by reference from the section captioned “Transactions with Related Persons, Promoters and Certain Control Persons” in the proxy statement for our 2015 annual meeting of stockholders.

Item 14. Principal Accounting Fees and Services

We incorporate information regarding our principal accountant fees and services into this section by reference from the section captioned “Independent Registered Public Accounting Firm” in the proxy statement for our 2015 annual meeting of stockholders.

Part IV

Item 15. Exhibits, Financial Statement Schedules

Financial Statements

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Consolidated Financial Statements as of December 31, 2014 and 2013 and for the three years ended December 31, 2014:	
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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, which is incorporated by reference herein.
- 3.1 Amended and Restated Certificate of Incorporation, 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, 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, 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, which is incorporated herein by reference.
- 3.4 Amended and Restated By-Laws of BioMarin Pharmaceutical Inc., previously filed with the SEC on December 23, 2010 as Exhibit 3.1 to the Company's Current Report on Form 8-K, 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, 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, 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, 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, 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, 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, which is incorporated herein by reference.
- 4.7

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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, 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, 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, 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, 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, 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, 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, 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, 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, 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, which is incorporated herein by reference.
- 10.10† Amended and Restated BioMarin Pharmaceutical Inc. 2006 Share Incentive Plan adopted on May 12, 2010, as amended on March 28, 2013, previously filed with the SEC on May 16, 2013 as Exhibit 4.5 to the Company's Registration Statement on Form S-8, 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, 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, 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, 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, which is incorporated herein by reference.
- 10.15† Amended and Restated Employment Agreement with Robert A. Baffi effective January 1, 2009 previously filed with the SEC on December 23, 2008 as Exhibit 10.3 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
- 10.16† Amended and Restated Employment Agreement with G. Eric Davis effective January 1, 2009, previously filed with the SEC on December 23, 2008 as Exhibit 10.6 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
- 10.17†

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Employment Agreement with Henry Fuchs, effective March 18, 2009, previously filed with the SEC on March 23, 2009 as Exhibit 10.1 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.

- 10.18 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.
- 10.19 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, 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 Development, License and Commercialization Agreement dated May 13, 2005, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on July 6, 2005 as Exhibit 10.1 to the Company's Current Report on Form 8-K/A, 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 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.
- 10.22 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, 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.
- 10.23 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, 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.
- 10.24 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, 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.
- 10.25†Employment Agreement with Daniel Spiegelman effective as of May 29, 2012, previously filed with the SEC on May 9, 2012 as Exhibit 10.1 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
- 10.26†Amendment No. 1 to Employment Agreement with Robert A. Baffi dated as of May 8, 2012, previously filed with the SEC on May 9, 2012 as Exhibit 10.4 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
- 10.27†Amendment No. 1 to Employment Agreement with G. Eric Davis dated as of May 8, 2012, previously filed with the SEC on May 9, 2012 as Exhibit 10.5 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
- 10.28†Amendment No. 1 to Employment Agreement with Henry J. Fuchs dated as of May 8, 2012, previously filed with the SEC on May 9, 2012 as Exhibit 10.6 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
- 10.29†Amendment No. 2 to Employment Agreement with Robert A. Baffi dated as of May 24, 2012, previously filed with the SEC on May 24, 2012 as Exhibit 10.1 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
- 10.30†

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Amendment No. 2 to Employment Agreement with Henry J. Fuchs dated as of May 24, 2012, previously filed with the SEC on May 24, 2012 as Exhibit 10.2 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.

10.31†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, which is incorporated herein by reference.

10.32†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, which is incorporated herein by reference.

10.33†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, which is incorporated herein by reference.

10.34†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, which is incorporated herein by reference.

- 10.35†Employment Agreement with Jeffrey R. Ajer dated as of September 5, 2012, previously filed with the SEC on September 5, 2012 as Exhibit 10.1 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
- 10.36†Amendment No. 1 to Employment Agreement with Daniel Spiegelman dated as of December 17, 2012, previously filed with the SEC on December 18, 2012 as Exhibit 10.1 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
- 10.37†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, which is incorporated herein by reference.
- 10.38†Amendment No. 1 to Employment Agreement with Jeffery R. Ajer dated as of December 17, 2012, previously filed with the SEC on December 18, 2012 as Exhibit 10.3 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
- 10.39†Amendment No. 3 to Employment Agreement with Robert A. Baffi dated as of December 17, 2012, previously filed with the SEC on December 18, 2012 as Exhibit 10.4 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
- 10.40†Amendment No. 3 to Employment Agreement with Henry J. Fuchs dated as of December 17, 2012, previously filed with the SEC on December 18, 2012 as Exhibit 10.5 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
- 10.41†Amendment No. 2 to Employment Agreement with G. Eric Davis dated as of December 17, 2012, previously filed with the SEC on December 18, 2012 as Exhibit 10.6 to the Company's Current Report on Form 8-K, which is incorporated herein by reference.
- 10.42 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, which is incorporated herein by reference.
- 10.43 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, which is incorporated herein by reference.
- 10.44 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, which is incorporated herein by reference.
- 10.45 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, which is incorporated herein by reference.
- 10.46 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, which is incorporated herein by reference.

