BIOMARIN PHARMACEUTICAL INC
Form 10-K
February 26, 2014
Table of Contents

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

Form 10-K

(Mark One)

x **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2013

Or

¨ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from to .

Commission file number: 000-26727

BioMarin Pharmaceutical Inc.

(Exact name of registrant as specified in its charter)

Delaware	**68-0397820**
(State of other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
770 Lindaro Street San Rafael, California	**94901**
(Address of principal executive offices)	**(Zip Code)**

Registrant s telephone number, including area code: (415) 506-6700

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 x 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 x

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 x 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 x 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	x	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 Exchange Act.) Yes ¨ No x

Indicate the number of shares outstanding of each of the issuer s classes of common stock, as of the latest practicable date: 143,623,224 shares common stock, par value $0.001, outstanding as of February 14, 2014. The aggregate market value of the voting and non-voting stock held by non-affiliates of the registrant as of June 30, 2013 was $5,076.0 million.

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 4, 2014, are incorporated by reference into Part III.

Table of Contents

BIOMARIN PHARMACEUTICAL INC.

2013 FORM 10-K ANNUAL REPORT

TABLE OF CONTENTS

Table of Contents

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, projects, continues, estimates, potential, opportunity and similar expressions. These forward-looking statements 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

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 investigational product candidates. Approved products include Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (laronidase), Firdapse (amifampridine phosphate) and VIMIZIM (elosulfase alpha).

Naglazyme received marketing approval in the United States (the U.S.) in May 2005, in the European Union (the EU) in January 2006 and subsequently in other countries. Kuvan was granted marketing approval in the U.S. and EU in December 2007 and December 2008, respectively. In December 2009, the European Medicines Agency (the EMA) granted marketing approval for Firdapse, which was launched in the EU in April 2010. 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. VIMIZIM received marketing approval in the U.S. on February 14, 2014.

We are conducting clinical trials on several investigational product candidates for the treatment of various diseases including: PEG PAL, an enzyme substitution therapy for the treatment of phenylketonuria or PKU, BMN 701, an enzyme replacement therapy for Pompe disease, a glycogen storage disorder, 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 and BMN 190, an enzyme replacement therapy for the treatment of late infantile neuronal ceroid lipofuscinosis, or CLN2, a form of Batten disease. We are conducting or planning to conduct preclinical development of several other product candidates for genetic and other metabolic diseases and recently announced the selection of two new drug development candidates, 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. BMN 250 is a novel fusion of alpha-N-acetyglucosaminidase (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).

Table of Contents

Recent Developments

VIMIZIM Marketing Approval in the U.S. and Positive CHMP Opinion in the EU

On February 14, 2014, the Food and Drug Administration (the FDA) granted marketing approval for VIMIZIM for the treatment of mucopolysaccharidosis Type IV A (Morquio Syndrome Type A or MPS IV A). We immediately began marketing VIMIZIM in the U.S. using our own existing sales force and commercial organization and completed our first commercial sale in the U.S.

On February 20, 2014, the Committee for Medical Product for Human Use (CHMP) of the EMA adopted a positive opinion for our Marketing Authorization Application (MAA) for VIMIZIM. The CHMP s recommendation has been referred to the European Commission (EC). The EC is expected to render an approval decision for VIMIZIM in the second quarter of 2014.

Factor VIII Gene Therapy Drug Development Candidate BMN 270 for the Treatment of Hemophilia A

In January 2014, we announced the selection of an AAV-factor VIII vector, BMN 270, to develop for the treatment of patients with hemophilia A, the initiation of IND-enabling toxicology studies of BMN 270 and that we expect to initiate a clinical trial in early 2015. The Company s gene therapy program for hemophilia A was originally licensed from University College London and St. Jude Children s research Hospital in February 2013 and has since been developed at BioMarin s facilities.

NAGLU Fusion Protein Drug Development Candidate BMN 250 for the Treatment of Sanfilippo B (MPS IIIB)

In February 2014, we announced the selection of a new drug development candidate, BMN 250, a novel fusion of alpha-N-acetyglucosaminidase (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 have initiated IND-enabling studies and expect to initiate clinical studies with BMN 250 in mid-2015. Discovered by BioMarin, BMN 250 is an enzyme replacement therapy using recombinant human NAGLU with an IGF2, or Glycosylation Independent Lysosomal Targeting (GILT) tag. BMRN 250 is delivered directly to the brain using our patented technology.

Contract to Purchase of San Rafael Corporate Center

On December 17, 2013, BioMarin, through a wholly-owned subsidiary entered into a Contract of Purchase and Sale and Joint Escrow Instructions (the Agreement) to purchase the office complex and vacant land commonly known as the San Rafael Corporate Center, located in the City of San Rafael, County of Marin, California (the SRCC) from SR Corporate Center Phase One, LLC, and SR Corporate Center Phase Two, LLC, each a Delaware limited liability company. We currently lease approximately 40% of the complex, which we use as our global headquarters. The purchase of the SRCC is expected to close during the first quarter of 2014 for a purchase price of $116.5 million.

Convertible Debt Offering

On October 15, 2013, we completed a convertible debt offering of $750.0 million of our senior subordinated convertible notes consisting of $375.0 million 0.75% senior subordinated convertible notes due 2018 (the 2018 Notes) and $375.0 million 1.50% senior subordinated convertible notes due 2020 (the 2020 Notes and together with the 2018 Notes, the Notes). The Notes will be convertible, under certain circumstances, into cash, shares of our common stock or a combination of cash and common stock at our election. The initial conversion rate will be 10.6213 shares of common stock per $1,000 principal amount of Notes (representing an initial conversion price of approximately $94.15

per common share), subject to customary adjustments. The initial conversion rate represents approximately a 40% premium to the last reported sale price of our common stock on the NASDAQ Global Select Market on October 8, 2013. We also entered into privately-negotiated capped call transactions with respect to 50% of the principal amount of the Notes with three of the underwriters or their affiliates. The capped

Table of Contents

call transactions are generally expected to reduce potential dilution to our common stock upon conversion of the relevant Notes in excess of the principal amount of such converted Notes. The cap price of the capped call transactions entered into with respect to 50% of the Notes will initially be, in each case, approximately $121.05, which represents a premium of approximately 80% over the NASDAQ closing price of a share of our common stock on October 8, 2013 and is subject to certain adjustments under the terms of such capped call transactions. We received net proceeds after fees, transaction costs and the purchase of the capped call of approximately $696.4 million, which we intend to use for general corporate purposes.

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, 2013, is provided below:

Commercial Products	Indication	Orphan Drug Exclusivity Expiration U.S.	Orphan Drug Exclusivity Expiration EU	2013 Total Net Product Revenues (in millions)	2013 Research & Development Expense (in millions)
Naglazyme	MPS VI (1)	Expired	September 2015	$ 271.2	$ 12.5
Kuvan	PKU (2)	December 2014	NA (12)	$ 167.4	$ 14.4
Aldurazyme (3)	MPS I (4)	Expired	Expired	$ 83.6	$ 1.7
Firdapse	LEMS (5)	NA (11)	2019	$ 16.1	$ 8.7
VIMIZIM	MPS IV A (6)	2021	NA (13)	$ 0.1	$ 82.0

Products in Development	Target Indication	Stage	2013 Research & Development Expense (in millions)
PEG PAL	PKU	Clinical Phase 3	$ 54.5
BMN 701	POMPE (7)	Clinical Phase 1/2 (8)	$ 45.6
BMN 673 (9)	BRCA BREAST CANCER	Clinical Phase 3	$ 29.5
BMN 111	ACHONDROPLASIA	Clinical Phase 2	$ 15.0
BMN 190	CLN2 (10)	Clinical Phase 1/2	$ 13.8

(1) Mucopolysaccharidosis VI, or MPS VI
(2) Phenylketonuria, or PKU
(3) 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.
(4) Mucopolysaccharidosis I, or MPS I
(5) Lambert Eaton Myasthenic Syndrome, or LEMS

(6) Mucopolysaccharidosis IV Type A, or MPS IVA
(7) Pompe disease, a glycogen storage disorder
(8) The Phase 2 clinical trial began in January 2014.
(9) BMN 673 is an orally available poly (ADP-ribose) polymerase, or PARP inhibitor for the treatment of patients with certain cancers.
(10) CLN2, or late infantile neuronal ceroid lipofuscinosis, is a lysosomal storage disorder primarily affecting the brain.
(11) Firdapse has not received marketing approval in the U.S. and we have the North American rights to develop and market Firdapse to a third party.
(12) Merck Serono markets Kuvan in the EU.
(13) We anticipate receiving marketing approval in the EU in the second quarter of 2014.

Table of Contents

Commercial Products

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 and in the EU in January 2006. We market Naglazyme in the U.S., 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, 2013, 2012 and 2011 totaled $271.2 million, $257.0 million and $224.9 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 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 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 expect to commercially launch this new form of Kuvan in the first quarter of 2014.

Kuvan was granted marketing approval for the treatment of PKU in the U.S. in December 2007. 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 seven years of market exclusivity in the U.S. for the treatment of PKU, expiring in December 2014. 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, 2013, 2012 and 2011 totaled $167.4 million, $143.1 million, and $116.8 million, respectively.

In May 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 PEG PAL for PKU. Through the agreement, as amended in 2007, Merck Serono acquired exclusive rights to market these

Table of Contents

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 PEG PAL 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 2013, 2012 and 2011 we earned $2.0 million, $1.9 million and $1.6 million, respectively, in net royalties on net sales of $51.0 million, $46.8 million and $40.4 million of Kuvan by Merck Serono, respectively. We recorded collaborative agreement revenue associated with shared Kuvan development costs in the amounts of $1.0 million, $1.8 million, and $0.5 million in 2013, 2012 and 2011, respectively.

Aldurazyme

Aldurazyme has been approved for marketing in the U.S., EU and 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, 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 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 restructuring, 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, 2013, 2012 and 2011 totaled $83.6 million, $82.2 million and $82.8 million, respectively. The net product revenues for each of the years ended December 31, 2013, 2012 and 2011 include $88.5 million, $80.4 million and $74.2 million, respectively, of royalty revenue on net Aldurazyme sales by Genzyme. Net sales of Aldurazyme by Genzyme totaled $212.4 million, $193.1 million and $185.2 million for the years ended December 31, 2013, 2012 and 2011, respectively. For the years ended December 31, 2013, 2012 and 2011 Aldurazyme net product revenue included previously recognized Aldurazyme net product transfer revenue of $4.9 million in 2013 and incremental product transfer revenue of $1.8 million, and $8.6 million, in 2012 and 2011, respectively. 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.

Table of Contents

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 (AP-HP). 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 beginning in April 2010. Firdapse net product revenues for the years ended December 31, 2013, 2012 and 2011 totaled $16.1 million, $14.2 million and $13.1 million, respectively. In October 2012, we licensed to Catalyst Pharmaceutical Partners, Inc. 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 year ended December 31, 2013 we recognized collaborative revenue of $2.9 million 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.

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 Nacetylgalactosamine- 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 1,500 patients worldwide including approximately 200 patients in the U.S. suffering from MPS IV A and if approved in the EU and other countries, we expect that VIMIZIM could be our largest commercial product to date.

VIMIZIM was granted marketing approval in the U.S. on February 14, 2014. We immediately began marketing VIMIZIM in the U.S. using our own existing sales force and commercial organization and we completed our first commercial sale in the U.S. Now that we have received approval for VIMIZIM in the U.S., we plan to pursue registration and/or market VIMIZIM on a named patient basis in other regions. Additionally, 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. The EMA has validated the MAA, for VIMIZIM and has recently moved from an accelerated assessment to a standard assessment for this MAA. On February 20, 2014, the CHMP of the EMA adopted a positive

opinion for our MAA for VIMIZIM. The EC is expected to render approval decision for VIMIZIM in the second quarter of 2014.

Table of Contents

Products in Clinical Development

PEG PAL

PEG PAL 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 PEG PAL 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 PEG PAL. 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 PEG PAL, to evaluate the immune response to PEG PAL, and to evaluate steady-state phamacokinetics in all patients and accumulation of PEG PAL 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 milligram per kilogram per week of PEG PAL 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. This study is fully enrolled and ongoing with 24 subjects. A Phase 3 clinical trial of PEG PAL was initiated in May 2013. This 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. This ongoing Phase 2 study has enrolled 24 patients to date and has demonstrated Phe reduction using the standard indication period we are using for the Phase 3 study. The FDA has indicated that lowering Phe blood levels in adults could support accelerated approval, even if neurocognitive endpoints are not demonstrated. We expect to report results from these trials in the fourth quarter of 2014.

BMN 673

BMN 673 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 BMN 673 for the treatment of patients with solid tumors. This clinical trial is an open-label study of once daily, orally administered BMN 673 in approximately 85 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 breast and ovarian cancers characterized by BRCA mutations, Ewing s sarcoma and small cell lung cancer, and has been expanded to include prostate and pancreatic cancers. In September 2013, we announced an update on the study at the 2013 San Antonio Breast Cancer Symposium. As presented, among 14 enrolled gBRCA breast cancer patients treated at the 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 rate at this dose of 86% (12/14). In the complete cohort of 18 gBRCA breast cancer patients, which included six patients from the dose escalation cohort at doses ranging from 900 µg to 1100 µg and 12 patients from the dose expansion cohort at a dose of 1.0 mg, the RECIST response rate was 44% (8/18), with one complete and seven partial responses. The clinical benefit rate was 72% (13/18), with five patients having stable disease in excess of 24 weeks. At all doses (n=18) there has been a best response of partial response or better in 12 patients, and four patients progressed prior to confirmation. Of the 14 patients treated at 1 mg, there has been a best response of partial response or better in 8 patients, and one patient progressed prior to confirmation. Safety data continues to show that BMN 673 is generally well-tolerated. The dose-limiting toxicity has been thrombocytopenia. Myelosuppression is generally mild-to-moderate in severity. Greater than grade 1 anemia, thrombocytopenia and neutropenia has occurred in 23%,

18% and 11% of patients, respectively, with chronic dosing. Fatigue, nausea and alopecia were observed in 26-31% of patients. Enrollment continues for this study.

Table of Contents

Based on the results of the Phase 2, BioMarin initiated a Phase 3 trial in gBRCA mutated breast cancer in October 2013. The Phase 3 trial is an open-label, 2:1 randomized, parallel, two-arm study of BMN 673 as compared to the physicians choice of chemotherapy in germline BRCA mutation subjects with locally advanced and/or metastatic breast cancer who have received no more than two prior chemotherapy regimens for metastatic disease. The study is enrolling approximately 429 subjects and is being conducted at approximately 100 sites in twelve countries. The primary objective of the study is to compare progression-free survival of subjects treated with BMN 673 as a monotherapy relative to those treated with protocol-specified physicians choice. The secondary objectives are to evaluate objective response rate, overall survival, safety and the pharmacokinetics of BMN 673.

BMN 701

BMN 701 is a novel fusion of acid alpha glucosidase (GAA) with a peptide derived from insulin-like growth factor 2. We acquired the BMN 701 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 BMN 701. This clinical trial was an open-label study to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamic and clinical activity of BMN 701 administered as an intravenous infusion every two weeks at doses of 20 milligrams per kilogram. 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 BMN 701 as well as determine the antibody response to BMN 701. The secondary objectives of the study are to determine the single and multi-dose pharmacokinetics of BMN 701 and determine mobility and functional exercise capacity in patients receiving BMN 701. 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 6-minute walk distance, 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 BMN 701 were generally consistent with those seen for other enzyme replacement therapies.

The FDA recently indicated that MIP is a potentially approvable primary endpoint for our anticipated Phase 3 switching trial with BMN 701. Subject to completing discussions with European health authorities, we expect to initiate a Phase 3 switching trial in the first quarter of 2014 in late-onset Pompe patients who have previously been treated with alglucosidase alfa.

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 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

Table of Contents

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. This is an international study that will enroll approximately 24 subjects for a treatment duration of six months.

BMN 190

BMN 190 is a recombinant human tripeptidyl peptidase 1 for the treatment of patients with CLN2, a form of Batten disease. In September 2013, we announced the initiation of a Phase 1/2 study for BMN 190. 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 BMN 190 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. The study is currently enrolling patients and plans to enroll approximately 22 subjects at up to ten clinical sites worldwide for a treatment duration of 48 weeks.

Manufacturing

We manufacture Naglazyme, Aldurazyme, VIMIZIM, PEG PAL, BMN 111 and BMN 190 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 the U.S. and EU.

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, substantial modifications to the facility will be required and we will need to requalify and validate certain systems in the facility. We have begun the build-out of this 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 and Naglazyme. 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, or API, for Kuvan. Firdapse, BMN 701 and BMN 673 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

Table of Contents

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 2014, the size of our sales force will be appropriate to effectively reach our target audience in markets where Naglazyme, Kuvan, Firdapse and VIMIZIM 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 Naglazyme, Kuvan and Firdapse customers include a limited number of specialty pharmacies and end-users, such as hospitals and foreign government agencies, which act as retailers. We also sell 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. During 2013, 41% of our net Naglazyme, Kuvan and Firdapse product revenues were generated by three customers. Genzyme is our sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third-parties.