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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, which is incorporated herein by reference.

- 10.48 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, which is incorporated herein by reference.
- 10.49 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, which is incorporated herein by reference.
- 10.50 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, which is incorporated herein by reference.
- 10.51 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, which is incorporated herein by reference.

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- 10.52 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, which is incorporated herein by reference.
- 10.53 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, which is incorporated herein by reference.
- 10.54 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, which is incorporated herein by reference.
- 10.55 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, which is incorporated herein by reference.
- 10.56 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, which is incorporated herein by reference.
- 10.57 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, which is incorporated herein by reference.
- 10.58† 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, which is incorporated herein by reference.
- 10.59 Form of Contingent Value Rights Agreement, dated as of January 14, 2015, by and between BioMarin Pharmaceutical Inc., BioMarin Falcons B.V. and American Stock Transfer & Trust Company, LLC, previously filed with the SEC on January 16, 2015 as Exhibit 10.1 to the Company's Current Report on Form 8-K, which is incorporated by reference herein.
- 10.60†* Form of Stock Options Agreement for the BioMarin Pharmaceutical Inc. 2014 Inducement Plan.
- 10.61†* Form of Agreement Regarding Restricted Share Units for the BioMarin Pharmaceutical Inc. 2014 Inducement Plan.
- 21.1* Subsidiaries of BioMarin Pharmaceutical Inc.
- 23.1* Consent of KPMG LLP, Independent Registered Public Accounting Firm for BioMarin Pharmaceutical Inc.
- 24.1* Power of Attorney (Included in Signature Page)
- 31.1* Certification of Chief Executive Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.

- 31.2* Certification of Chief Financial Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
- 32.1* Certification of Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. This Certification accompanies this report and shall not, except to the extent required by the Sarbanes-Oxley Act of 2002, be deemed filed for purposes of §18 of the Securities Exchange Act of 1934, as amended.

101.INS XBRL Instance Document

101.SCH XBRL Taxonomy Extension Schema Document

101.CAL XBRL Taxonomy Extension Calculation Document

101.DEF XBRL Taxonomy Extension Definition Linkbase

101.LAB XBRL Taxonomy Extension Labels Linkbase Document

101.PRE XBRL Taxonomy Extension Presentation Link Document

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*Filed herewith
Management contract or compensatory plan or arrangement

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SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

BIOMARIN PHARMACEUTICAL INC.

Dated: March 2, 2015 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	Chief Executive Officer (Principal Executive Officer)	March 2, 2015
/S/ DANIEL SPIEGELMAN Daniel Spiegelman	Executive Vice President and Chief Financial Officer (Principal Financial Officer)	March 2, 2015
/S/ BRIAN R. MUELLER Brian R. Mueller	Group Vice President, Corporate Controller and Chief Accounting Officer (Principal Accounting Officer)	March 2, 2015
/S/ PIERRE LAPALME Pierre LaPalme	Chairman and Director	March 2, 2015
/S/ KENNETH BATE Kenneth Bate	Director	March 2, 2015
/S/ MICHAEL G. GREY Michael G. Grey	Director	March 2, 2015
/S/ ELAINE HERON Elaine Heron	Director	March 2, 2015
V. Bryan Lawlis	Director	March 2, 2015
Alan J. Lewis	Director	March 2, 2015
/S/ RICHARD A. MEIER Richard A. Meier	Director	March 2, 2015
/S/ DENNIS J. SLAMON	Director	

Dennis J. Slamon

March 2,
2015

/S/ WILLIAM YOUNG Director
William Young

March 2,
2015

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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, 2014 and 2013, 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, 2014. 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, 2014 and 2013, and the results of their operations and their cash flows for each of the years in the three year period ended December 31, 2014, 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, 2014, 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 March 2, 2015 expressed an unqualified opinion on the effectiveness of the Company's internal control over financial reporting.