Despite the significant concentration of customers, the demand for Naglazyme, Kuvan and Firdapse is driven primarily by patient therapy requirements and we are not dependent upon any individual distributor with respect to Naglazyme, Kuvan or Firdapse sales. Due to the pricing of Naglazyme, Kuvan and Firdapse and the limited number of patients, the specialty pharmacies and wholesalers generally carry a very limited inventory, resulting in sales of Naglazyme, Kuvan and Firdapse being closely tied to end-user demand. However, in certain countries particularly in Latin America, governments place large periodic orders for Naglazyme. The timing of these orders can create significant quarter to quarter variation in our revenue.

We expect VIMIZIM customers and their ordering patterns to be substantially similar to our Naglazyme customers.

Table of Contents

Competition

The biopharmaceutical industry is rapidly evolving and highly competitive. The following is a summary analysis of known competitive threats for each of our major product programs:

Naglazyme, Aldurazyme and VIMIZIM

Small companies and academic groups continue to evaluate various approaches to treating MPS VI, MPS I and MPS IVA. However, we are not aware of any active competitive program for enzyme replacement therapy for MPS VI, MPS I or MPS IV A that has entered clinical trials.

Bone marrow transplantation has been used to treat severely affected patients, generally under the age of two, with some success. Bone marrow transplantation is associated with high morbidity and mortality rates as well as with problems inherent in the procedure itself, including graft versus host disease, graft rejection and donor availability, which limits its utility and application. There are other developing technologies, including gene therapy, that are potential competitive threats to enzyme replacement therapies. However, we know of no such technology that has entered clinical trials related to MPS VI, MPS I or MPS IV A.

Kuvan and PEG PAL

There are currently no other approved drugs for the treatment of PKU. PKU is commonly treated with a medical food diet that is highly-restrictive and unpalatable. We perceive medical foods as a complement to Kuvan and PEG PAL and not a significant competitive threat. Dietary supplements of large neutral amino acids (LNAA), have also been used in the treatment of PKU. This treatment may be a competitive threat to Kuvan and PEG PAL. However, because LNAA is a dietary supplement, the FDA has not evaluated any claims of efficacy of LNAA. At least one company has filed a drug master file with the FDA for production of the active ingredient in Kuvan. However, we have no knowledge that any company has filed an abbreviated new drug application (ANDA), for Kuvan or performed the bioequivalence study that would be required for an ANDA. See the ANDA discussion under The Hatch-Waxman Act for additional information.

Firdapse and LEMS

There are no other approved drugs for the treatment of LEMS. Current options rely on intravenous immunoglobulin, plasmapherisis and/or immuno suppressant drugs. 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. Firdapse is the only approved version of 3,4 DAP. One other aminopyridine, 4AP, has been approved in the U.S. by another pharmaceutical company. However, this is for the treatment of fatigue associated with Multiple Sclerosis. The role of 4AP in LEMS is unproven and uncertain.

BMN 673

There are several other PARP inhibitors ahead of BMN 673 in clinical development for the treatment of various solid and hematologic malignancies. None of these PARP inhibitors has yet been approved by the FDA or any other regulatory agency. However, several of the competitive programs are either at approximately the same stage of development or are more advanced than BMN 673. The most advanced is AstraZeneca s product olaparib. AstraZeneca has filed an MAA with the EMA for the use of olaparib in treating ovarian cancer, and is simultaneously conducting a Phase 3 trial in ovarian cancer to support an NDA filing in the U.S.

BMN 701

There are two approved enzyme replacement therapies for Pompe disease in the U.S. and at least two more in preclinical studies. Gene therapy is also being tested in clinical trials and a pharmaceutical company initiated a

Table of Contents

Phase 2 clinical trial to test its small molecule chaperone as a combination therapy with enzyme replacement therapy.

BMN 111

There are currently no approved drugs for the treatment of achondroplasia. There are other peptides in early development for achondroplasia, although BMN 111 is the only peptide therapeutic that has entered clinical trials for achondroplasia.

BMN 190

There are currently no approved drugs for the treatment of patients with CLN2.

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 issued patents now stands at approximately 297, including approximately 64 patents issued by the U.S. Patent and Trademark Office (the USPTO). Furthermore, our portfolio of pending patent applications totals approximately 302 applications, including approximately 42 pending U.S. applications.

With respect to Naglazyme, we have 11 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. These patents will expire between 2021 and 2023 (methods of detecting).

With respect to Kuvan and BH4, 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 formulation of BH4, methods of treating PKU using a once daily dosing regimen, methods of administration of Kuvan with food, crystalline forms of BH4, and methods of producing 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

Table of Contents

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 (EPO) countries for Firdapse for the treatment of LEMS. We have no issued patents in the U.S. for Firdapse for the treatment of LEMS.

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 United States 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 EU typically involves preclinical laboratory and animal tests, the submission to the applicable regulatory agency of an application (e.g. 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.

Table of Contents

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 E.U 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 (EC), for approval. An IRB/EC may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB/EC s requirements, or may impose other conditions.

Clinical trials to support new drug applications (NDAs), or biological product licenses (BLAs), or marketing authorization applications (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 European Commission 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 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 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

Table of Contents

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 authorisation 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 European Commission will then grant marketing authorization in approximately 67 days. The European Commission follows the recommendation of the EMA in almost all cases.

During the review period, FDA and/or EMA will typically inspect one or more clinical sites and/or the sponsor to assure compliance with Good Clinical Practice regulations and will inspect the facility or the facilities at which the drug is manufactured to ensure compliance with Good Manufacturing Practice regulations. Neither the FDA nor 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

Table of Contents

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 Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the 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.

16

Table of Contents

Orphan Drug Designation

Naglazyme, Aldurazyme, Kuvan and Firdapse have received orphan drug designations from the FDA and EMA. Orphan drug designation is granted to drugs intended to treat a rare disease or condition, which in the United States 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 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.

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.

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 (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.

Table of Contents

Post-Approval Regulatory Requirements

Following approval, the FDA and 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 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 cGMPs after approval. Drug and biological product manufacturers and certain of their subcontractors are subject to periodic unannounced inspections by the FDA or 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.

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 (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 Biologics Price Competition and Innovation Act of 2009 (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

Table of Contents

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 will be 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 on August 1, 2013, with the first reports due in the second quarter of 2014. The reported data will be 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 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 EU and may require us to perform additional pre-clinical or clinical testing. The amount of time required to obtain necessary approvals may be longer or shorter than that required for FDA or EMA approval. In many countries outside of the United States, coverage, pricing and reimbursement approvals are also required.

Anti-Corruption Legislation

The U.S. Foreign Corrupt Practices Act, 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

Table of Contents

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 payor. 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 BioMarin s 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.

Table of Contents

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 payors, 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 United States our products are paid for by a variety of payors, 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. Payors 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, or 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, 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

Table of Contents

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, or 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, or DoD, 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, or FCP, which is at least 24% below the Non-Federal Average Manufacturer Price, or 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.

Table of Contents

Employees

As of January 24, 2014, we had 1,341 full-time employees, 545 of whom are in operations, 401 of whom are in research and development, 185 of whom are in sales and marketing and 210 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 2013, 2012 and 2011, 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.

Net product revenues by geography are based on patients locations for 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,		
	2013	2012	2011
Net product revenues:			
United States	$ 277,495	$ 249,745	$ 224,630
Europe	116,896	108,138	100,348
Latin America	67,338	74,390	56,950
Rest of the World	76,631	64,224	55,719
Total net product revenues	$ 538,360	$ 496,497	$ 437,647

Total revenue generated outside the U.S. was $267.3 million, $251.0 million and $217.1 million, in the years ended December 31, 2013, 2012 and 2011, respectively.

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

	December 31,		
	2013	2012	2011
Non-monetary long-lived assets:			

United States	$ 621,172	$ 612,974	$ 615,052
International	82,130	80,067	80,459
Total long-lived assets	$ 703,302	$ 693,054	$ 695,511

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. The decrease in non-monetary long-lived assets in 2012 compared to 2011 was primarily attributed to amortization of intangible assets and deprecation of property, plant and equipment, offset by capital expenditures.

Table of Contents

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 Lindaro Street, San Rafael, California 94901 and our telephone number is (415) 506-6700. Our annual reports on Form 10-K, quarterly reports on Form 10-Q, proxy statements, current reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the 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.

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., 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 but has not been approved in the EU or any other jurisdiction and may never receive additional regulatory approvals for any jurisdiction outside of the U.S.

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

Table of Contents

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, and record keeping. If we do not comply with the applicable regulations, the range of possible sanctions includes issuance of adverse publicity, product recalls or seizures, fines, total or partial suspensions of production and/or distribution, suspension of marketing applications, and 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.

Table of Contents

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 pharmaceutical products, we may not be the first to obtain marketing approval for any particular orphan indication. 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, 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 FDC Act, and the Public Health Service Act (the PHS Act). Biologics require the submission of a Biologics License Application (BLA), and approval by the FDA prior to being marketed in the U.S. Historically, a biologic product approved under a BLA was not subject to the generic drug review and approval provisions of the FDC Act. However, the 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 abbreviated BLAs.

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.

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;

Table of Contents

adverse medical events or side effects in treated patients;

lack of effectiveness of the product candidate being tested; and

regulatory requests for additional clinical trials or pre-clinical studies.

Typically, if a drug product is intended to treat a chronic disease, as is the case with some of our product candidates, safety and efficacy data must be gathered over an extended period of time, which can range from nine months to three years or more. We also rely on independent third-party CROs to perform most of our clinical studies and many important aspects of the services performed for us by the CROs are out of our direct control. If we fail to adequately manage our CROs, or if there is any dispute or disruption in our relationship with our CROs, our clinical trials may be delayed. Moreover, in our regulatory submissions, we rely on the quality and validity of the clinical work performed by third-party CROs. If any of our CROs processes, methodologies or results were determined to be 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 very substantial research and development and operated at a net loss until 2008. Although we were profitable in 2008 and 2010, we operated at a net loss in 2009, 2011 and 2012. 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 Naglazyme, Kuvan, Firdapse and VIMIZIM, 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, we or our contract manufacturers, must obtain regulatory approval of our manufacturing facilities, processes and quality systems. 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 Naglazyme, Kuvan, Firdapse, Aldurazyme and VIMIZIM have also been inspected and approved by various regulatory authorities. The manufacturing facility located in Shanbally, Cork, Ireland that we purchased in 2011 has not yet been approved by the FDA or the EMA. 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, 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 Naglazyme, Kuvan, Aldurazyme, Firdapse and VIMIZIM or our product candidates may be unable to comply with GMP regulations in a cost effective manner and may be unable to initially or continue to pass a federal or international regulatory inspection.

Table of Contents

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 products 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, 2013, we had cash, cash equivalents and short and long-term investments totaling $1,052.4 million and long-term debt obligations of $655.6 million. In October 2013, we completed an offering of senior subordinated convertible notes and received net proceeds of approximately $696.4 million, after deducting commissions and estimated offering expenses payable by us. We will need cash to not only repay the principal amount of the Notes but also the ongoing interest due on the Notes during their term. In addition, 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 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 Naglazyme, Kuvan, Firdapse and VIMIZIM;
- 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;

Table of Contents

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

Table of Contents

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 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 finished products, 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 delay regulatory approval for our product candidates.

Table of Contents

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, if approved, 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

Table of Contents

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.

32

Table of Contents

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 new 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 up to 2% per fiscal year, which went into effect on April 1, 2013. 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 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 payors. 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

Table of Contents

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 also have 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 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. The CMS has issued a final rule that requires manufacturers to begin collecting required information on August 1, 2013 with the first reports due March 31, 2014 (and by the 90th day of each calendar year thereafter) and publication of the reported data in a searchable form on a public website beginning September 30, 2014.

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.

34

Table of Contents

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 United States. 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.

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 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.

Table of Contents

We own or have licensed patents and patent applications related to Naglazyme, Kuvan, Aldurazyme, Firdapse and VIMIZIM. 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 recently enacted America Invents Act, 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 USPO 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 or 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 product 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 properties, such as BMN 673, BMN 701, 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 their 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.

Table of Contents

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 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 BioMarin/Genzyme LLC (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.

Table of Contents

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 PEG PAL 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 PEG PAL 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 PEG PAL. However, Merck Serono has opted out of the PEG PAL 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 PEG PAL development program, we do not have any right to commercialize PEG PAL 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 PEG PAL development program before the unblinding of the first Phase 3 trial for PEG PAL, 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 PEG PAL, 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 PEG PAL 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 PEG PAL 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 BMN 701 and BMN 673 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

Table of Contents

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 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 for the same uses but does not generally require the conduct and submission of clinical efficacy studies for that 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 based on pharmacokinetic studies. Pursuant to the Hatch-Waxman Act, companies were able to file an ANDA application for the active ingredient in Kuvan at any time after December 2011. At present, we have not received information that any other party has filed or has conducted the bioequivalency study necessary to file an ANDA for Kuvan.

The Hatch Waxman Act requires an applicant for a drug that relies, at least in part, on our data regarding the safety and efficacy of Kuvan, to notify us of their application and potential infringement of our patents listed in the Orange Book. Upon receipt of a notice alleging that our patents listed in the Orange Book are invalid or not infringed by the proposed competitor product (a paragraph iv notice), we would have 45 days to bring a patent infringement suit in federal district court against the company seeking approval for its product. The discovery, trial and appeals process in such suits can take several years. If we commence such a suit alleging infringement of one or more of our Orange Book listed patents within 45 days from receipt of the paragraph iv notice, the Hatch-Waxman Act provides a 30-month stay on the FDA s approval of the competitor s application. If the litigation is resolved in favor of the applicant or the challenged patent expires during the 30-month stay period, the stay is lifted and the FDA s review of the application may be completed. Such litigation is often time-consuming, costly and may result in competition if such patent(s) are not upheld or if the competitor does not infringe such patent(s). However, generic versions of Kuvan would be prohibited until the expiration of orphan drug exclusivity in December 2014 or June 2015 if we receive pediatric exclusivity.

The filing of an ANDA application in respect to Kuvan could have an adverse impact on our stock price and litigation to enforce our patents is likely to cost a substantial amount and require significant management attention. If the patents covering Kuvan were not upheld in litigation or if the generic competitor is found to not infringe these patents, the resulting generic competition following the expiration of orphan 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

Table of Contents

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 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.

40

Table of Contents

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 Naglazyme, Kuvan, Aldurazyme, Firdapse and VIMIZIM, or our clinical trials for PEG PAL, BMN 701, BMN 673, BMN 111, BMN 190 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 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.

Table of Contents

For the year ended December 31, 2013 approximately 4% of our net product revenues were from the Southern European countries of Italy, Spain, Portugal and Greece. Approximately 16% of our total accounts receivable as of December 31, 2013 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.

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 Naglazyme, Aldurazyme, Kuvan, Firdapse and VIMIZIM;
- manufacture, supply or distribution of Naglazyme, Aldurazyme, Kuvan, Firdapse and VIMIZIM;
- 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;
- 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., 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.

Table of Contents

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.

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.

43

Table of Contents

Item 2. Properties

The following table contains information about our current significant owned and leased properties:

Location	Approximate Square Feet	Use	Lease Expiration Date
Several locations in Novato, California	273,000	Office, laboratory and warehouse	2016-2020
San Rafael facility, San Rafael, California	120,400	Corporate headquarters, office	NA: in escrow
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
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

We have no material legal proceedings pending.

Item 4. Mine Safety Disclosures

Not applicable

Table of Contents

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 closing sales prices for our common stock for the periods noted, as reported by NASDAQ.

		Prices	
Year	**Period**	**High**	**Low**
2012	First Quarter	$ 38.34	$ 33.68
2012	Second Quarter	$ 39.58	$ 32.13
2012	Third Quarter	$ 43.30	$ 37.02
2012	Fourth Quarter	$ 50.17	$ 36.78
2013	First Quarter	$ 62.39	$ 51.56
2013	Second Quarter	$ 70.30	$ 54.72
2013	Third Quarter	$ 78.39	$ 58.64
2013	Fourth Quarter	$ 75.92	$ 59.30

On February 14, 2014, the last reported sale price on the NASDAQ Global Select Market for our common stock was $75.81. We have never paid any cash dividends on our common stock and we do not anticipate paying cash dividends in the foreseeable future.

Issuer Purchases of Equity Securities

We did not make any purchases of our common stock during the year ended December 31, 2013.

Holders

As of February 14, 2014, there were 53 holders of record of 143,623,224 outstanding shares of our common stock. Additionally, on such date, options to acquire 13.0 million shares of our common stock were outstanding.

Table of Contents

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, 2008 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, 2008 in stock or index, including reinvestment of dividends.

	Fiscal Year Ending December 31,					
	2008	**2009**	**2010**	**2011**	**2012**	**2013**
BioMarin Pharmaceutical Inc.	100.00	105.67	151.29	193.15	276.40	395.22
NASDAQ Composite Index	100.00	144.88	170.58	171.30	199.99	283.39
NASDAQ Biotechnology Index	100.00	104.67	112.89	127.04	169.50	288.38

Table of Contents

Item 6. Selected Consolidated Financial Data

The information set forth below for the five years ended December 31, 2013 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)				
	2013	2012	2011	2010	2009
Consolidated statements of operations data:					
REVENUES:					
Net product revenues	$ 538,360	$ 496,497	$ 437,647	$ 369,701	$ 315,721
Collaborative agreement revenues	3,918	1,955	468	682	2,379
Royalty and license revenues	6,207	2,271	3,243	5,884	6,556
Total revenues	548,485	500,723	441,358	376,267	324,656
OPERATING EXPENSES:					
Cost of sales (excludes amortization of certain acquired intangible assets)	95,742	91,830	84,023	70,285	65,909
Research and development	354,780	302,218	214,374	147,309	115,116
Selling, general and administrative	235,356	198,173	175,423	151,723	124,290
Intangible asset amortization and contingent consideration	18,614	18,717	1,428	6,406	2,914
Total operating expenses	704,492	610,938	475,248	375,723	308,229
INCOME (LOSS) FROM OPERATIONS	(156,007)	(110,215)	(33,890)	544	16,427
Equity in the loss of BioMarin/Genzyme LLC	(1,149)	(1,221)	(2,426)	(2,991)	(2,594)
Interest income	3,083	2,584	2,934	4,112	5,086
Interest expense	(10,447)	(7,639)	(8,409)	(10,818)	(14,404)
Debt conversion expense	(12,965)	0	(1,896)	(13,728)	0
Impairment loss on equity investments	0	0	0	0	(5,848)
Net gain from sale of investments	0	0	0	902	1,585
Other income (expense)	982	(1,787)	60	489	314
INCOME (LOSS) BEFORE INCOME TAXES	(176,503)	(118,278)	(43,627)	(21,490)	566
Provision for (benefit from) income taxes	(150)	(3,931)	10,209	(227,309)	1,054
NET INCOME (LOSS)	$ (176,353)	$ (114,347)	$ (53,836)	$ 205,819	$ (488)
NET INCOME (LOSS) PER SHARE, BASIC	$ (1.28)	$ (0.95)	$ (0.48)	$ 2.00	$ (0.00)

NET INCOME (LOSS) PER SHARE, DILUTED	$ (1.28)	$ (0.95)	$ (0.48)	$ 1.73	$ (0.00)
Weighted average common shares outstanding, basic	137,755	120,271	112,122	103,093	100,271
Weighted average common shares outstanding, diluted	137,755	120,271	112,122	125,674	100,271

Table of Contents

	December 31, (in thousands)				
	2013	2012	2011	2010	2009
Consolidated balance sheet data:					
Cash, cash equivalents and investments	$ 1,052,423	$ 563,798	$ 289,477	$ 402,283	$ 470,526
Total current assets	1,137,418	743,431	469,802	504,260	467,727
Total assets	2,249,217	1,568,347	1,270,582	1,226,106	917,163
Long-term convertible senior notes	655,566	324,859	348,629	377,521	497,083
Total stockholders equity	1,341,041	1,015,763	773,048	717,257	322,185

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)			
	March 31,	June 30,	September 30,	December 31,
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)
2012:				
Total revenue	$ 116,649	$ 124,019	$ 128,117	$ 131,938
Net loss	(23,972)	(32,006)	(5,357)	(53,012)
Net loss per share, basic and diluted	(0.21)	(0.27)	(0.04)	(0.43)

Table of Contents

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

The following discussion of our financial condition and results of operations should be read in conjunction with our Consolidated Financial Statements and notes to those statements included elsewhere in this Annual Report on Form 10-K.

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.

Key components of our results of operations for the years ended December 31, 2013, 2012 and 2011 include the following (in millions):

	Years Ended December 31,		
	2013	2012	2011
Total net product revenues	$ 538.4	$ 469.5	$ 437.6
Cost of sales	95.7	91.8	84.0
Research and development expense	354.8	302.2	214.4
Selling, general and administrative expense	235.4	198.2	175.4
Intangible asset amortization and contingent consideration	18.6	18.7	1.4
Net loss	(176.4)	(114.3)	(53.8)
Stock-based compensation expense	64.4	48.0	43.8

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

Our product portfolio is comprised of five approved products and multiple investigational product candidates. Our approved products are Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Firdapse (amifampridine phosphate) Aldurazyme (laronidase) and VIMIZIM (elosulfase alpha).

Naglazyme, a recombinant form of N-acetylgalactosamine 4-sulfatase indicated for patients with mucopolysaccharidosis VI (MPS VI), a debilitating life-threatening genetic disease for which no other drug treatment currently exists and which is caused by the deficiency of arylsufatase B, received marketing approval in the U.S. in May 2005, in the EU in January 2006 and subsequently in other countries. Naglazyme net product revenues for the year ended December 31, 2013 totaled $271.2 million, compared to $257.0 million and $224.9 million for the years ended December 31, 2012 and 2011, respectively.

Kuvan was granted marketing approval for the treatment of phenylketonuria (PKU) in the U.S. in December 2007 and in the EU in December 2008. Kuvan net product revenues for the year ended December 31, 2013 totaled $167.4 million, compared to $143.1 million and $116.8 million for the years ended December 31, 2012 and 2011, respectively.

In December 2009, the European Medicines Agency granted marketing approval for Firdapse, a proprietary form of 3-4-diaminopyridine (amifampridine phosphate), for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS). We launched this product on a country-by-country basis in the EU beginning in April 2010. Firdapse net product

revenues for the year ended December 31, 2013 totaled $16.1 million, compared to $14.2 million and $13.1 million for the years ended December 31, 2012 and 2011, respectively.

Aldurazyme (laronidase), 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 for patients with

Table of Contents

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

mucopolysaccharidosis I (MPS I). Aldurazyme net product revenues for the year ended December 31, 2013 totaled $83.6 million, compared to $82.2 million and $82.8 million for the years ended December 31, 2012 and 2011, respectively.

In February 2014, the Food and Drug Administration (FDA) granted marketing approval for VIMIZIM for the treatment mucopolysaccharidosis Type IV or Morquio Syndrome Type A, a lysosomal storage disorder. We immediately began marketing VIMIZIM in the U.S. using our existing sales force and commercial organization and completed our first commercial sale in the U.S.

We are conducting clinical trials on several investigational product candidates for the treatment of various diseases including:

- PEG PAL, an enzyme substitution therapy for the treatment of PKU;
- BMN 701, an enzyme replacement therapy for Pompe disease, a glycogen storage disorder;
- BMN 673, an orally available poly-ADP ribose polymerase inhibitor for the treatment of patients with certain cancers;
- BMN 111, a peptide therapeutic for the treatment of achondroplasia, the leading cause of dwarfism: and
- BMN 190 for the treatment of late infantile neuronal ceroid lipofuscinosis(CLN2), lysomal 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 and recently announced the selection of two new drug development candidates, 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. BMN 250 is a novel fusion of alpha-N-acetyglucosaminidase (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).

Cost of sales includes raw materials, personnel and facility and other costs associated with manufacturing Naglazyme, VIMIZIM 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.

Research and development 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 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.

Intangible asset amortization and contingent consideration includes amortization expense related to our finite-lived intangible assets associated with marketing rights in the EU for Firdapse, impairment losses (if any) on intangible assets and changes in the fair value of contingent acquisition consideration payable. Changes in fair value can result from changes in estimated probability adjustments, changes in estimated timing of when a milestone may be achieved, changes in assumed discount periods and rates and passage of time.

Table of Contents

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

Our cash, cash equivalents, short-term investments and long-term investments totaled $1,052.4 million as of December 31, 2013, compared to $563.8 million as of December 31, 2012. We have historically financed our operations primarily through our cash flows from operating activities, the issuance of common stock and convertible debt and by relying on equipment and other commercial financing. 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 senior subordinated convertible note 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 (SEC), we make assumptions, judgments and estimates that can have a significant impact on our net 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 in-process research and development 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.

Table of Contents

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 net operating loss carryforwards and 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, long-term investments 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 which have not reached technological feasibility, are reviewed annually for impairment and whenever events or changes in circumstances indicate that the carrying amount may not be recoverable. We determine impairment by comparing the fair value of the asset to its carrying value. If the asset s carrying value exceeds its fair value, an impairment charge is recorded for the difference and its carrying value is reduced accordingly.

Estimating future cash flows of an IPR&D product candidate for purposes of an impairment analysis requires us to make significant estimates and assumptions regarding the amount and timing of costs to complete the project and the amount, timing and probability of achieving revenues from the completed product similar to how the acquisition date fair value of the project was determined, as described above. There are often major risks and uncertainties associated with IPR&D projects as we are required to obtain regulatory approvals in order to be

able to market these products. Such approvals require completing clinical trials that demonstrate a product candidate is safe and effective. Consequently, the eventual realized value of the acquired IPR&D project may vary from its estimated fair value at the date of acquisition, and IPR&D impairment charges may occur in future periods which could have a material adverse effect on our results of operations.

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

When reviewing goodwill for impairment, we assess whether goodwill should be allocated to operating levels lower than our single operating segment for which discrete financial information is available and reviewed for decision-making purposes. These lower levels are referred to as reporting units. Currently, we have identified only one reporting unit as per Financial Accounting Standards Board (FASB) Accounting Standards Codification (ASC) Topic 350-20, *Intangibles Goodwill and Other*. We perform our annual impairment review of goodwill during the fourth quarter and whenever events or circumstances indicate that the carrying amount of an asset may not be recoverable. Our impairment review was based on a qualitative assessment including expected future revenues and cash flows, 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, 2013.

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 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 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, 2013 and 2012, accounts receivable included $26.3 million and $32.4 million, respectively, of unbilled accounts receivable related to net incremental Aldurazyme product transfers to Genzyme.

Table of Contents

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

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

We record allowances for product returns, if appropriate, as a reduction of revenue at the time product sales are recorded. Several factors are considered in determining whether an allowance for product returns is required, including market exclusivity of the products based on their orphan drug status, the patient population, the customers limited return rights and our experience with returns. Because of the pricing of our products, the limited number of patients and customers limited return rights, most customers and retailers carry a limited inventory.

Certain international customers, usually government entities, tend to purchase larger quantities of product less frequently. Although such buying patterns may result in revenue fluctuations from quarter to quarter, we have not experienced an increase in product returns and do not believe these buying patterns increase the risk of product returns. We rely on historical return rates to estimate returns for our commercial products. Genzyme s contractual return rights for Aldurazyme are limited to defective product. Based on these factors and the fact that we have not experienced significant product returns to date, management has concluded that product returns will be minimal. In the future, if any of these factors and/or the history of product returns changes, an allowance for product returns may be required.

Bad debt reserves are based on estimated uncollectible accounts receivable. Given our historical experience with bad debts, combined with our credit management policies and practices, we do not presently maintain significant bad debt reserves. However some of our customers are based in countries where the economic conditions continue to present challenges. We continue to monitor these conditions and associated impacts on the financial performance and credit worthiness of our large customers so that we can properly assess and respond to changes in customer credit profiles. As of December 31, 2013, our allowance for doubtful accounts was $0.5 million, compared to $0.3 million as of December 31, 2012.

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 Naglazyme, Kuvan and Firdapse to derive net sales are described in the table below.

Revenue Dilution Item	Percentage of Gross Sales Years Ended December 31,		Description
	2013	2012	
Rebates	1.0-4.3%	0.9-5.0%	Rebates payable to state Medicaid, other government programs and certain managed care providers
Distributor Fees	0.2-3.6%	0.3-3.8%	Fees paid to authorized distributors
Cash Discounts	0.7-1.9%	0.5-1.9%	Discounts offered to customers for prompt payment of accounts receivable

Total	1.9-9.8%	1.7-10.7%

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.

Table of Contents

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

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 research and development collaboration are initially deferred and recognized as collaborative agreement license revenue over the estimated period for we continue to have a performance obligation.

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

It can only be achieved based in whole or in part on either our performance or on the occurrence of a specific outcome resulting from our performance;

There is substantive uncertainty at the date an arrangement is entered into that the event will be achieved; and

It would result in additional payments being due to us.

Royalty and license revenues Royalty and license 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. 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 other royalty 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.

Table of Contents

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

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 Phase 3 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.

Results of Operations

Net Loss

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 change in net loss was primarily a result of the following (in millions):

Net loss for the year ended December 31, 2012	$ (114.3)
Increased research and development expense	(52.6)
Increased selling, general and administrative 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 research and development expense was primarily attributed to increased development expenses for our BMN 701, BMN 673 and PEG PAL 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.

Table of Contents

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

Our net loss for the year December 31, 2012 was $114.3 million, compared to net loss of $53.8 million for the year ended December 31, 2011. The increase in net loss was primarily a result of the following (in millions):

Net loss for the year ended December 31, 2011	$ (53.8)
Increased research and development expense	(87.8)
Increased selling, general and administrative expense	(22.7)
Increased intangible asset amortization and contingent consideration expense	(10.6)
Impairment loss on intangible assets	(6.7)
Loss on conversion of promissory note	(2.0)
Increased gross profit from product sales	51.0
Decreased income tax expense	14.1
Absence of debt conversion expense	1.9
Other individually insignificant fluctuations	2.3
Net loss for the year ended December 31, 2012	$ (114.3)

The increase in gross profit from product sales during the year ended December 31, 2012 as compared to the year ended December 31, 2011 was primarily a result of additional Naglazyme patients initiating therapy and additional Kuvan patients initiating therapy in the U.S. The increase in research and development expense was primarily attributed to increased development expenses for our VIMIZIM, BMN 701, BMN 673 and PEG PAL programs. The increase in selling, general and administrative expense was primarily due to increased facility and employee related costs and the continued international expansion of Naglazyme.

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

Net Product Revenues, Cost of Sales and Gross Profit

Net product revenues were as follows (in millions):

	Years Ended December 31,				
	2013	2012	2011	2013 v. 2012	2012 v. 2011
Naglazyme	$ 271.2	$ 257.0	$ 224.9	$ 14.2	$ 32.1
Kuvan	167.4	143.1	116.8	24.3	26.3
Firdapse	16.1	14.2	13.1	1.9	1.1
Aldurazyme	83.6	82.2	82.8	1.4	(0.6)
VIMIZIM	0.1	0	0	0.1	0
Total net product revenues	$ 538.4	$ 496.5	$ 437.6	$ 41.9	$ 58.9

Gross profit by product was as follows (in millions):

	Years Ended December 31,				
	2013	2012	2011	2013 v. 2012	2012 v. 2011
Naglazyme	$ 232.4	$ 218.5	$ 186.9	$ 13.9	$ 31.6
Kuvan	140.9	118.9	98.1	22.0	20.8
Firdapse	12.4	11.4	10.8	1.0	0.6
Aldurazyme	56.9	55.8	57.8	1.1	(2.0)
VIMIZIM	0.1	0	0	0.1	0
Total gross profit	$ 442.7	$ 404.6	$ 353.6	$ 38.1	$ 51.0

Table of Contents

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

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

	Years Ended December 31,				
	2013	2012	2011	2013 v. 2012	2012 v. 2011
Aldurazyme revenue reported by Genzyme	$ 212.4	$ 193.1	$ 185.2	$ 19.3	$7.9

	Years Ended December 31,				
	2013	2012	2011	2013 v. 2012	2012 v. 2011
Royalties earned from Genzyme	$ 88.5	$ 80.4	$ 74.2	$ 8.1	$ 6.2
Incremental (previously recognized) Aldurazyme product transfer revenue	(4.9)	1.8	8.6	(6.7)	(6.8)
Total Aldurazyme net product revenues	$ 83.6	$ 82.2	$ 82.8	$ 1.4	$ (0.6)

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.

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.

Table of Contents

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

2012 compared to 2011

Net product revenues for Naglazyme for the year ended December 31, 2012 totaled $257.0 million, of which $222.8 million was earned from customers based outside the U.S., compared to $224.9 million for the year ended December 31, 2011, of which $194.2 million was earned from costumers based outside the U.S. The impact of foreign currency exchange rates on Naglazyme sales denominated in currencies other than the U.S. dollar was negative by $0.9 million for the year ended December 31, 2012. Naglazyme gross margins in 2012 were 85%, compared to 2011 when Naglazyme gross margins were 83%.The increased Naglazyme gross margins in 2012 were consistent with expectations and primarily a result of our purchase of the Naglazyme royalty rights from SA Pathology in November 2011 and the price increase in the U.S. and Latin America that occurred in March 2012. Prior to the purchase of the royalty rights, we licensed the intellectual property from SA Pathology to whom we paid a 5% royalty on net sales of Naglazyme. See Note 10 to our accompanying Consolidated Financial Statements for additional discussion of the transaction.

Net product revenue for Kuvan for the year ended December 31, 2012 was $143.1 million, compared to $116.8 million for the year ended December 31, 2011. Kuvan gross margins for 2012 were 83%, compared to 2011 when gross margins were 84%. Cost of goods sold for the years ended December 31, 2012 and 2011 reflect royalties paid to third-parties of 10%. Kuvan gross margins in 2012 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 during 2012 were $1.9 million, compared to $1.6 million during 2011.