/s/ KPMG LLP

San Francisco, California
March 2, 2015

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, 2014, 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, 2014, 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, 2014 and 2013, 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, 2014, and our report dated March 2, 2015 expressed an unqualified opinion on those consolidated financial statements.

/s/ KPMG LLP

San Francisco, California

March 2, 2015

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BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED BALANCE SHEETS

December 31, 2014 and 2013

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

	December 31, 2014	December 31, 2013
ASSETS		
Current assets:		
Cash and cash equivalents	\$875,486	\$568,781
Short-term investments	69,706	215,942
Accounts receivable, net (allowance for doubtful accounts: \$490 and \$529, at December 31, 2014 and 2013, respectively)	144,472	117,822
Inventory	199,452	162,605
Current deferred tax assets	31,203	30,561
Other current assets	105,310	41,707
Total current assets	1,425,629	1,137,418
Noncurrent assets:		
Investment in BioMarin/Genzyme LLC	1,039	816
Long-term investments	97,856	267,700
Property, plant and equipment, net	523,516	319,316
Intangible assets, net	156,578	163,147
Goodwill	54,258	54,258
Long-term deferred tax assets	166,296	145,234
Other assets	65,281	156,171
Total assets	\$2,490,453	\$2,244,060
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	\$235,739	\$183,271
Total current liabilities	235,739	183,271
Noncurrent liabilities:		
Long-term convertible debt	657,976	655,566
Long-term contingent acquisition consideration payable	38,767	30,790
Other long-term liabilities	30,077	33,392
Total liabilities	962,559	903,019
Stockholders' equity:		
Common stock, \$0.001 par value: 250,000,000 shares authorized at December 31, 2014 and 2013: 149,093,647 and 143,463,668 shares issued and outstanding at December 31, 2014 and 2013, respectively.		
	149	144

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Additional paid-in capital	2,359,744	2,059,101
Company common stock held by Nonqualified Deferred Compensation Plan	(9,695)	(7,421)
Accumulated other comprehensive income	27,466	5,018
Accumulated deficit	(849,770)	(715,801)
Total stockholders' equity	1,527,894	1,341,041
Total liabilities and stockholders' equity	\$2,490,453	\$2,244,060

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, 2014, 2013 and 2012

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

	2014	2013	2012
REVENUES:			
Net product revenues	\$738,416	\$538,360	\$496,497
Collaborative agreement revenues	1,592	3,918	1,955
Royalty, license and other revenues	11,032	6,207	2,271
Total revenues	751,040	548,485	500,723
OPERATING EXPENSES:			
Cost of sales	129,764	95,742	91,830
Research and development	461,543	354,780	302,218
Selling, general and administrative	302,156	235,356	198,173
Intangible asset amortization and contingent consideration	17,968	18,614	18,717
Gain on sale of intangible asset	(67,500)	—	—
Total operating expenses	843,931	704,492	610,938
LOSS FROM OPERATIONS	(92,891)	(156,007)	(110,215)
Equity in the loss of BioMarin/Genzyme LLC	(877)	(1,149)	(1,221)
Interest income	5,937	3,083	2,584
Interest expense	(36,642)	(10,447)	(7,639)
Debt conversion expense	(674)	(12,965)	—
Other income (expense)	279	982	(1,787)
LOSS BEFORE INCOME TAXES	(124,868)	(176,503)	(118,278)
Provision for (benefit from) income taxes	9,101	(150)	(3,931)
NET LOSS	\$(133,969)	\$(176,353)	\$(114,347)
NET LOSS PER SHARE, BASIC AND DILUTED	\$(0.92)	\$(1.28)	\$(0.95)
Weighted average common shares outstanding, basic and diluted	146,349	137,755	120,271