Net product revenue for Firdapse during the year ended December 31, 2012 was $14.2 million, compared to $13.1 million during the year ended December 31, 2011. Firdapse gross margins during 2012 were 80%, compared to the 82% during 2011. Cost of goods sold for the periods presented reflect royalties paid to third-parties of approximately 8%. Firdapse gross margins for the year ended December 31, 2012 decreased compared to the year ended December 31, 2011 due to increased manufacturing costs and the depletion of inventory manufactured prior to approval. Firdapse gross margins during 2012 were consistent with expectations and are not expected to fluctuate significantly in the future.

During the year ended December 31, 2012, Aldurazyme gross margins were 68%, compared to 70% during the year ended December 31, 2011. Aldurazyme gross margins reflect the profit earned on royalty revenue and net incremental product transfer revenue. The change in margins is attributed to the shift in revenue mix between royalty revenue and net product transfer revenues. 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, 2012 was $91.8 million, compared to $84.0 million for the year ended December 31, 2011. The increase in cost of sales was primarily attributed to the increase in product sales and the amortization of the cost of the Naglazyme royalty rights purchased in the fourth quarter of 2011 and the shift in Aldurazyme revenue mix between royalty revenue and net product revenues.

Research and Development

We manage our research and development 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.

Table of Contents

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

Research and development 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 research and development expense was primarily a result of the following (in millions):

Research and development expense for the year ended December 31, 2012	$ 302.2
Increased PEG PAL development expenses	27.8
Increased BMN 673 development expenses	18.1
Increased BMN 701 development expenses	14.0
Increased development expenses on early development stage programs	13.2
Increased stock-based compensation expenses related to research and development	7.0
Increased development expenses related to commercial products	4.1
Increased BMN 111 development expenses	2.9
Increased BMN 190 development expenses	2.7
Decreased VIMIZIM development expenses	(15.0)
Decrease in non-allocated research and development expenses and other net changes	(22.2)
Research and development expense for the year ended December 31, 2013	$ 354.8

The increase in PEG PAL, BMN 673 and BMN 701 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 Pharmaceuticals, Inc. (Zacharon). The increase in stock-based compensation is 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 BMN 190 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 research and development expense as significant uncertainty existed over the recoverability of the costs. The decrease in non-allocated research and development expense is primarily attributed to a decline in research and development personnel costs and facility costs that are not allocated to specific programs.

During 2014, we expect research and development spending to increase over 2013 levels due to our PEG PAL, BMN 673, BMN 701, BMN 111 and BMN 190 programs progressing, including a few of those programs progressing to more advanced phases of clinical studies. Phase 3 clinical trials for PEG PAL and BMN 673 were initiated in the second and fourth quarters of 2013, respectively, and we expect to initiate a Phase 3 trial of BMN 701 in the first quarter of 2014. We also expect increased spending on pre-clinical and clinical activities for our early development stage programs including BMN 270 and programs acquired from Zacharon. Additionally, we expect to continue incurring significant research and development 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 regulatory

approval and recoverability are highly likely and therefore future revenues are expected, the costs 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 research and development expenses.

Table of Contents

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

Research and development expense increased to $302.2 million for the year ended December 31, 2012, from $214.4 million for the year ended December 31, 2011. The increase in research and development expense was primarily a result of the following (in millions):

Research and development expense for the year ended December 31, 2011	$ 214.4
Increased VIMIZIM development expenses	42.5
Increased BMN 701 development expenses	14.1
Increased BMN 190 development expenses	9.9
Increased BMN 673 development expenses	4.0
Increased stock-based compensation expense related to research and development	4.4
Decreased development expense related to commercial products	(1.6)
Decreased BMN 111 development expenses	(1.5)
Decreased PEG PAL development expenses	(1.0)
Decreased development expenses on early development stage programs	(0.7)
Increase non-allocated research and development expenses and other net changes	17.7
Research and development expense for the year ended December 31, 2012	$ 302.2

The increase in VIMIZIM development expenses in 2012 was attributed to increased clinical trial and manufacturing activities related to the product candidate. The increases in BMN 673 and BMN 701 development expenses were in 2012 attributed to increased clinical trial activities related to these product candidates. The increase in BMN 190 development expenses was attributed to increased pre-clinical activities related to this product candidate. The decrease in PEG PAL development expenses was attributed to the timing of purchases of materials to produce the drug substance for the clinical trial. The decrease in BMN 111 development expenses was attributed to a decrease in pre-clinical activities related to this product candidate. The increase in stock-based compensation expense is a result of an increased number of options outstanding due to an increased number of employees. The increase in non-allocated research and development expenses primarily includes increased research and development personnel and facility costs that are not allocated to specific programs.

Selling, General and Administrative

Selling, general and administrative 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 selling, general and administrative expenses was primarily a result of the following (in millions):

Selling, general and administrative expense for the year ended December 31, 2012	$ 198.2
Increased sales and marketing expenses related to commercial products	10.7
Increased VIMIZIM pre-commercial expenses	15.4
Increased stock-based compensation	9.5
Increased foreign exchange losses on unhedged transactions	1.3

Net increase in corporate support and other administrative expenses	0.3
Selling, general and administrative expense for the year ended December 31, 2013	$ 235.4

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 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 2013 and the recognition of approximately $4.9 million of expense related to performance awards granted to certain executive officers. We expect selling, general and administrative expenses to increase in future periods as a result of the international expansion of Naglazyme, the U.S. commercialization activities for Kuvan, pre-commercial activities for VIMIZIM and the administrative support of our expanding operations.

Table of Contents

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

Selling, general and administrative expense increased to $198.2 million for the year ended December 31, 2012, from $175.4 million for the year ended December 31, 2011. The increase in selling, general and administrative expenses was primarily a result of the following (in millions):

Selling, general and administrative expense for the year ended December 31, 2011	$ 175.4
Net increase in corporate support and other administrative expenses	16.0
Increased sales and marketing expenses related to commercial products	6.2
Increased VIMIZIM pre-commercial expenses	2.9
Decreased foreign exchange losses on unhedged transactions	(2.3)
Selling, general and administrative expense for the year ended December 31, 2012	$ 198.2

The increase in corporate support and other administrative costs was primarily comprised of increased employee-related costs and facility costs. The increase in employee-related costs was primarily attributed to the increase in headcount. The increase in facility costs was primarily driven by the occupation of our new corporate headquarters in San Rafael, California. 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.

Intangible Asset Amortization and Contingent Consideration

Intangible asset amortization and contingent consideration expense is comprised of amortization of the European marketing rights for Firdapse, changes in the fair value of contingent acquisition consideration payable to former stockholders of our acquired businesses and impairment loss (if any) on 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				
	2013	2012	2011	2013 v. 2012	2012 v.02011
Changes in the fair value of contingent acquisition consideration payable	14.5	8.8	(1.8)	5.7	10.6
Amortization of Firdapse European marketing rights	$ 3.2	$ 3.2	$ 3.2	$ 0	$ 0
Impairment loss on intangible assets	0.9	6.7	0	(5.8)	6.7
Total intangible asset amortization and contingent consideration	$ 18.6	$ 18.7	$ 1.4	$ (0.1)	$ 17.3

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 2013 and 2012, the majority of the changes related to the development progress of BMN 701 and BMN 673.

In the second quarter of 2013, we recorded an impairment charge of $0.9 million related to acquired IPR&D assets consisting of 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 10 to our accompanying Consolidated Financial Statements for additional discussion.

Table of Contents

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

Equity in the Loss of BioMarin/Genzyme LLC

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

Equity in the loss of the joint venture totaled $1.1 million for the year ended December 31, 2013, compared to $1.2 million and $2.4 million for the years ended December 31, 2012 and 2011, 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 $3.1 million for the year ended December 31, 2013, compared to $2.6 million and $2.9 million for the years ended December 31 2012 and 2011, respectively. The increase in interest income during 2013, as compared to 2012 was primarily due to higher cash and investment balances. The reduction in interest income during 2012, as compared to 2011 was primarily due to lower market interest rates. We expect future interest income to increase due to the $696.4 million of net proceeds from the October 2013 issuance of $750.0 million of senior subordinated convertible notes. See Note 5 to our accompanying Consolidated Financial Statements for additional discussion.

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				
	2013	2012	2011	2013 v. 2012	2012 v. 2011
Coupon interest	$ 4.5	$ 6.6	$ 7.4	$ (2.1)	$ (0.6)
Amortization of issuance costs	1.1	1.0	1.0	0.1	0
Accretion of discount on convertible notes	4.8	0	0	4.8	0
Total interest expense	$ 10.4	$ 7.6	$ 8.4	$ 2.9	$ (0.6)

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. In 2013 we recognized debt conversion expense of $13.0 million, related to the early conversion of $262.8 million in aggregate principal of the senior subordinated convertible notes due in 2017 Notes (the 2017 Notes) in 2013. The decrease in interest expense in 2012 compared to 2011 was attributed to the early conversion of $29.2 million in aggregate principal of our senior subordinated convertible notes due in 2013 (the 2013 Notes) in September 2011. In connection with the early conversion of the 2013 Notes, we recognized debt conversion expense of $1.9 million in 2011. We expect future interest expense to increase due to the October 2013 issuance of $750.0 million of senior subordinated convertible notes and the accretion of the related debt discount. See Note 5 to our accompanying Consolidated Financial Statements for additional discussion.

Provision for (Benefit from) Income Taxes

For the year ended December 31, 2013 we recognized an income tax benefit of $0.2 million, compared to an income tax benefit of $3.9 million in 2012 and income tax expense of $10.2 million during 2011. Income tax expense for 2013 and 2012 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

provisions for 2013 and 2012 were further reduced by the benefit related to stock option exercises during the years ended December 31, 2013 and 2012. Additionally, the American Taxpayer Relief Act of 2012 (the Relief Act), was enacted on January 2, 2013, which reinstated the federal R&D credit retroactively to January 1, 2012. 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 $1.2 million related to R&D expenses incurred during 2012 as a discrete item during the year ended December 31, 2013, which further increased our income tax benefit for the current period provision. 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 20 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 $226.6 million of foreign net losses. Other foreign operations generated U.S. GAAP income of approximately $3.4 million with an effective tax rate of approximately 61%.

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, acquisitions of complementary technologies, products and companies or if we elect to settle all or a portion of our 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 offering of senior subordinated convertible notes.

We consider the unrepatriated cumulative earnings of certain of our foreign subsidiaries to be invested indefinitely outside the U.S. As of December 31, 2013, $86.8 million of our $1,052.4 million balance of cash, cash equivalents and marketable securities was from foreign subsidiary operations and is intended to fund future foreign operations. 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 adjust our business processes, as

appropriate, to mitigate these risks to our business.

As of December 31, 2013, we had placed $116.5 million in an escrow account for the purchase of the San Rafael Corporate Center (SRCC), which is expected to be completed during the first quarter of 2014. The escrow balance was included in Other Assets on our Consolidated Balance Sheet at December 31, 2013.

Table of Contents

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

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

	2013	2012	2011	2013 v. 2012	2012 v. 2011
Cash and cash equivalents	$ 568.8	$ 180.5	$ 46.3	$ 388.3	$ 134.2
Short-term investments	215.9	267.3	148.8	(51.4)	118.5
Long-term investments	267.7	116.0	94.4	151.7	21.6
Cash, cash equivalents and investments	$ 1,052.4	$ 563.8	$ 289.5	488.6	$ 274.3
Current assets	$ 1,137.4	$ 743.4	$ 469.8	$ 394.0	$ 273.6
Current liabilities	183.3	170.4	94.1	12.9	76.3
Working capital	$ 954.1	$ 573.0	$ 375.7	$ 381.1	$ 197.4
Convertible debt	$ 655.6	$ 348.2	$ 348.3	$ 307.4	$ (0.1)

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

	2013	2012	2011	2013 v. 2012	2012 v. 2011
Cash and cash equivalents at the beginning of the period	$ 180.5	$ 46.3	$ 88.1	$ 134.2	$ (41.8)
Net cash provided by (used in) operating activities	(59.6)	17.6	18.8	(77.2)	(1.2)
Net cash used in investing activities	(298.8)	(195.6)	(89.6)	(103.2)	(106.0)
Net cash provided by financing activities	746.7	312.2	29.0	434.5	283.2
Cash and cash equivalents at the end of the period	$ 568.8	$ 180.5	$ 46.3	$ 388.3	$ 134.2
Short-term and long-term investments	483.6	383.3	243.2	100.3	140.1
Cash, cash equivalents and investments	$ 1,052.4	$ 563.8	$ 289.5	$ 488.6	$ 274.3

Cash, Cash Equivalents and Investments

The increase in cash, cash equivalents and investments in 2013 from December 31, 2012 was primarily attributed to the net proceeds of $696.4 million from our October 2013 offering of senior subordinated convertible notes and employee stock exercises, offset by increases in cash used in operating activities; purchases of property, plant and equipment; the acquisition of Zacharon; the purchase of capped calls in connection with our October 2013 offering of senior subordinated convertible notes; payments to the former stockholders of LEAD Therapeutics, Inc. (LEAD) for the attainment of a clinical milestone and payments to holders of the 2017 Notes upon early conversion of the 2017 Notes.

Working Capital

Working capital increased by $381.1 million, from $573.0 million at December 31, 2012 to $954.1 million at December 31, 2013. The increase in working capital was attributed to the following:

Working capital at December 31, 2012	$ 573.0
Increased cash, cash equivalents and short-term investments	336.9
Maturity of 2013 Notes in March 2013	23.4
Increased accounts payable and accrued liabilities	(36.2)
Net increase in other current operating assets	57.0
Working capital at December 31, 2013	$ 954.1

The increase in cash, cash equivalents and short-term investments was primarily attributed to the net proceeds of $726.2 million from our October 2013 offering of senior subordinate convertible notes of which

Table of Contents

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

$29.8 was used to purchase a capped call share option. The net proceeds from the convertible note offering was partially offset by $59.6 million of cash used in operating activities, $9.9 million of net cash payments related to the Zacharon acquisition and $13.0 million paid to certain holders of the 2017 Notes in connection with the early conversion of $262.8 million in aggregate principal. During 2013 we also received proceeds of $66.2 million from employee stock option exercises.

The net increase in other current operating assets is attributed to increases of $33.9 million, $16.2 million and $8.8 million in inventory, other current assets, and accounts receivable, respectively. The increase in inventory was primarily attributed to the capitalization of VIMIZIM pre-launch inventory. The increase in other current assets is primarily attributed to a $10.0 million increase in prepaid expenses, a $3.4 million increase in short-term restricted investments and a $3.2 million increase in deferred offering costs, offset by decreases of $3.4 million in other assets. The increase in accounts receivable is attributed to timing.

Our product sales to government-owned or government-funded customers in certain Southern European countries, including 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 a default in Greece, or in other Southern European 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, 2013, approximately 16% of our outstanding accounts receivable relate to such countries. See Note 19 of our accompanying Consolidated Financial Statements for additional discussion. We also sell our products in other countries that face economic crises and local currency devaluation. Although we have historically collected receivables from customers in those countries, sustained weakness or further deterioration of the local economies and currencies may cause our customers in those countries to be unable to pay for our products with the same negative effect on our operations.

Cash Provided by (Used in) Operating Activities

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 research and development expense related to increased clinical trial activities for our product candidates PEG PAL, BMN 673 and BMN 701, 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 operating activities for the year ended December 31, 2012 was $17.6 million, compared to cash provided by operating activities of $18.8 million for the year ended December 31, 2011. The decrease in cash provided by operating activities was primarily related to increased research and development expense that drove the increase in our net loss of $114.3 million, adjusted for non-cash items such as $45.3 million of depreciation and amortization expenses, $47.3 million of stock-based compensation expense, $6.7 million of impairment loss on

intangible assets, $8.8 million decrease in the fair value of contingent acquisition consideration payable, $9.9 million decrease in deferred income taxes, $6.5 million of unrealized foreign exchange gain on forward foreign currency exchange contracts and $33.1 million of net cash inflow related to changes in operating assets and liabilities.

Table of Contents

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

Cash Used in Investing Activities

Net cash used in investing activities during the year ended December 31, 2013 was $298.8 million compared to net cash used in investing activities of $195.6 million and $89.6 million during the years ended December 31, 2012 and 2011, respectively. Our investing activities have consisted primarily of purchases and sales and maturities of investments and capital expenditures, such as manufacturing equipment and facility improvements. The increase in net cash used in investing activities for the year ended December 31, 2013 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 SRCC, offset by an increase in net maturities of investment securities of $37.9 million. The increase in net cash used in investing for the year ended December 31, 2012 was primarily comprised of a $210.9 million increase in net purchases of available-for-sale investments, offset by a $81.0 million decrease in purchases of intellectual property and a $28.6 million decrease in capital expenditures. We expect to make significant capital investments in our Shanbally, Ireland manufacturing facility beginning in 2014 to enable future commercial manufacturing of our products at the facility.

Cash Provided by Financing Activities

Net cash provided by financing activities for the year ended December 31, 2013 was $746.7 million, compared to net cash provided by financing activities of $312.2 million and $29.0 million for the years ended December 31, 2012 and 2011, 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 Employee Stock Purchase Plan (the ESPP) and employee stock option exercises. 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 $15.2 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. The increase in net cash provided by financing activities for the year ended December 31 2012, was primarily attributed to the June 2012 public offering of our common stock which generated net cash proceeds of $235.5 million, an increase of $47.8 million in proceeds from the ESPP and employee stock option exercises, a $2.2 million decrease in debt conversion expense, offset by a $2.5 million increase in payments of contingent acquisition consideration.

Other Information

On October 15, 2013, we completed an offering of $750.0 million in aggregate principal of senior subordinated convertible notes consisting of $375.0 million 0.75% due in October 2018 (the 2018 Notes) and $375.0 million 1.50% due in October 2020 (the 2020 Notes). The net proceeds from the offering were $696.4 million, after deducting commissions and offering expenses and the purchase of capped calls. The 2018 Notes and the 2020 Notes were issued at face value and accrue interest at annual rates of 0.75% and 1.50%, respectively, which is payable semiannually in arrears on April 15 and October 15 of each year beginning on April 15, 2014. See Note 5 to our accompanying Consolidated Financial Statements for additional discussion regarding the 2018 Notes and the 2020 Notes.

In April 2007, we sold approximately $324.9 million of the 2017 Notes of which $62.0 million remained outstanding at December 31, 2013. The debt was issued at face value and bears interest at the rate of 1.875% per annum, payable

semi-annually in cash. During 2013, we entered into separate agreements with 18 of the existing holders of the 2017 Notes pursuant to which such holders converted $262.8 million in aggregate principal of the 2017 Notes into 12.9 million shares of our common stock. In addition to issuing the requisite number of shares of common stock pursuant to the 2017 Notes, we also made varying cash payments to each of the holders, totaling

Table of Contents

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

an aggregate of $14.8 million, of which $13.0 million was recognized as Debt Conversion Expense in our Consolidated Statement of Operations for the year ended December 31, 2013. The remaining 2017 Notes are convertible, at the option of the holder, at any time prior to maturity, into shares of our common stock at a conversion price of approximately $20.36 per share, subject to adjustment in certain circumstances. Our debt does not contain a call provision and we are unable to unilaterally redeem the debt prior to maturity in 2017. We also must repay the debt if there is a qualifying change in control or termination of trading of our common stock. If a change of control occurs, we will pay a make whole premium by increasing the conversion rate applicable to the 2017 Notes. See Note 5 to our accompanying Consolidated Financial Statements for additional discussion.

In March 2006, we sold approximately $172.5 million the 2013 Notes, which fully matured on March 29, 2013. The debt was issued at face value and bore interest at the rate of 2.5% per annum, payable semi-annually in cash. The debt did not contain a call provision and we were unable to unilaterally redeem the remaining debt prior to maturity in March 2013. Upon maturity of the 2013 Notes, we issued 1.4 million shares of our common stock pursuant to the terms of the 2013 Notes and paid a bond holder $98,000 in cash for the par value at maturity. See Note 5 to our accompanying Consolidated Financial Statements for additional discussion.

Our $655.6 million of total convertible debt as of December 31, 2013 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 or 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

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.

Table of Contents

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

Our investment in our product development programs and continued development of our existing commercial products has a major impact on our operating performance. Our research and development expenses in each of the three years ended December 31 and the period since inception of the major programs were as follows (in millions):

	2013	2012	2011	Since Program Inception
VIMIZIM	$ 82.0	$ 97.0	$ 54.5	$293.8
Naglazyme	12.5	12.4	10.3	177.3
Kuvan	14.4	14.1	12.6	155.2
Firdapse	8.7	5.4	11.0	34.4
BMN 673	29.5	11.4	7.4	56.6
BMN 701	45.6	31.6	17.5	97.2
BMN 111	15.0	12.1	13.6	46.9
BMN 190	13.8	11.1	1.2	31.5
PEG PAL	54.5	26.7	27.7	167.7
Not allocated to specific major current projects	78.8	80.4	58.6	Not meaningful
Totals	$ 354.8	$ 302.2	$ 214.4	

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 Naglazyme, Aldurazyme, Kuvan, Firdapse and VIMIZIM; 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 Naglazyme, Aldurazyme, Kuvan, Firdapse and VIMIZIM;
- manufacture, supply or distribution of Naglazyme, Aldurazyme, Kuvan, Firdapse and VIMIZIM;
- 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;

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.

Table of Contents

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 research and development activities, purchase commitments, licenses and sales royalties with annual minimums. Our contractual obligations for non-cancelable purchase commitments as of December 31, 2013 are presented in the table below (in millions).

	Payments Due within				
	Less Than 1 Year	1-3 Years	3-5 Years	More Than 5 Years	Total
2017 Notes and related interest	$ 1.2	$ 2.4	$ 62.6	$ 0	$ 66.2
2018 Notes and related interest	2.8	5.6	380.6	0	389.0
2020 Notes and related interest	5.6	11.2	11.2	386.3	414.3
Operating leases	10.9	19.0	16.3	20.3	66.5
Research and development and purchase commitments	27.3	8.1	2.8	0	38.2
Total	$ 47.8	$ 46.3	$ 473.5	$ 406.6	$ 974.2

At December 31, 2013, our operating lease obligations included $35.9 million related to our SRCC leases, which will be terminated upon closing of the SRCC purchase in the first quarter of 2014.

We are also subject to contingent payments totaling approximately $422.2 million as of December 31, 2013, which are due upon achievement of certain regulatory and licensing milestones if they occur before certain dates in the future. Of this amount, $56.4 million relates to programs that are no longer being developed.

Table of Contents

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 $62.0 million of 1.875% convertible senior notes due 2017, $375.0 million of 0.75% convertible senior notes due 2018 and $375.0 million of 1.50% convertible senior notes due 2020. 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, 2013 the fair value of our convertible debt was $1,012.3 million.

In connection with the October 2013 offering of the 2018 Notes and the 2020 Notes, we paid $29.8 million to purchase a capped call covering 3,982,988 shares of our common stock. If the per share price of our common stock remains below $94.15, these capped call transactions would provide us no benefit in offsetting potential dilution from the 2018 Notes and the 2020 Notes. If the per share price of our common stock exceeds $121.05, then to the extent of the excess, these capped call transactions would result in additional dilution from conversion of the 2018 Notes and the 2020 Notes.

As of December 31, 2013, 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, 2013, we believe that a 100 basis point decrease in interest rates could result in a potential loss in fair value of our investment portfolio of approximately $5.6 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, 2013 (in millions):

	Carrying Value
Cash and cash equivalents	$ 568.8*
Short-term investments	215.9**
Long-term investments	267.7***
Total	$ 1,052.4

* 73% of cash and cash equivalents are invested in money market instruments and 27% in cash.
** 46% of short-term investments are invested in corporate debt securities, 40% in commercial paper and 14% in certificates of deposit.
*** 91% of long-term investments are invested in corporate debt securities, 6% in certificates of deposit and 3% in U.S. government agency securities.

Table of Contents

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 hedge a percentage of our forecasted Euro denominated revenue and operating expenses denominated in Brazilian Reais with forward foreign currency exchange contracts. 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 of less than 12 months.

As of December 31, 2013, we had forward foreign currency exchange contracts to sell approximately 41.8 million Euros and to buy approximately 36.7 million Euros. As of December 31, 2013, our outstanding forward foreign currency exchange contracts had a net fair value of $2.2 million, of which $59,000 was included in other current assets and $2.2 million 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, 2013, 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 $6.3 million. We expect to enter into new transactions based in foreign currencies that could be impacted by changes in exchange rates.

At December 31, 2013, we had cash of approximately $40.3 million denominated in foreign currencies, which represented approximately 4% 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-77 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

Table of Contents

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, 2013. 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 (1992).

Based on the COSO criteria, we believe our internal control over financial reporting as of December 31, 2013 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

Table of Contents

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 2014 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 2014 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 2014 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 2014 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 2014 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 2014 annual meeting of stockholders.

Table of Contents

Part IV

Item 15. Exhibits, Financial Statement Schedules

Financial Statements

In accordance with Rule 3-09 of Regulation S-X, the comparative unaudited 2013, 2012 and 2011 Consolidated Financial Statements and accompanying notes of BioMarin/Genzyme LLC, are filed herewith as Exhibit 99.1 to this Annual Report on Form 10-K.

Exhibit Index

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 March 29, 2006, by and 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 April 23, 2007, by and 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.

Table of Contents

4.4	Indenture, dated October 15, 2013, by and 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 October 15, 2013, by and 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 October 15, 2013, by and 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	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, previously filed with the SEC on July 31, 2009 as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated by reference herein.
10.3	Amendment to 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 1997 Stock Plan, 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	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 (Registration No. 333-77701), which is incorporated herein by reference.
10.6	Amendment to 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 1998 Director Option Plan, as adopted 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 1998 Director Option Plan, 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	

Amended and Restated 2006 Employee Stock Purchase Plan, as adopted on June 21, 2006, previously filed with the SEC on August 3, 2006 as Exhibit 10.3 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference.

Table of Contents

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 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.11	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, previously filed with the SEC on December 23, 2008 as Exhibit 10.8 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.12	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.
10.13	Amended and Restated Employment Agreement with Jean-Jacques Bienaimé dated January 1, 2009 previously filed with the Commission on December 23, 2008, as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.14	Amended and Restated Employment Agreement with Robert A. Baffi dated 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.
10.15	Amended and Restated Employment Agreement with G. Eric Davis dated 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.
10.16	Amended and Restated Employment Agreement with Mark Wood dated January 1, 2009 previously filed with the SEC on December 23, 2008 as Exhibit 10.7 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.17	Employment Agreement with Henry Fuchs, dated 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 (Registration 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 (Registration

No. 333-77701), which is incorporated herein by reference.

Table of Contents

10.22	License Agreement between BioMarin Pharmaceutical Inc. and Women s and Children s Hospital dated February 7, 2007, previously filed with the SEC on May 3, 2007 as Exhibit 10.7 to the Company s Quarterly Report on Form 10-Q, 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	Asset Purchase Agreement dated November 30, 2011, by and between a wholly owned subsidiary of BioMarin Pharmaceutical Inc. and SA Pathology, a unit of the Central Adelaide Local Health Network, previously filed with the SEC on February 22, 2012 as Exhibit 10.25 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference.
10.24	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.
10.25	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.
10.26	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.
10.27	Stock Purchase Agreement by and among BioMarin Pharmaceutical Inc., and LEAD Therapeutics Inc. and the stockholders of LEAD Therapeutics, Inc. dated February 4, 2010, previously filed with the SEC on May 3, 2010 as Exhibit 10.1 to the Company s Quarterly Report on Form 10-Q, 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.
10.28	Stock Purchase Agreement by and between BioMarin Pharmaceutical Inc., Huxley Pharmaceuticals, Inc., and the stockholders of Huxley Pharmaceuticals, Inc., dated October 20, 2009, previously filed with the SEC on February 26, 2010 as Exhibit 10.37 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.
10.29	First Amendment to Stock Purchase Agreement effective as of March 26, 2010, that amends that certain Stock Purchase Agreement, dated as of October 20, 2009 by and among BioMarin Pharmaceutical Inc. and Huxley Pharmaceuticals, Inc. and the stockholders of Huxley previously filed with the SEC on August 4, 2010 as Exhibit 10.1 to the Company s Quarterly Report on Form 10-Q, 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.
10.30	Securities Purchase Agreement dated August 17, 2010 by and among BioMarin Pharmaceutical Inc., ZyStor Therapeutics Inc., the holders of outstanding capital stock and rights to acquire capital stock of ZyStor Therapeutics Inc. and George G. Arida, as the representative of such holders, previously filed with the SEC on August 23, 2010 as Exhibit 2.1 to the Company s Current Report on Form 8-K, which is

incorporated by reference herein. The SEC has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the SEC.

Table of Contents

10.31	Asset Purchase Agreement dated June 22, 2011 between BioMarin Manufacturing Ireland Limited and Pfizer Biotechnology Ireland, previously filed with the SEC on August 1, 2011 as Exhibit 10.1 to the Company s Quarterly Report on Form 10-Q, 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.
10.32	Lease Agreement entered into on January 6, 2012 between BioMarin Pharmaceutical Inc. and SR Corporate Center Phase Two, LLC for 770 Lindaro Street, San Rafael, CA, previously filed with the SEC on February 22, 2012 as Exhibit 10.34 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference.
10.33	Lease Agreement entered into on January 6, 2012 between BioMarin Pharmaceutical Inc. and SR Corporate Center Phase Two, LLC for 790 Lindaro Street, San Rafael, CA, previously filed with the SEC on February 22, 2012 as Exhibit 10.35 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference.
10.34	Employment Agreement with Daniel Spiegelman dated May 8, 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.
10.35	Amendment No. 1 to Employment Agreement with Robert A. Baffi dated 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.
10.36	Amendment No. 1 to Employment Agreement with G. Eric Davis dated 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.
10.37	Amendment No. 1 to Employment Agreement with Henry J. Fuchs dated 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.
10.38	Amendment No. 1 to Employment Agreement with Mark Wood dated May 8, 2012, previously filed with the SEC on May 9, 2012 as Exhibit 10.7 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.39	Amendment No. 2 to Employment Agreement with Robert A. Baffi dated 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.
10.40	Amendment No. 2 to Employment Agreement with Henry J. Fuchs dated 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.
10.41	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.
10.42	First Amendment to Stock Purchase Agreement dated February 4, 2010 by and among BioMarin Pharmaceutical Inc and LEAD Therapeutics, Inc. and the Stockholders of LEAD Therapeutics dated April 13, 2012, previously filed with the SEC on August 2, 2012 as Exhibit 10.10 to the Company s Quarterly Report on Form 10-Q, 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.

10.43 Form of Stock Option 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.

Table of Contents

10.44	Form of Restricted Stock Unit Agreement for the BioMarin Pharmaceutical 2006 Share Incentive Plan. (As Amended and Restated 2010), previously filed with the SEC on August 2, 2012 as Exhibit 10.12 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference.
10.45	Form of Stock Option 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.
10.46	Form of Restricted Stock Unit Agreement 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.
10.47	Employment Agreement with Jeffrey R. Ajer dated 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.
10.48	Amendment No. 1 to Employment Agreement with Daniel Spiegelman dated 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.
10.49	Amendment No. 1 to Amended and Restated Employment Agreement with Jean-Jacques Bienaimé dated 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.
10.50	Amendment No. 1 to Employment Agreement with Jeffery R. Ajer dated 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.
10.51	Amendment No. 3 to Employment Agreement with Robert A. Baffi dated 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.
10.52	Amendment No. 3 to Employment Agreement with Henry J. Fuchs dated 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.
10.53	Amendment No. 2 to Employment Agreement with G. Eric Davis dated 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.
10.54	Amendment No. 2 to Employment Agreement with Mark Wood dated December 17, 2012, previously filed with the SEC on December 18, 2012 as Exhibit 10.7 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.55	Second Amendment to Stock Purchase Agreement effective October 26, 2012 by and among BioMarin Pharmaceutical Inc. and Huxley Pharmaceuticals, Inc. and the former stockholders of Huxley, previously filed with the SEC on February 26, 2013 as Exhibit 10.60 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.
10.56	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.

10.57	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.

Table of Contents

10.58	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.
10.59	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.
10.60	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.
10.61	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.
10.62	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.
10.63	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.
10.64	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.
10.65	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.
10.66	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.
10.67	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.
10.68*	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.
21.1*	Subsidiaries of BioMarin Pharmaceutical Inc.

Table of Contents

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.
99.1*	BioMarin/Genzyme LLC Consolidated Financial Statements as of December 31, 2013 and 2012, and for the three years ended December 31, 2013.
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.PREXBRL	Taxonomy Extension Presentation Link Document

* Filed herewith
Management contract or compensatory plan or arrangement

Table of Contents

SIGNATURES

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

BIOMARIN PHARMACEUTICAL INC.

Dated: February 26, 2014

By: /S/ DANIEL SPIEGELMAN
Daniel Spiegelman
Executive Vice President and Chief Financial Officer

Table of Contents

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)	February 26, 2014
/S/ DANIEL SPIEGELMAN **Daniel Spiegelman**	Executive Vice President and Chief Financial Officer (Principal Financial Officer)	February 26, 2014
/S/ BRIAN R. MUELLER **Brian R. Mueller**	Vice President, Corporate Controller and Chief Accounting Officer (Principal Accounting Officer)	February 26, 2014
/S/ PIERRE LAPALME **Pierre LaPalme**	Chairman and Director	February 26, 2014
/S/ KENNETH BATE **Kenneth Bate**	Director	February 26, 2014
/S/ MICHAEL G. GREY **Michael G. Grey**	Director	February 26, 2014
/S/ ELAINE HERON **Elaine Heron**	Director	February 26, 2014
/S/ V. BRYAN LAWLIS **V. Bryan Lawlis**	Director	February 26, 2014

/S/ ALAN J. LEWIS **Alan J. Lewis**	Director	February 26, 2014
/S/ RICHARD A. MEIER **Richard A. Meier**	Director	February 26, 2014
/S/ WILLIAM YOUNG **William Young**	Director	February 26, 2014

Table of Contents

BIOMARIN PHARMACEUTICAL INC.