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

BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

Years Ended December 31, 2014, 2013 and 2012

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

	2014	2013	2012
NET LOSS	\$(133,969)	\$(176,353)	\$(114,347)
OTHER COMPREHENSIVE INCOME (LOSS):			
Net foreign currency gain (loss)	(75)	361	(301)
Available-for-sale securities:			
Unrealized holding gain arising during the period,			
net of tax impact of \$(2,931), \$(3,535) and \$(140) for the years			
ended December 31, 2014, 2013 and 2012, respectively.	5,088	6,275	388
Reclassifications to net loss, net of tax impact of \$0, \$0			
and \$40 for the years ended December 31, 2014, 2013			
and 2012, respectively.	—	(1)	(110)
Net change in unrealized holding gains, net of tax	5,088	6,274	278
Cash flow hedges:			
Unrealized holding gain (loss) arising during the period, net of tax			
impact of \$(1,214), \$789 and \$5,114 for the years ended			
December 31, 2014, 2013 and 2012, respectively.	18,078	(1,366)	(8,749)
Less reclassifications to net loss, net of tax impact of \$(365),			
\$(28) and \$2,153 for the years ended December 31, 2014,			
2013 and 2012, respectively.	643	49	(3,683)
Net change in unrealized holding gains (loss), net of tax	17,435	(1,415)	(5,066)
OTHER COMPREHENSIVE INCOME (LOSS), NET OF TAX	22,448	5,220	(5,089)
COMPREHENSIVE LOSS	\$(111,521)	\$(171,133)	\$(119,436)

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

BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY

Years Ended December 31, 2014, 2013 and 2012

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

	Common stock		Additional Paid-in Capital	Company Common Stock Held by Nonqualified Deferred Compensation Plan	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount					
Balance at December 31, 2011	114,790	\$ 115	\$ 1,197,082	\$ (3,935)	\$ 4,887	\$ (425,101)	\$ 773,048
Net loss						(114,347)	(114,347)
Other comprehensive loss					(5,089)		(5,089)
Issuance of common stock, net of offering costs	6,500	7	235,492				235,499
Issuance of common stock under the 2006 Employee Stock Purchase Plan (the ESPP)	254		5,495				5,495
Exercise of common stock options	4,097	4	77,562				77,566
Excess tax benefit from stock option exercises			473				473
Conversion of convertible notes	6		105				105
Restricted stock vested during the period, net	162		(1,659)				(1,659)
Common stock held by Nonqualified Deferred Compensation Plan				(2,668)			(2,668)
Stock-based compensation			47,340				47,340
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)

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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 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	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
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	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

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, 2014, 2013 and 2012

(In thousands of U.S. dollars)

	2014	2013	2012
CASH FLOWS FROM OPERATING ACTIVITIES:			
Net loss	\$(133,969)	\$(176,353)	\$(114,347)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	56,744	47,264	44,335
Non-cash interest expense	27,225	5,875	960
Accretion of discount on investments	7,211	5,780	4,469
Stock-based compensation	88,233	66,181	47,340
Gain on sale of intangible asset	(67,500)	—	—
Gain on termination of lease	(10,092)	—	—
Equity in the loss of BioMarin/Genzyme LLC	877	1,149	1,221
Impairment of intangible assets	—	939	6,707
Deferred income taxes	(25,483)	(9,156)	(9,921)
Excess tax benefit from stock option exercises	(1,491)	(733)	(473)
Unrealized foreign exchange gain on forward contracts	(832)	(658)	(6,529)
Non-cash changes in the fair value of contingent acquisition consideration payable	11,567	10,197	8,788
Debt conversion expense	674	12,965	—
Other	3,637	—	2,000
Changes in operating assets and liabilities:			
Accounts receivable, net	(26,650)	(8,756)	(4,227)
Inventory	(36,847)	(33,910)	1,423
Other current assets	(2,211)	(12,073)	(3,506)
Other assets	(6,435)	1,676	(4,076)
Accounts payable and accrued liabilities	38,710	20,420	37,411
Other long-term liabilities	3,093	9,559	6,034
Net cash provided by (used in) operating activities	(73,539)	(59,634)	17,609
CASH FLOWS FROM INVESTING ACTIVITIES:			
Purchases of property, plant and equipment	(118,834)	(65,124)	(44,571)
Restricted funds held in escrow for the purchase of San Rafael Corporate Center (the SRCC)	—	(116,500)	—
Maturities and sales of investments	808,313	288,643	237,837
Purchase of available-for-sale investments	(507,036)	(395,042)	(382,168)
Proceeds from sale of intangible asset	67,500	—	—
Business acquisitions, net of cash acquired	—	(9,875)	—
Investments in BioMarin/Genzyme LLC	(1,100)	(885)	(1,743)
Investment in convertible promissory note	(52,288)	—	(5,000)
Other	(2,000)	—	—
Net cash provided by (used in) investing activities	194,555	(298,783)	(195,645)

CASH FLOWS FROM FINANCING ACTIVITIES:			
Proceeds from exercises of stock options and ESPP	79,904	72,579	83,061
Taxes paid related to net share settlement of equity awards	(7,768)	(6,397)	(1,659)
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	117,464	—	235,499
Excess tax benefit from stock option exercises	1,491	733	473
Payments for debt conversion	(674)	(12,965)	—
Payment of contingent acquisition consideration payable	(4,691)	(3,061)	(4,405)
Other	(37)	(607)	(678)
Net cash provided by financing activities	185,689	746,671	312,291
NET INCREASE IN CASH AND CASH EQUIVALENTS	306,705	388,254	134,255
Cash and cash equivalents:			
Beginning of period	\$568,781	\$180,527	\$46,272
End of period	\$875,486	\$568,781	\$180,527
SUPPLEMENTAL CASH FLOW DISCLOSURES:			
Cash paid for interest, net of interest capitalized into fixed assets	9,324	2,159	6,665
Cash paid for income taxes	34,986	14,897	6,582
Stock-based compensation capitalized into inventory	8,166	6,121	4,347
Depreciation capitalized into inventory	10,952	11,016	7,335
SUPPLEMENTAL CASH FLOW DISCLOSURES FROM			
INVESTING AND FINANCING ACTIVITIES:			
Increase (decrease) in accounts payable and accrued liabilities related to fixed assets	16,766	5,001	(511)
Conversion of convertible debt	21,324	283,319	105
Deferred offering costs reclassified into additional paid-in-capital as a			
result of conversion of convertible debt	158	2,765	—

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 is comprised of five approved products and multiple clinical and pre-clinical product candidates. The Company's approved products are Vimizim (elosulfase alpha), Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (laronidase) and Firdapse (amifampridine phosphate).

Through December 31, 2014, the Company had accumulated losses of approximately \$849.8 million. 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, if approved; 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 are comprised 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. 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. 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.

Investment in BioMarin/Genzyme LLC and Equity in the Loss of BioMarin/Genzyme LLC

The Company accounts for its investment in BioMarin/Genzyme LLC (the LLC), the joint venture between the Company and Genzyme Corporation (Genzyme), using the equity method. Accordingly, the Company records an increase in its investment for contributions to BioMarin/Genzyme LLC and a reduction in its investment for its 50% share of any losses of the LLC or disbursements of profits from the LLC. Equity in the loss of the LLC includes the

Company's 50% share of the LLC's loss for the period. The investment in the LLC includes the Company's share of the net equity of the LLC.

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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)

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.

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, 2014, 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.

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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)

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 are primarily comprised 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. The Company also sells Kuvan to Merck Serono S.A. (Merck Serono) at a price near its manufacturing cost, and Merck Serono resells 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. 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 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.

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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)

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 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.

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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)

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.

Nonqualified Deferred Compensation Plan

The Company's Nonqualified Deferred Compensation Plan (the Deferred Compensation 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 Deferred Compensation 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 Deferred Compensation Plan on behalf of the participants without further action by the Board.

All of the investments held in the Deferred Compensation 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 Deferred Compensation 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 Deferred Compensation Plan. The restricted stock issued into the Deferred Compensation 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 Deferred Compensation Plan is included in Accounts Payable and Accrued Liabilities and Other Long-Term Liabilities in the Company's Consolidated Balance Sheets.

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BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

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 11 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.

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.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

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

Except for FASB Accounting Standards Update 2014-09 (ASU 2014-09), Revenue from Contracts with Customers, there have been no new accounting pronouncements or changes to accounting pronouncements during the year ended December 31, 2014, as compared to the recent accounting pronouncements described in the Company's Annual Report on Form 10-K for the year-ended December 31, 2013, that are of significance or potential significance to the Company.

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, 2016, including interim periods within that reporting period, which for the Company is January 1, 2017. 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.

(5) INVESTMENTS

All investments were classified as available-for-sale at December 31, 2014 and 2013. 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, 2014 and 2013 are summarized in the tables below:

Gross Gross

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	Amortized Cost	Unrealized Holding Gains	Unrealized Holding Losses	Aggregate Fair Value at December 31, 2014
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

	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Aggregate Fair Value at December 31, 2013
Certificates of deposit	\$ 47,008	\$ 2	\$ —	\$ 47,010
Corporate debt securities	341,519	313	(423)	341,409
Commercial paper	86,154	24	—	86,178
U.S. Government agency securities	8,900	1	—	8,901
Greek government-issued bonds	52	92	—	144
Total	\$ 483,633	\$ 432	\$ (423)	\$ 483,642

The Company has investments in marketable equity securities which are measured using quoted prices in their respective active market that are considered strategic investments. As of December 31, 2014, the fair value of the Company's strategic investments of \$30.8 million included an unrealized gain of \$18.3 million. As of December 31, 2013, the fair value of the Company's strategic investments of \$13.0 million includes an unrealized gain of \$10.1 million. These investments are recorded in Other Assets in the Company's Consolidated Balance Sheets.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

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

	December 31,	
	2014	2013
Maturing in one year or less	\$69,706	\$215,942
Maturing after one year through five years	97,856	267,700
Total	\$167,562	\$483,642

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, 2014, some of the Company's investments were in an unrealized loss position. However, the Company has the ability and intent to hold all investments that have been in a continuous loss position until maturity or recovery, thus no other-than-temporary impairment is deemed to have occurred.