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

Table of Contents

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 (the Company) as of December 31, 2013 and 2012, 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, 2013. 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, 2013 and 2012, and the results of their operations and their cash flows for each of the years in the three-year period ended December 31, 2013, 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. and subsidiaries internal control over financial reporting as of December 31, 2013, based on criteria established in *Internal Control Integrated Framework (1992)* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO), and our report dated February 25, 2014 expressed an unqualified opinion on the effectiveness of the Company s internal control over financial reporting.

/s/ KPMG LLP

San Francisco, California

February 26, 2014

Table of Contents

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, 2013, based on criteria established in *Internal Control Integrated Framework (1992)* 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, 2013, based on criteria established in *Internal Control Integrated Framework (1992)* issued by the Committee of Sponsoring Organizations of the Treadway Commission.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the consolidated balance sheets of BioMarin Pharmaceutical Inc. and subsidiaries as of December 31, 2013 and 2012, 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, 2013, and our report dated February 25, 2014 expressed an unqualified opinion on those consolidated financial statements.

/s/ KPMG LLP

San Francisco, California

February 26, 2014

Table of Contents

BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED BALANCE SHEETS

December 31, 2013 and 2012

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

	December 31, 2013	December 31, 2012
ASSETS		
Current assets:		
Cash and cash equivalents	$ 568,781	$ 180,527
Short-term investments	215,942	267,278
Accounts receivable, net (allowance for doubtful accounts: $529 and $348, respectively)	117,822	109,066
Inventory	162,605	128,695
Current deferred tax assets	30,561	32,356
Other current assets	41,707	25,509
Total current assets	1,137,418	743,431
Noncurrent assets:		
Investment in BioMarin/Genzyme LLC	816	1,080
Long-term investments	267,700	115,993
Property, plant and equipment, net	319,316	284,473
Intangible assets, net	163,147	162,980
Goodwill	54,258	51,543
Long-term deferred tax assets	150,391	189,303
Other assets	156,171	19,544
Total assets	$ 2,249,217	$ 1,568,347
LIABILITIES AND STOCKHOLDERS EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 183,271	$ 147,068
Convertible debt	0	23,365
Total current liabilities	183,271	170,433
Noncurrent liabilities:		
Long-term convertible debt	655,566	324,859
Long-term contingent acquisition consideration payable	30,790	30,618
Other long-term liabilities	38,549	26,674
Total liabilities	908,176	552,584
Stockholders equity:		

Common stock, $0.001 par value: 250,000,000 shares authorized at December 31, 2013 and 2012: 143,463,668 and 125,809,162 shares issued and outstanding at December 31, 2013 and 2012, respectively.	144	126
Additional paid-in capital	2,059,101	1,561,890
Company common stock held by Nonqualified Deferred Compensation Plan	(7,421)	(6,603)
Accumulated other comprehensive income (loss)	5,018	(202)
Accumulated deficit	(715,801)	(539,448)
Total stockholders equity	1,341,041	1,015,763
Total liabilities and stockholders equity	$ 2,249,217	$ 1,568,347

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

Table of Contents

BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF OPERATIONS

Years Ended December 31, 2013, 2012 and 2011

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

	2013	2012	2011
REVENUES:			
Net product revenues	$ 538,360	$ 496,497	$ 437,647
Collaborative agreement revenues	3,918	1,955	468
Royalty and license revenues	6,207	2,271	3,243
Total revenues	548,485	500,723	441,358
OPERATING EXPENSES:			
Cost of sales (excludes amortization of certain acquired intangible assets)	95,742	91,830	84,023
Research and development	354,780	302,218	214,374
Selling, general and administrative	235,356	198,173	175,423
Intangible asset amortization and contingent consideration	18,614	18,717	1,428
Total operating expenses	704,492	610,938	475,248
LOSS FROM OPERATIONS	(156,007)	(110,215)	(33,890)
Equity in the loss of BioMarin/Genzyme LLC	(1,149)	(1,221)	(2,426)
Interest income	3,083	2,584	2,934
Interest expense	(10,447)	(7,639)	(8,409)
Debt conversion expense	(12,965)	0	(1,896)
Other income (expense)	982	(1,787)	60
LOSS BEFORE INCOME TAXES	(176,503)	(118,278)	(43,627)
Provision for (benefit from) income taxes	(150)	(3,931)	10,209
NET LOSS	$ (176,353)	$ (114,347)	$ (53,836)
NET LOSS PER SHARE, BASIC AND DILUTED	$ (1.28)	$ (0.95)	$ (0.48)
Weighted average common shares outstanding, basic and diluted	137,755	120,271	112,122

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

Table of Contents

BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

Years Ended December 31, 2013, 2012 and 2011

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

	2013	2012	2011
NET LOSS	$ (176,353)	$ (114,347)	$ (53,836)
OTHER COMPREHENSIVE INCOME (LOSS):			
Net foreign currency gain (loss)	361	(301)	6
Available-for-sale securities:			
Unrealized holding gain (loss) arising during the period, net of tax impact of $(3,537), $(140) and $229 for the years ended December 31, 2013, 2012 and 2011, respectively.	6,275	388	(508)
Reclassifications to net income (loss), net of tax impact of $1, $40 and $(12) for the years ended December 31, 2013, 2012 and 2011, respectively.	(1)	(110)	27
Net Change	6,274	278	(481)
Cash flow hedges:			
Unrealized holding gain (loss) arising during the period, net of tax impact of $789, $5,114, and $(4,500) and for the years ended December 31, 2013, 2012 and 2011, respectively.	(1,366)	(8,749)	8,163
Less reclassifications to net income (loss), net of tax impact of $28, $(2,153), and $1,648 for the years ended December 31, 2013, 2012 and 2011, respectively.	49	(3,683)	2,989
Net Change	(1,415)	(5,066)	5,174
OTHER COMPREHENSIVE INCOME (LOSS), NET OF TAX	5,220	(5,089)	4,699
COMPREHENSIVE LOSS	$ (171,133)	$ (119,436)	$ (49,137)

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

Table of Contents

BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF STOCKHOLDERS EQUITY

Years Ended December 31, 2013, 2012 and 2011 (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, 2010	110,634	$ 111	$ 1,090,188	$ (1,965)	$ 188	$ (371,265)	$ 717,257
Net loss						(53,836)	(53,836)
Other comprehensive income					4,699		4,699
Issuance of common stock under Employee Stock Purchase Plan (ESPP)	333		4,411				4,411
Exercise of common stock options	1,925	2	29,710				29,712
Excess tax benefit from stock option exercises			415				415
Conversion of convertible notes	1,761	2	28,980				28,982
Restricted stock vested during the period, net	137		(531)				(531)
Common stock held by Nonqualified Deferred Compensation Plan				(1,970)			(1,970)
Stock-based compensation			43,909				43,909
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 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
	162		(1,659)				(1,659)

Restricted stock vested during the period, net							
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)
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

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

Table of Contents

BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS

Years Ended December 31, 2013, 2012 and 2011

(In thousands of U.S. dollars)

	2013	2012	2011
CASH FLOWS FROM OPERATING ACTIVITIES:			
Net loss	$ (176,353)	$ (114,347)	$ (53,836)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	47,264	44,335	35,046
Non-cash interest expense	5,875	960	1,048
Accretion of discount on investments	5,780	4,469	4,036
Equity in the loss of BioMarin/Genzyme LLC	1,149	1,221	2,426
Stock-based compensation	66,181	47,340	43,909
Impairment of intangible assets	939	6,707	0
Loss on conversion of convertible promissory note	0	2,000	0
Deferred income taxes	(9,156)	(9,921)	4,363
Excess tax benefit from stock option exercises	(733)	(473)	(415)
Unrealized foreign exchange (gain) loss on forward contracts	(658)	(6,529)	7,174
Non-cash changes in the fair value of contingent acquisition consideration payable	10,197	8,788	(1,795)
Debt conversion expense	12,965	0	1,896
Changes in operating assets and liabilities:			
Accounts receivable, net	(8,756)	(4,227)	(18,456)
Inventory	(33,910)	1,423	(20,420)
Other current assets	(12,073)	(3,506)	2,543
Other assets	1,676	(4,076)	(837)
Accounts payable and accrued liabilities	20,420	37,411	9,771
Other long-term liabilities	9,559	6,034	1,962
Net cash provided by (used in) operating activities	(59,634)	17,609	18,415
CASH FLOWS FROM INVESTING ACTIVITIES:			
Purchases of property, plant and equipment	(65,124)	(44,571)	(73,219)
Restricted funds held in escrow	(116,500)	0	0
Maturities and sales of investments	288,643	237,837	281,991
Purchase of available-for-sale investments	(395,042)	(382,168)	(215,429)
Purchase of intellectual property	0	0	(81,000)
Business acquisitions, net of cash acquired	(9,875)	0	0
Investments in BioMarin/Genzyme LLC	(885)	(1,743)	(1,903)
Investment in convertible promissory note	0	(5,000)	0
Net cash used in investing activities	(298,783)	(195,645)	(89,560)

CASH FLOWS FROM FINANCING ACTIVITIES:			
Proceeds from exercises of stock options and ESPP	66,182	81,402	33,592
Proceeds from convertible senior note offering, net	726,202	0	0
Purchase of capped call share options	(29,813)	0	0
Proceeds from public offering of common stock, net	0	235,499	0
Excess tax benefit from stock option exercises	733	473	415
Payments for debt conversion	(12,965)	0	(1,896)
Payment on maturity of 2013 convertible note	(98)	0	0
Payment of contingent acquisition consideration payable	(3,061)	(4,405)	(1,894)
Repayment of capital lease obligations	(509)	(678)	(879)
Net cash provided by financing activities	746,671	312,291	29,338
NET INCREASE IN CASH AND CASH EQUIVALENTS	388,254	134,255	(41,807)
Cash and cash equivalents:			
Beginning of period	$ 180,527	$ 46,272	$ 88,079
End of period	$ 568,781	$ 180,527	$ 46,272
SUPPLEMENTAL CASH FLOW DISCLOSURES:			
Cash paid for interest, net of interest capitalized into fixed assets	$ 2,159	$ 6,665	7,215
Cash paid for income taxes	14,897	6,582	4,395
Stock-based compensation capitalized into inventory	6,121	4,347	5,298
Depreciation capitalized into inventory	11,016	7,335	6,576
SUPPLEMENTAL CASH FLOW DISCLOSURES FROM INVESTING AND FINANCING ACTIVITIES:			
Increase (decrease) in accounts payable and accrued liabilities related to fixed assets	$ 5,001	$ (511)	320
Conversion of convertible debt	286,085	105	29,192
Deferred offering costs reclassified into additional paid-in-capital as a result of conversion of convertible debt	2,765	0	210
Increase in asset retirement obligation	90	886	2,991

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

Table of Contents

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 investigational product candidates. The Company s approved products are Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Firdapse (amifampridine phosphate), Aldurazyme (laronidase) and VIMIZIM (elosufase alpha).

Through December 31, 2013, the Company had accumulated losses of approximately $715.8 million. 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 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.

The Company is subject to a number of risks, including: the financial performance of Naglazyme, Kuvan, Firdapse, Aldurazyme and VIMIZIM; the potential need for additional financings; its ability to successfully commercialize its product candidates, if approved; the uncertainty of the Company s research and development efforts resulting in future successful commercial products; obtaining 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 transaction 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.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

(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 current 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 (Loss) 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.

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. 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 Phase 3 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

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

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 the joint venture between the Company and Genzyme Corporation (BioMarin/Genzyme LLC) 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 BioMarin/Genzyme LLC or disbursements of profits from the BioMarin/Genzyme LLC. Equity in the loss of BioMarin/Genzyme LLC includes the Company s 50% share of BioMarin/Genzyme LLC loss for the period. The investment in BioMarin/Genzyme LLC includes the Company s share of the net equity of BioMarin/Genzyme LLC.

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 projects with no alternative uses are expensed as incurred.

Leasehold improvements	Shorter of life of asset or lease term
Building and improvements	20 to 30 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	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 definite 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

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

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, 2013, the Company has only one reporting unit.

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

Revenue Recognition

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

Net Product Revenues The Company recognizes revenues from product sales when title and risk of loss have passed to the customer, which typically occurs upon delivery. Product sales transactions are evidenced by customer purchase orders, customer contracts, invoices and/or the related shipping documents. Upon recognition of revenue from product sales, provisions are made for government rebates such as Medicaid reimbursements, customer incentives such as cash discounts for prompt payment, distributor fees and expected returns of expired products, as appropriate. Amounts collected from customers and remitted to governmental authorities, which 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.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

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

The Company records allowances for product returns, if appropriate, as a reduction of revenue at the time product sales are recorded. Several factors are considered in determining whether an allowance for product returns is required, including market exclusivity of the products based on their orphan drug status, the patient population, the customers limited return rights and the Company s experience with returns. Because of the pricing of the Company s commercial products, the limited number of patients and the customers limited return rights, most customers and retailers carry a limited inventory.

However, certain international customers, usually government entities, tend to purchase larger quantities of product less frequently. Although such buying patterns may result in revenue fluctuations from quarter to quarter, the Company has not experienced any increased product returns or risk of product returns. The Company relies on historical return rates to estimate returns. Genzyme s contractual return rights for Aldurazyme are limited to defective product. Based on these factors and the fact that the Company has not experienced significant product returns to date, management has concluded that product returns will be minimal. In the future, if any of these factors and/or the history of product returns change, an allowance for product returns may be required.

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

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

The delivered item or items have value to the customer on a stand-alone basis.

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 research and development 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;

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

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 and License 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. 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 other royalty revenues but instead to include them as a component of Net Product Revenues in the Company s Consolidated Statements of Operations.

Research and Development

Research and development expenses include expenses associated with contract research and development provided by third parties, product manufacturing prior to regulatory approval, clinical and regulatory costs, and internal research and development costs. In instances where the Company enters into agreements with third parties for research and development 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 research and development projects. Amounts due under such arrangements may be either fixed fee or fee for service and may include upfront payments, monthly payments and payments upon the completion of milestones or receipt of deliverables. The Company accrues costs for clinical trial activities based upon the services received and estimates of related expenses incurred that have yet to be invoiced by the vendors that perform the activities.

Convertible Debt Transactions

The Company separately accounts for the liability and equity components of convertible debt instruments that can be settled in cash by allocating the proceeds from issuance between the liability component and the embedded conversion option, or equity component, in accordance with accounting for convertible debt instruments that may be settled in cash (including partial cash settlement) upon conversion. The value of the equity component is calculated by first measuring the fair value of the liability component, using the interest rate of a similar liability that does not have a conversion feature, as of the issuance date. The difference between the proceeds from the convertible debt issuance and the amount measured as the liability component is recorded as the equity component with a corresponding discount recorded on the debt. The Company recognizes the accretion of the resulting discount using the effective interest method as part of Interest Expense in its Consolidated Statements of Operations.

Net Loss Per Common Share

Basic net loss per share is calculated by dividing net loss by the weighted average shares of common stock outstanding during the period. Diluted net loss per share reflects the potential dilution that would occur if securities or other contracts to issue common stock were exercised or converted into common stock; however, potential common equivalent shares are excluded if their effect is anti-dilutive. The Company currently has no dilutive securities and as such, basic and diluted net loss per share are the same for the periods presented.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

Stock-Based Compensation

The Company uses the Black-Scholes option-pricing model to determine the fair value of stock options and the Company s Employee Stock Purchase Plan (the 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 several key assumptions, including closing price of the Company s stock price on grant date, 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. Restricted 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.

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.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

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, then all or a portion of the valuation allowance may need to be re-established, 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 it 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 assess, 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 14 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 or discounted cash flows. 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

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

adjustments to Intangible Asset Amortization and Contingent Consideration in the Company s Consolidated Statements of Operations. Changes in the fair value of the contingent acquisition consideration payable can result from adjustments to the estimated probability and assumed timing of achieving the underlying milestones, as well as from changes to the discount rates and periods.

Comprehensive Income (Loss) and Accumulated Other Comprehensive Income (Loss)

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 2013-02 (ASU 2013-02), *Reporting of Amounts Reclassified Out of Accumulated Other Comprehensive Income,* there have been no new accounting pronouncements or changes to accounting pronouncements during the year ended December 31, 2013, as compared to the recent accounting pronouncements described in the Company s Annual Report on Form 10-K for the year-ended December 31, 2012, that are of significance or potential significance to the Company. ASU 2013-02 requires an entity to present either on the face of the financial statements where income is presented or in the notes to the financial statements, significant amounts reclassified out of accumulated other comprehensive income by the respective line items of net income. See Note 18 to these Consolidated Financial Statements for the expanded disclosures required by ASU 2013-02.

(5) CONVERTIBLE DEBT

2018/2020 Notes

On October 15, 2013, the Company issued $750.0 million senior subordinated convertible notes consisting of $375.0 million 0.75% due in October 2018 (the 2018 Notes) and $375.0 million 1.50% due in October 2020 (the 2020 Notes and collectively the Notes). Net proceeds from the offering were $726.2 million.

The 2018 Notes and the 2020 Notes bear interest at a rate of 0.75% and 1.5% per year, respectively, which is payable semiannually in arrears on April 15 and October 15 of each year, beginning on April 15, 2014.