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

(6) INTANGIBLE ASSETS

Intangible assets consisted of the following:

	December 31,	
	2014	2013
Intangible assets:		
Finite-lived intangible assets	\$123,365	\$118,242
Indefinite-lived intangible assets	74,430	74,430
Gross intangible assets:	197,795	192,672
Less: Accumulated amortization	(41,217)	(29,525)
Net carrying value	\$156,578	\$163,147

Finite-Lived Intangible Assets

The following table summarizes the annual amortization of the finite-lived intangible assets through 2025:

	Net Balance at December 31, 2014	Estimated Useful Life	Average Remaining Life	Annual Amortization
Repurchased royalty rights	\$ 60,188	12 years	8.9 years	\$ 6,750
License payments for marketing approvals	2,892	7 to 10 years	6.6 years	512
Acquired intellectual property	16,919	10 years	5.2 years	3,223
SRCC in-place and above market tenant leases	2,149	Remaining lease terms	Varies	Varies
Total	\$ 82,148			\$ 10,485

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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)

Indefinite-Lived Intangible Assets

Indefinite-lived intangible assets consisted of the following:

	December 31,	
	2014	2013
In-Process Research and Development:		
Talazoparib (BMN 673)	\$35,150	\$35,150
Reveglucosidase alfa (BMN 701)	25,010	25,010
SENSI-Pro technology	11,680	11,680
Other acquired pre-clinical compounds	2,590	2,590
Net carrying value	\$74,430	\$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 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.

During the fourth quarter of 2014, the Company performed its annual impairment review and determined that no impairments existed as of December 31, 2014.

During the first quarter of 2012, the Company recorded an impairment charge of \$6.7 million related to certain Firdapse IPR&D assets. These IPR&D assets were associated with marketing rights in the U.S. The Company was exploring strategic options for the Firdapse U.S. program, including the potential outlicense of rights in the U.S. In March 2012, the Company recognized an impairment charge based on the status of business development efforts at the time and the related discounted cash flow projections that no longer supported the carrying-value of the IPR&D intangible assets. The impairment charge was included in Intangible Asset Amortization and Contingent Consideration in the Company's Consolidated Statement of Operations for the year ended December 31, 2012.

(7) ACQUISITION OF 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, which was held in escrow as of December 31, 2013.

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	

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.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

The following table sets forth the 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 in-place 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 in-place lease intangible assets to expense over the remaining initial term of each lease. The Company will amortize the capitalized value of above market leases to expense over the remaining lives of the underlying leases.

The amount of third-party tenant revenue (included in the line item Royalty, License and Other Revenues) included in the Company's Consolidated Statement of Operations for the year ended December 31, 2014, was \$4.0 million. The amount of net income/loss from third-party tenants for the year ended December 31, 2014, was insignificant 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.

(8) PROPERTY, PLANT AND EQUIPMENT

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

	December 31,	
	2014	2013
Leasehold improvements	\$39,297	\$73,420
Building and improvements	335,991	159,678
Manufacturing and laboratory equipment	124,564	95,126
Computer hardware and software	97,032	74,948
Furniture and equipment	13,717	12,367
Land improvements	4,106	—
Land	29,358	11,608
Construction-in-progress	108,340	77,212
	752,405	504,359
Less: Accumulated depreciation	(228,889)	(185,043)
Total property, plant and equipment, net	\$523,516	\$319,316

The construction-in-process balance is primarily comprised of costs related to active construction projects including the construction of a laboratory on the Company's California campus and the build-out of the manufacturing facility in Shanbally, Ireland.

During the year ended December 31, 2014, the Company purchased two buildings in Novato, California which were accounted for as asset purchases. The total purchase price for the buildings was \$20.0 million.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

The following table summarizes the allocation of the total purchase price of the buildings:

	Estimated Fair Value	Estimated Useful Lives
Building and improvements	\$ 15,836	35 to 45 years
Land	3,216	
Land improvements	919	10 years
Total identifiable net assets	\$ 19,971	

Depreciation expense for the years ended December 31, 2014, 2013 and 2012 was \$44.3 million, \$36.5 million and \$34.9 million, respectively, of which \$11.0 million, \$11.0 million and \$7.3 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, 2014 was insignificant.

(9) INVENTORY

Inventory consisted of the following:

	December 31,	
	2014	2013
Raw materials	\$22,488	\$15,309
Work-in-process	114,393	88,417
Finished goods	62,571	58,879
Total inventory	\$199,452	\$162,605

(10) SUPPLEMENTAL BALANCE SHEET INFORMATION

Other current assets consisted of the following:

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	December 31,	
	2014	2013
Prepaid expenses	28,865	\$21,139
Short-term forward currency exchange contract assets	10,513	59
Promissory notes receivable, net	46,946	—
Restricted investments	2,354	5,670
Convertible promissory note conversion option	2,386	—
Other receivables	9,733	11,513
Other	4,513	3,326
Total other current assets	\$105,310	\$41,707

Other assets consisted of the following:

	December 31,	
	2014	2013
Deposits	12,021	\$7,196
Escrow balance for SRCC purchase	—	116,500
Deferred debt offering costs	11,763	15,374
Strategic investments	30,811	13,000
Long-term forward foreign currency exchange contract assets	5,387	—
Other	5,299	4,101
Total other assets	\$65,281	\$156,171

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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)

Accounts payable and accrued liabilities consisted of the following:

	December 31,	
	2014	2013
Accounts payable	\$32,779	\$36,894
Accrued accounts payable	98,490	58,408
Accrued compensation expense	45,479	33,496
Accrued vacation expense	12,540	10,487
Current portion of contingent acquisition		
consideration payable	3,895	11,882
Accrued rebates payable	14,859	10,429
Accrued royalties payable	9,050	5,829
Value added taxes payable	5,479	3,603
Other accrued operating expenses	8,244	4,875
Current portion of nonqualified deferred		
compensation liability	2,304	1,363
Other	2,620	6,005
Total accounts payable and accrued liabilities	\$235,739	\$183,271

The roll forward of significant estimated accrued rebates, reserve for cash discounts and allowance for doubtful accounts for the years ended December 31, 2014, 2013 and 2012 were as follows:

	Balance at	Provision/	Provision/	Actual Charges	Actual Charges	Balance at
	Beginning	for Current	(Reversals)	Related to	Related to	End of
	of Period	Period Sales	for Prior	Current	Prior Period	Period
			Period Sales	Period Sales	Sales	
Year ended December 31, 2014:						
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

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Year ended December 31, 2013:						
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
Year ended December 31, 2012:						
Accrued rebates	\$ 6,025	\$ 16,449	\$ (434) \$ (8,193) \$ (4,222) \$ 9,625
Reserve for cash discounts	342	4,214	—	(4,184) —	372
Allowance for doubtful accounts	513	—	(165) —	—	348

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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)

(11) DERIVATIVE INSTRUMENTS AND HEDGING STRATEGIES

Foreign Currency Exchange Rate Exposure

The Company uses forward foreign currency exchange contracts to hedge certain operational exposures resulting from potential changes in foreign currency exchange rates. Such exposures result from portions of the Company's forecasted revenues and operating expenses being denominated in currencies other than the U.S. dollar, primarily the Euro, 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. Details of the specific instruments used by the Company to hedge its exposure to foreign currency exchange rate fluctuations are discussed below. See Note 12 to these Consolidated Financial Statements for additional discussion regarding the fair value of forward foreign currency exchange contracts.

At December 31, 2014, the Company had 92 forward foreign currency exchange contracts outstanding to sell a total of 116.1 million Euros with expiration dates ranging from January 2015 through February 2017. These hedges were entered into in order to protect against the fluctuations in revenue associated with Euro-denominated sales. 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 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, 2014, the Company had 3 outstanding forward foreign currency exchange contract to sell 82.7 million Euros and one contract to sell 4.3 million British Pounds, which were not designated as a hedge for accounting purposes and matured on January 31, 2015.