The Notes are senior unsecured obligations, and rank (i) equally to any of the Company s existing and future unsecured senior debt, (ii) senior to any of the Company s future indebtedness that is expressly subordinated to the Notes, and (iii) effectively junior to any secured indebtedness to the extent of the value of the assets securing such indebtedness.

Upon the occurrence of a fundamental change , as defined in the indenture, the holders may require the Company to repurchase all or a portion of the Notes for cash at 100% of the principal amount of the Notes being purchased, plus any accrued and unpaid interest.

The Notes are convertible into 7,965,975 shares of the Company s common stock under certain circumstances prior to maturity at a conversion rate of 10.6213 shares per $1,000 principal amount of the Notes,

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

which represents a conversion price of $94.15 per share, subject to adjustment under certain conditions. Holders may convert their notes at their option at any time prior to July 15, 2018, in the case of the 2018 Notes, and July 15, 2020, in the case of the 2020 Notes, only under the following circumstances: (1) during any calendar quarter commencing after the calendar quarter ending on March 31, 2014 (and only during such calendar quarter), if the last reported sale price of the Company s common stock for at least 20 trading days (whether or not consecutive) during a period of 30 consecutive trading days ending on the last trading day of the immediately preceding calendar quarter is greater than or equal to 130% of the applicable conversion price on each applicable trading day; (2) during the five business day period after any five consecutive trading day period (the measurement period) in which the trading price per $1,000 principal amount of the relevant notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price of the Company s common stock and the applicable conversion rate on each such trading day; or (3) upon the occurrence of specified corporate events.

Upon conversion, the Company may pay cash, shares of the Company s common stock or a combination of cash and stock, as determined by the Company in its discretion.

The Company has separately accounted for the liability and equity components of the Notes by allocating the proceeds from issuance of the Notes between the liability component and the embedded conversion option, or equity component. This allocation was done by first estimating an interest rate at the time of issuance for similar notes that do not include the embedded conversion option. The Company allocated $156.2 million to the equity component, net of offering costs of $5.1 million. The Company recorded a discount on the notes of $161.3 million which will be accreted and recorded as additional interest expense over the life of the Notes. During 2013, the Company recognized $2.6 million and $2.2 million, for the 2018 Notes and the 2020 Notes, respectively. The effective interest rate on the liability component of the Notes for the year ended December 31, 2013 was 7.5%.

In connection with the issuance of the Notes, the Company incurred $23.8 million of issuance costs. These costs are being amortized and are recorded as additional interest expense over the life of the Notes. During 2013, the Company recognized $0.4 million and $0.3 million of amortization of deferred offering costs, for the 2018 Notes and the 2020 Notes, respectively.

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

2017 Notes

In April 2007, the Company sold $324.9 million of senior subordinated convertible notes due in April 2017 (the 2017 Notes), of which $62.0 million remained outstanding at December 31, 2013. The 2017 Notes were issued at face value and bear interest at the rate of 1.875% per annum, payable semi-annually in cash. The 2017 Notes are convertible, at the option of the holder, at any time prior to maturity or redemption, into shares of the Company s common stock at a conversion price of $20.36 per share, subject to adjustment in certain

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

circumstances. The 2017 Notes do not include a call provision and the Company is unable to unilaterally redeem the 2017 Notes prior to maturity on April 23, 2017. The Company also must repay the 2017 Notes if there is a qualifying change in control or termination of trading of its common stock. If a change of control occurs, the Company will pay a make whole premium by increasing the conversion rate applicable to the 2017 Notes.

In connection with the placement of the 2017 Notes, the Company paid $8.5 million in offering costs, which have been deferred and are included in other assets. The deferred offering costs are being amortized as interest expense over the life of the debt. For the year ended December 31, 2013, the Company recognized amortization expense of $0.4 million, compared to $0.9 million in each of the years ended December 31, 2012 and 2011.

During 2013, the Company entered into separate agreements with 18 of the existing holders of the 2017 Notes pursuant to which such holders converted $262.8 million in aggregate principal amount of the 2017 Notes into 12,906,780 shares of the Company s common stock. In addition to issuing the requisite number of shares of the Company s common stock pursuant to the 2017 Notes, the Company also made varying cash payments to each of the holders, totaling $14.8 million in the aggregate, of which $13.0 million was recognized in total as Debt Conversion Expense in the Company s Consolidated Statement of Operations for the year ended December 31, 2013 and $1.8 million was for accrued interest. Additionally, the Company reclassified $2.8 million of deferred offering costs to additional paid-in capital in connection with the conversion of the 2017 Notes.

2013 Notes

In March 2006, the Company sold $172.5 million of senior subordinated convertible notes due in March 2013 (the 2013 Notes), which fully matured on March 29, 2013. The 2013 Notes were issued at face value and bore interest at the rate of 2.5% per annum, payable semi-annually in cash. The 2013 Notes were convertible, at the option of the holder, at any time prior to maturity or redemption, into shares of the Company s common stock at a conversion price of $16.58 per share, subject to adjustment in certain circumstances. The 2013 Notes did not include a call provision and the Company was unable to unilaterally redeem the debt prior to maturity on March 29, 2013. Upon maturity of the remaining 2013 Notes outstanding in March 2013, the Company issued the requisite 1,403,735 shares of common stock pursuant to the 2013 Notes to the bond holders, in exchange for $23.3 million in principal and paid one bond holder the par value at maturity in cash totaling $98.

In September 2011, the Company entered into separate agreements with six of the existing holders of its 2013 Notes pursuant to which such holders converted $29.2 million in aggregate principal amount of the 2013 Notes into 1,760,178 shares of the Company s common stock. In addition to issuing the requisite number of shares of the Company s common stock pursuant to the 2013 Notes, the Company paid the holders future interest of approximately $1.1 million along with $0.8 million related to varying cash premiums for agreeing to convert the 2013 Notes, which was recognized in total as debt conversion expense on the Company s Consolidated Statement of Operations for the year ended December 31, 2011. Additionally, the Company reclassified $0.2 million of deferred offering costs to Additional Paid-In Capital in connection with the conversion of the 2013 Notes. During 2012 and 2011, certain note holders voluntarily exchanged an insignificant number of convertible notes for shares of the Company s common stock.

In connection with the placement of the 2013 Notes, the Company paid approximately $5.5 million in offering costs, which were deferred and were included in other assets. The deferred offering costs were amortized as interest expense over the life of the debt. For the year ended December 31, 2013, the Company recognized amortization expense of $27, compared to $0.1 million and $0.2 million in the years ended December 31, 2012 and 2011, respectively.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

The following table summarizes information regarding the Company s convertible debt at December 31:

	2013	2012
Short-Term:		
Convertible Notes due 2013:	$ 0	$ 23,365
Total short-term convertible debt	$ 0	$ 23,365
Long-term:		
Convertible Notes due 2020, net of unamortized discount of $87,975	$ 287,025	$ 0
Convertible Notes due 2018, net of unamortized discount of $68,500	306,500	0
Convertible Notes due 2017	62,041	324,859
Total long-term convertible debt, net of unamortized discount	$ 655,566	$ 324,859
Total convertible debt, net of unamortized discount	$ 655,566	$ 348,224
Fair value of fixed rate convertible debt		
Convertible Notes due in 2020 (1)	$ 400,879	$ 0
Convertible Notes due in 2018 (1)	397,691	0
Convertible Notes due in 2017 (1)	213,765	788,433
Convertible Notes due in 2013 (1)	0	23,365
Total	$ 1,012,335	$ 811,798

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

Interest expense on the Company s convertible debt was comprised of the following:

	Years Ended December, 31		
	2013	2012	2011
Coupon interest	$ 4,550	$ 6,678	$ 7,361
Amortization of issuance costs	1,053	960	1,048
Accretion of debt discount	4,821	0	0
Total interest expense on convertible debt	$ 10,424	$ 7,638	$ 8,409

See Note 6 to these Consolidated Financial Statements for further discussion of the effect of conversion on net loss per common share.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

(6) NET LOSS PER COMMON SHARE

Potentially issuable shares of common stock include shares issuable upon the exercise of outstanding employee stock option awards, common stock issuable under the ESPP, unvested restricted stock, common stock held by the Deferred Compensation Plan and contingent issuances of common stock related to convertible debt. The table below presents potential shares of common stock that were excluded from the computation as they were anti-dilutive using the treasury stock method (in thousands):

	2013	2012	2011
Options to purchase common stock	13,157	13,895	16,319
Common stock issuable under the 2013 and 2017 Notes	3,047	17,365	17,372
Common stock issuable under the 2018 and 2020 Notes	7,966	0	0
Unvested restricted stock units	1,159	1,165	1,068
Potentially issuable common stock for ESPP purchases	197	263	241
Common stock held by the Nonqualified Deferred Compensation Plan	193	233	173
Total number of potentially issuable shares	25,719	32,921	35,173

The Company accounts for the effect of the 2018 Notes and the 2020 Notes on diluted net loss per share using the treasury stock method since they may be settled in cash or shares at the Company s option. As a result, the 2018 Notes and the 2020 Notes have no effect on diluted net loss per share until the Company s stock price exceeds the conversion price of $94.15 per share for the Notes. In the period of conversion, the Notes will have no impact on diluted net loss if the Notes are settled in cash and will have an impact on dilutive loss per share if the Notes are settled in shares upon conversion.

(7) ACQUISITION OF ZACHARON PHARMACEUTICALS, INC.

On January 4, 2013, the Company entered into a merger agreement with Zacharon Pharmaceuticals, Inc. (Zacharon), a private biotechnology company focused on developing small molecules targeting pathways of glycan and glycolipid metabolism, for a total purchase price of $11.5 million.

In connection with its acquisition of Zacharon, the Company made an upfront payment of $9.7 million in cash to the Zacharon stockholders for all of the outstanding common stock of Zacharon, net of transaction cost of $0.8 million paid on behalf of the Zacharon stockholders. The transactions costs related to this acquisition were recognized as Sales, General and Administrative expense on the Company s Statement of Operations for the year ended December 31, 2013. The Company also 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. The fair value of the contingent acquisition consideration payments was $1.9 million and was estimated by applying a probability-based income approach utilizing an appropriate discount rate. This estimation was based on significant inputs that are not observable in the market, referred to as Level 3 inputs. Key assumptions

included a discount rate of 4.7% and various probability factors. The range of outcomes and assumptions used to develop these estimates have been updated to estimate the fair value of the contingent consideration payable as of December 31, 2013. See Note 15 to these Consolidated Financial Statements for additional discussion regarding fair value measurements of the contingent acquisition consideration payable.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

The following table presents the final allocation of the purchase consideration for the Zacharon acquisition, including the contingent acquisition consideration payable, based on fair value. The final allocation includes an adjustment to goodwill and the deferred tax assets of approximately $0.7 million resulting from the finalization of Zacharon s tax returns.

Cash and cash equivalents	$ 560
Other current assets	216
Property, plant and equipment	398
Acquired deferred tax assets	2,625
Other assets	38
IPR&D	11,680
Total identifiable assets acquired	$ 15,517
Accounts payable and accrued expenses	$ (1,182)
Debt assumed	(1,313)
Deferred tax liability	(4,217)
Total liabilities assumed	$ (6,712)
Net identifiable assets acquired	$ 8,805
Goodwill	2,715
Net assets acquired	$ 11,520

A substantial portion of the assets acquired consisted of intangible assets related to Zacharon s SENSI-Pro assay. The Company determined that the estimated acquisition-date fair value of the intangible assets related to the SENSI-Pro assay was $11.7 million.

The $2.6 million of deferred tax assets resulting from the acquisition was primarily related to federal and state net operating loss and tax credit carryforwards. The $4.2 million of deferred tax liabilities relates to the tax impact of future amortization or possible impairments associated with the identified intangible assets acquired, which are not deductible for tax purposes.

The excess of the consideration transferred over the fair values assigned to the assets acquired and liabilities assumed was $2.7 million, which represents the amount of goodwill resulting from the acquisition. The Company believes that the goodwill primarily represents synergies expected from the acquisition and other benefits that do not qualify for separate recognition as acquired intangible assets. None of the goodwill is expected to be deductible for income tax purposes. The Company recorded the goodwill in the Company s Consolidated Balance Sheet as of the acquisition date.

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

See Note 10 to these Consolidated Financial Statements for further discussion of the acquired intangible assets.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

(8) INVESTMENTS

All investments were classified as available-for-sale at December 31, 2013 and 2012. 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, 2013 and 2012 are summarized in the tables below:

	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Aggregate Fair Value at December 31, 2013
Certificates of deposit	$ 47,008	$ 2	$ 0	$ 47,010
Corporate debt securities	341,519	313	(423)	341,409
Commercial paper	86,154	24	0	86,178
U.S. Government agency securities	8,900	1	0	8,901
Greek government-issued bonds	52	92	0	144
Total	$ 483,633	$ 432	$ (423)	$ 483,642

	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Aggregate Fair Value at December 31, 2012
Certificates of deposit	$ 48,741	$ 14	$ (1)	$ 48,754
Corporate debt securities	316,709	402	(211)	316,900
U.S. Government agency securities	17,512	5	0	17,517
Greek government-issued bonds	48	52	0	100
Total	$ 383,010	$ 473	$ (212)	$ 383,271

Strategic Investments

The Company has an investment in marketable equity securities which is measured using quoted prices in its respective active market that is considered a strategic investment. As of December 31, 2013, the fair value of the Company s marketable equity securities of $13.0 million includes an unrealized gain of $10.1 million. As of December 31, 2012, the fair value of the Company s marketable equity securities of $2.9 million included an unrealized loss of $0.1 million. This investment is recorded in Other Assets in the Company s Consolidated Balance

Sheets.

The fair values of available-for-sale securities by contractual maturity at December 31, 2013 and 2012 were as follows:

	December 31,	
	2013	2012
Maturing in one year or less	$ 215,942	$ 267,278
Maturing after one year through three years	267,700	115,993
Total	$ 483,642	$ 383,271

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, 2013, some of the Company s investments were in an unrealized loss position. However, none of the underlying investments has been in a continuous loss position longer than twelve months, and no other-than-temporary impairment is deemed to have occurred.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

See Note 15 to these Consolidated Financial Statements for additional discussion regarding the fair value of the Company s available-for-sale securities.

(9) GOODWILL

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

The following table represents the changes in goodwill for the year ended December 31, 2013:

Balance at December 31, 2012	$ 51,543
Addition of goodwill related to the acquisition of Zacharon	2,715
Balance at December 31, 2013	$ 54,258

(10) INTANGIBLE ASSETS

Intangible assets consisted of the following:

	December 31,	
	2013	**2012**
Intangible assets:		
Finite-lived intangible assets	$ 118,242	$ 118,242
Indefinite-lived intangible assets	74,430	63,689
Gross intangible assets:	192,672	181,931
Less: Accumulated amortization	(29,525)	(18,951)
Net carrying value	$ 163,147	$ 162,980

Finite-Lived Intangible Assets

The following table summarizes the annual amortization of the finite-lived intangible assets through 2023:

	Net Balance at December 31, 2013	Estimated Useful Life	Remaining Life	Annual Amortization
Naglazyme intellectual property	$ 66,938	12 years	9.9 years	$ 6,750
EU marketing rights for Firdapse	20,141	10 years	6.2 years	3,223
License payment for Kuvan FDA Approval	316	7 years	1.0 years	332
License payment for Kuvan EMEA Approval	1,322	10 years	4.9 years	269
Total	$ 88,717			$ 10,574

In November 2011, the Company entered into an asset purchase agreement to purchase certain intellectual property from SA Pathology, a unit of the Central Adelaide Local Health Network located in Adelaide, Australia, for an upfront cash payment of $81.0 million. The intellectual property purchased by the Company includes issued and pending patents related to the purified form of Naglazyme and the method of using the enzyme in the treatment of Mucopolysaccharidosis VI, which expire between 2022 and 2023. Prior to this purchase, the Company licensed this intellectual property from SA Pathology and paid to them a 5% royalty on net sales of Naglazyme. In the years ended December 31, 2013, 2012 and 2011, the Company recognized amortization

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

expense of $6.8 million, $6.8 million and $0.5 million, respectively, related to the Naglazyme intellectual property as a component of Cost of Sales in the Company s Consolidated Statements of Operations.

Indefinite-Lived Intangible Assets

Indefinite-lived intangible assets consist of IPR&D assets related to both early and late stage product candidates purchased in the acquisitions of Huxley, LEAD Therapeutics, Inc. (LEAD), ZyStor Therapeutics, Inc. (ZyStor) and Zacharon. In estimating fair value of the IPR&D assets, the Company compensated for the differing phases of development of each asset by probability-adjusting its estimation of the expected future cash flows associated with each asset. The Company then determined the present value of the expected future cash flows. The projected cash flows from the IPR&D assets were based on key assumptions such as estimates of revenues and operating profits related 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.