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 February 2017. Over the next twelve months, the Company expects to reclassify \$10.3 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:

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	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 and accrued liabilities	\$ —
Forward foreign currency exchange contracts	Other assets	5,387	Other long- term liabilities	—
Total		\$ 15,593		\$ —
Derivatives not designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	\$ 307	Accounts payable and accrued liabilities	\$ 12
Total		307		12
Total value of derivative contracts		\$ 15,900		\$ 12

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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)

	Asset Derivatives December 31, 2013		Liability Derivatives December 31, 2013	
	Balance Sheet Location	Fair Value	Balance Sheet Location	Fair Value
Derivatives designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	\$ —	Accounts payable and accrued liabilities	\$ 2,186
Forward foreign currency exchange contracts	Other assets	—	Other long- term liabilities	—
Total		\$ —		\$ 2,186
Derivatives not designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	\$ 59	Accounts payable and accrued liabilities	\$ —
Total		59		—
Total value of derivative contracts		\$ 59		\$ 2,186

The effect of the Company's derivative instruments on the Consolidated Financial Statements for the years ended December 31, 2014, 2013 and 2012 was as follows:

	Forward Foreign Currency Exchange Contracts		
	2014	2013	2012
Derivatives Designated as Hedging Instruments:			
Net gain (loss) recognized in Other Comprehensive Income (OCI) ⁽¹⁾	\$ 18,078	\$ (1,366)	\$ (8,749)
Net gain (loss) reclassified from accumulated OCI into income ⁽²⁾	643	49	(3,683)
Net gain (loss) recognized in net loss ⁽³⁾	(294)	310	927
Derivatives Not Designated as Hedging Instruments:			
Net gain (loss) recognized in net loss ⁽⁴⁾	\$ 8,010	\$ (2,041)	\$ 674

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

(2) Effective portion classified as net product revenue.

(3) Ineffective portion and amount excluded from effectiveness testing classified as selling, general and administrative expense.

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

At December 31, 2014, 2013 and 2012, accumulated other comprehensive income before taxes associated with forward foreign currency exchange contracts qualifying for hedge accounting treatment was a gain of \$15.9 million, loss of \$2.4 million and a gain of \$0.2 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.

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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)

(12) FAIR VALUE MEASUREMENTS

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

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

	Fair Value Measurements at December 31, 2014			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	
Assets:				
Cash and cash equivalents:				
Overnight deposits	\$225,159	\$ —	\$ —	\$225,159
Money market instruments	—	650,327	—	650,327
Total cash and cash equivalents	\$225,159	\$ 650,327	\$ —	\$875,486
Available-for-sale securities:				
Short-term:				
Certificates of deposit	\$—	\$ 54,174	\$ —	\$54,174
Corporate debt securities	—	15,532	—	15,532
Long-term:				
Certificates of deposit	—	18,129	—	18,129
Corporate debt securities	—	79,604	—	79,604
Greek government-issued bonds	—	123	—	123
Total available-for-sale securities	\$—	\$ 167,562	\$ —	\$167,562
Other Current Assets:				
Nonqualified Deferred Compensation Plan assets	\$—	\$ 514	\$ —	\$514
Forward foreign currency exchange contract ⁽¹⁾	—	10,513	—	10,513
Restricted investments ⁽²⁾	—	2,354	—	2,354
Embedded derivative ⁽³⁾	—	—	2,386	2,386
Total other current assets	\$—	\$ 13,381	\$ 2,386	\$15,767
Other Assets:				

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Nonqualified Deferred Compensation Plan assets	\$—	\$ 5,112	\$ —	\$5,112
Forward foreign currency exchange contract ⁽¹⁾	—	5,387	—	5,387
Strategic investment ⁽⁴⁾	30,811	—	—	30,811
Total other assets	\$30,811	\$ 10,499	\$ —	\$41,310
Total assets	\$255,970	\$ 841,769	\$ 2,386	\$1,100,125
Liabilities:				
Current Liabilities:				
Nonqualified Deferred Compensation Plan liability	\$1,790	\$ 514	\$ —	\$2,304
Forward foreign currency exchange contract ⁽¹⁾	—	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

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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, 2013				
Quoted Price in				
	Active Markets	Significant Other Observable Inputs	Significant Unobservable Inputs	Total
	For Identical Assets (Level 1)	(Level 2)	(Level 3)	
Assets:				
Cash and cash equivalents:				
Overnight deposits	\$ 156,228	\$ —	\$ —	\$ 156,228
Money market instruments	—	412,553	—	412,553
Total cash and cash equivalents	\$ 156,228	\$ 412,553	\$ —	\$ 568,781
Available-for-sale securities:				
Short-term:				
Certificates of deposit	\$—	\$ 30,513	\$ —	\$ 30,513
Corporate debt securities	—	99,251	—	99,251
Commercial paper	—	86,178	—	86,178
Long-term:				
Certificates of deposit				