Indefinite-lived intangible assets consisted of the following:

	December 31,	
	2013	2012
In-Process Research and Development:		
BMN 673 acquired through LEAD	$ 35,150	$ 36,089
BMN 701 acquired through ZyStor	25,010	25,010
SENSI-Pro assay acquired through Zacharon	11,680	0
Other acquired pre-clinical compounds	2,590	2,590
Net carrying value	$ 74,430	$ 63,689

Intangible assets related to IPR&D assets are considered to be indefinite-lived until the completion or abandonment of the associated research and development 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 2013, the Company performed its annual impairment review and determined that no impairments existed as of December 31, 2013.

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.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

(11) PROPERTY, PLANT AND EQUIPMENT

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

	December 31,	
	2013	2012
Leasehold improvements	$ 73,973	$ 65,918
Building and improvements	159,125	144,700
Manufacturing and laboratory equipment	95,126	79,915
Computer hardware and software	74,948	56,011
Furniture and equipment	12,367	11,143
Land	11,608	11,608
Construction-in-progress	77,212	64,300
	504,359	433,595
Less: Accumulated depreciation	(185,043)	(149,122)
Total property, plant and equipment, net	$ 319,316	$ 284,473

Depreciation expense for the years ended December 31, 2013, 2012 and 2011 was $36.5 million, $34.9 million and $31.9 million, respectively, of which $11.0 million, $7.3 million and $6.6 million was capitalized into inventory, respectively.

As of December 31, 2013 and 2012, $59.1 million and $53.5 million, respectively of our property, plant and equipment was related to the Company s manufacturing facilities in Shanbally, Cork, Ireland.

On December 17, 2013, the Company entered into a Contract of Purchase and Sale and Joint Escrow Instructions (the Purchase Agreement) to purchase the office complex and vacant land commonly known as the San Rafael Corporate Center (the SRCC), located in the City of San Rafael, California. The Company currently leases approximately 40% of the complex, which it uses as its corporate headquarters. Subject to the adjustments provided in the Purchase Agreement, the purchase price of the SRCC is expected to be $116.5 million. At December 31, 2013 the Company had deposited $116.5 million into escrow in connection with the pending transaction which is expected to close during the first quarter of 2014. The Purchase Agreement contains customary representations and warranties, covenants, closing conditions and termination provisions. See Note 24 to these Consolidated Financial Statements for additional discussion regarding the Company s Minimum Lease Commitments related to SRCC.

Capitalized interest related to the Company s property, plant and equipment purchases for each of the three years ended December 31, 2013 was insignificant.

(12) INVENTORY

Inventory consisted of the following:

	December 31,	
	2013	2012
Raw materials	$ 15,309	$ 11,943
Work-in-process	88,417	71,443
Finished goods	58,879	45,309
Total inventory	$ 162,605	$ 128,695

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

Inventory as of December 31, 2013 and 2012 included $40.5 million and $0, respectively, of VIMIZIM inventory related to the pre-launch VIMIZIM manufacturing campaign. The Company believes that all material uncertainties related to the ultimate regulatory approval of VIMIZIM for commercial sale have been significantly reduced based on positive data from Phase 3 clinical trial results, successful pre-filing meetings with the FDA for the Biologics License Application (the BLA), the filing of the BLA with the FDA in the first quarter of 2013, and the filing of the Marketing Authorization Application (MAA) filed with the EMA in April 2013. In its evaluation, the Company also considered its historical experience with developing and commercially producing similar products.

Inventory as of December 31, 2013 and 2012 also included $0.3 million and $12.0 million, respectively, of product manufactured using certain process and specification changes that have not yet received regulatory approval. Although a product may have been approved by a regulatory agency, the process and specification changes must also be approved before product produced with the alternate processes and specifications can be sold commercially.

The Company expects to receive regulatory approval and has determined that it is probable that the Company will realize the future economic benefit associated with the costs of these inventories through future sales.

(13) SUPPLEMENTAL BALANCE SHEET INFORMATION

Other Assets consisted of the following:

	December 31,	
	2013	**2012**
Deposits	$ 7,196	$ 6,844
Restricted investments	412	3,493
Escrow balance for SRCC purchase	116,500	0
Deferred offering costs	15,374	3,675
Strategic investment	13,000	2,933
Other	3,689	2,599
Total other assets	$ 156,171	$ 19,544

Accounts payable and accrued liabilities consisted of the following:

	December 31,	
	2013	**2012**
Accounts payable	$ 36,894	$ 23,993
Accrued accounts payable	58,408	43,156
Accrued vacation expense	10,487	8,403

Accrued compensation expense	33,496	27,530
Accrued royalties payable	5,829	4,991
Accrued rebates payable	10,429	9,625
Other accrued operating expenses	4,875	6,179
Current portion of nonqualified deferred compensation liability	1,363	6,440
Value added taxes payable	3,603	2,072
Current portion of contingent acquisition consideration payable	11,882	10,764
Other	6,005	3,915
Total accounts payable and accrued liabilities	$ 183,271	$ 147,068

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

The roll forward of significant estimated accrued rebates, reserve for cash discounts and allowance for doubtful accounts for 2013, 2012 and 2011 was as follows:

	Balance at Beginning of Period	Provision for Current Period Sales	Provision/ (Reversals) for Prior Period Sales	Actual Charges Related to Current Period Sales	Actual Charges Related to Prior Period Sales	Balance at End of Period
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	0	(4,191)	(342)	388
Sales return reserve	0	907	0	0	0	907
Allowance for doubtful accounts	348	138	43	0	0	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	0	(4,184)	0	372
Allowance for doubtful accounts	513	0	(165)	0	0	348
Year ended December 31, 2011:						
Accrued rebates	$ 5,899	$ 14,369	$ (639)	$ (10,042)	$ (3,562)	$ 6,025
Reserve for cash discounts	304	3,543	0	(3,209)	(296)	342
Allowance for doubtful accounts	64	0	1,053	0	(604)	513

(14) 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 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 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 Naglazyme product revenues, Aldurazyme royalty revenues, operating expenses and net 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 15 to these Consolidated

Financial Statements for additional discussion regarding the fair value of forward foreign currency exchange contracts.

At December 31, 2013, the Company had 34 forward foreign currency exchange contracts outstanding to sell a total of 41.8 million Euros with expiration dates ranging from January 2014 through December 2014. These hedges were entered into in order to protect against the fluctuations in revenue associated with Euro denominated Naglazyme and Aldurazyme 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.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

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 Selling, General and Administrative expense in the Company s Consolidated Statements of Operations. At December 31, 2013, the Company had one outstanding forward foreign currency exchange contract to sell 36.7 million Euros, which was not designated as a hedge for accounting purposes and which matured on January 31, 2014.

The maximum length of time over which the Company is hedging its exposure to the reduction in value of forecasted foreign currency cash flows through forward foreign currency exchange contracts is through December 31, 2014. Over the next twelve months, the Company expects to reclassify $2.4 million from accumulated other comprehensive income to earnings as the forecasted revenue transactions.

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

	Asset Derivatives		Liability Derivatives	
	December 31, 2013		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	$ 0	Accounts payable and accrued liabilities	$ 2,186
Forward foreign currency exchange contracts	Other assets	0	Other long-term liabilities	0
Total		$ 0		$ 2,186
Derivatives not designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	$ 59	Accounts payable and accrued liabilities	$ 0
Total		59		0
Total value of derivative contracts		$ 59		$ 2,186

	Asset Derivatives		Liability Derivatives	
	December 31, 2012		December 31, 2012	
	Balance Sheet Location	Fair Value	Balance Sheet Location	Fair Value
Derivatives designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	$ 1,463	Accounts payable and accrued liabilities	$ 1,078
Forward foreign currency exchange contracts	Other assets	0	Other long-term liabilities	368
Total		$ 1,463		$ 1,446
Derivatives not designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	$ 84	Accounts payable and accrued liabilities	$ 0
Total		84		0
Total value of derivative contracts		$ 1,547		$ 1,446

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

The effect of the Company s derivative instruments on the Consolidated Financial Statements for the years ended December 31, 2013, 2012 and 2011 was as follows:

	Forward Foreign Currency Exchange Contracts		
	2013	2012	2011
Derivatives Designated as Hedging Instruments:			
Net gain (loss) recognized in Other Comprehensive Income (OCI) (1)	$ (1,366)	$ (8,749)	$ 8,163
Net gain (loss) reclassified from accumulated OCI into income (2)	49	(3,683)	2,989
Net gain (loss) recognized in income (3)	310	927	(1,486)
Derivatives Not Designated as Hedging Instruments:			
Net gain (loss) recognized in income (4)	$ (2,041)	$ 674	$ 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, 2013, 2012 and 2011, accumulated other comprehensive income before taxes associated with forward foreign currency exchange contracts qualifying for hedge accounting treatment was a loss of $2.4 million and a gain of $0.2 million and a loss of $8.0 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.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

(15) 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, 2013			
	Quoted Price in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Cash and cash equivalents:				
Overnight deposits	$ 156,228	$ 0	$ 0	$ 156,228
Money market instruments	0	412,553	0	412,553
Total cash and cash equivalents	$ 156,228	$ 412,553	$ 0	$ 568,781
Available-for-sale securities:				
Short-term:				
Certificates of deposit	$ 0	$ 30,513	$ 0	$ 30,513
Corporate debt securities	0	99,251	0	99,251
Commercial paper	0	86,178	0	86,178
Long-term:				
Certificates of deposit	0	16,497	0	16,497
Corporate debt securities	0	242,158	0	242,158
U.S. Government agency securities	0	8,901	0	8,901
Greek government-issued bonds	0	144	0	144
Total available-for-sale securities	$ 0	$ 483,642	$ 0	$ 483,642
Other Current Assets:				
Nonqualified Deferred Compensation Plan assets	$ 0	$ 136	$ 0	$ 136
Forward foreign currency exchange contract assets (1)	0	59	0	59
Restricted investments (2)	0	5,670	0	5,670
Total other current assets	$ 0	$ 5,865	$ 0	$ 5,865

Other Assets:				
Nonqualified Deferred Compensation Plan assets	$ 0	$ 3,459	$ 0	$ 3,459
Restricted investments (2)	0	412	0	412
Strategic investment (3)	13,000	0	0	13,000
Total other assets	$ 13,000	$ 3,871	$ 0	$ 16,871
Total assets	$ 169,228	$ 905,931	$ 0	$ 1,075,159
Liabilities:				
Current Liabilities:				
Nonqualified Deferred Compensation Plan liability	$ 1,227	$ 136	$ 0	$ 1,363
Forward foreign currency exchange contract liability (1)	0	2,186	0	2,186
Contingent acquisition consideration payable	0	0	11,882	11,882
Total current liabilities	$ 1,227	$ 2,322	$ 11,882	$ 15,431
Other long-term liabilities:				
Nonqualified Deferred Compensation Plan liability	$ 12,345	$ 3,459	$ 0	$ 15,804
Contingent acquisition consideration payable	0	0	30,790	30,790
Asset retirement obligation	0	0	4,122	4,122
Total other long-term liabilities	$ 12,345	$ 3,459	$ 34,912	$ 50,716
Total liabilities	$ 13,572	$ 5,781	$ 46,794	$ 66,147

Table of Contents

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, 2012			
	Quoted Price in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Cash and cash equivalents:				
Overnight deposits	$ 54,018	$ 0	$ 0	$ 54,018
Money market instruments	0	126,509	0	126,509
Total cash and cash equivalents	$ 54,018	$ 126,509	$ 0	$ 180,527
Available-for-sale securities:				
Short-term:				
Certificates of deposit	$ 0	$ 36,615	$ 0	$ 36,615
Corporate debt securities	0	222,147	0	222,147
U.S. Government agency securities	0	8,516	0	8,516
Long-term:				
Certificates of deposit	0	12,139	0	12,139
Corporate debt securities	0	94,753	0	94,753
U.S. Government agency securities	0	9,001	0	9,001
Greek government-issued bonds	0	100	0	100
Total available-for-sale securities	$ 0	$ 383,271	$ 0	$ 383,271
Other Current Assets:				
Nonqualified Deferred Compensation Plan assets	$ 0	$ 2,052	$ 0	$ 2,052
Forward foreign currency exchange contract asset (1)	0	1,547	0	1,547
Restricted investments (2)	0	2,243	0	2,243
Total other current assets	$ 0	$ 5,842	$ 0	$ 5,842
Other Assets:				
Nonqualified Deferred Compensation Plan assets	$ 0	$ 2,375	$ 0	$ 2,375
Restricted investments (2)	0	3,492	0	3,492
Strategic investment (3)	2,933	0	0	2,933

Total other assets	$ 2,933	$ 5,867	$ 0	$ 8,800
Total assets	$ 56,951	$ 521,489	$ 0	$ 578,440
Liabilities:				
Current Liabilities:				
Nonqualified Deferred Compensation Plan liability	$ 6,440	$ 0	$ 0	$ 6,440
Forward foreign currency exchange contract liability (1)	0	1,078	0	1,078
Contingent acquisition consideration payable	0	0	10,764	10,764
Asset retirement obligation	0	0	1,685	1,685
Total current liabilities	$ 6,440	$ 1,078	$ 12,449	$ 19,967
Other long-term liabilities:				
Nonqualified Deferred Compensation Plan liability	$ 5,041	$ 4,427	$ 0	$ 9,468
Forward foreign currency exchange contract liability (1)	0	368	0	368
Contingent acquisition consideration payable	0	0	30,618	30,618
Asset retirement obligation	0	0	2,192	2,192
Total other long-term liabilities	$ 5,041	$ 4,795	$ 32,810	$ 42,646
Total liabilities	$ 11,481	$ 5,873	$ 45,259	$ 62,613

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

(1) See Note 14 to these Consolidated Financial Statements for further information regarding the derivative instruments.
(2) The restricted investments secure the Company s irrevocable standby letter of credit obtained in connection with the Company s new corporate facility lease agreements and certain commercial agreements.
(3) The Company has an investment in marketable equity securities measured using quoted prices in an active market that is considered a strategic investment. See Note 6 to these Consolidated Financial Statements for additional discussion regarding the Company s strategic investment.

There were no transfers between levels during the year ended December 31, 2013.

The Company s Level 2 securities are valued using third-party pricing sources. The pricing services utilize industry standard valuation models, including both income and market-based approaches, for which all significant inputs are observable, either directly or indirectly, to estimate fair value. These inputs include reported trades of and broker/dealer quotes on the same or similar securities, issuer credit spreads, benchmark securities, prepayment/default projections based on historical data and other observable inputs.

The Company validates the prices provided by its third-party pricing services by understanding the models used, obtaining market values from other pricing sources, analyzing pricing data in certain instances and confirming those securities traded in active markets. See Note 8 to these Consolidated Financial Statements for further information regarding the Company s financial instruments.

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

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

Contingent acquisition consideration payable at December 31, 2012	$ 41,382
Changes in the fair value of the contingent acquisition consideration payable	14,453
Addition of contingent consideration payable related to the Zacharon acquisition	1,857
Milestone payments to former LEAD shareholders	(15,020)
Contingent acquisition consideration payable at December 31, 2013	$ 42,672

Under certain of the Company s lease agreements, the Company is contractually obligated to return leased space to its original condition upon termination of the lease agreement. The Company records an asset retirement obligation liability and a corresponding capital asset in an amount equal to the estimated fair value of the obligation when

estimable. In subsequent periods, for each such lease, the Company records interest expense to accrete the asset retirement obligation liability to full value and depreciates each capitalized asset retirement obligation asset, both over the term of the associated lease agreement.

Asset retirement obligations at December 31, 2012	$ 3,877
Accretion	155
Accruals added for new leases	90
Asset retirement obligations at December 31, 2013	$ 4,122

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

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

(16) STOCKHOLDERS EQUITY

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 (the Share Incentive Plan). The 2012 Inducement Plan expired in March 2013.

Share Incentive Plan

BioMarin s 2006 Share Incentive Plan (Share Incentive Plan), which replaced the Company s previous stock option plans (the 1997 Stock Plan and the 1998 Directors Options Plan), provides for grants of options to employees to purchase common stock at the fair market value of such shares on the grant date, as well as other forms of equity compensation. As of December 31, 2013, awards issued under the 2006 Share Incentive Plan include both stock options and restricted RSUs. Stock option awards granted to employees generally vest over a four-year period on a cliff basis six months after the grant date and then monthly thereafter. The term of the outstanding options is generally ten years. RSUs granted to employees generally vest in a straight-line annually over a four-year period after the grant date. Restricted stock units granted to directors generally vest in full one year after the grant date.

As of December 31, 2013, options to purchase approximately 0.4 million, 12.3 million and 0.5 million shares were outstanding under the 2012 Inducement Plan, the Share Incentive Plan, and the Company s previous stock option plans, respectively.

As of December 31, 2013, an aggregate of approximately 21.5 million and 0.7 million unissued shares were authorized for future issuance under the Share Incentive Plan and 2012 Inducement Plan, respectively.

Employee Stock Purchase Plan

Under BioMarin s ESPP, which was approved in June 2006 and replaced the Company s previous plan, employees meeting specific employment qualifications are eligible to participate and can purchase shares on established dates (each purchase date) semi-annually through payroll deductions at the lower of 85% of the fair market value of the stock at the commencement of the offering period or each purchase date of the offering period. Each offering period will span up to two years. The ESPP permits eligible employees to purchase common stock through payroll deductions for up to 10% of qualified compensation, up to an annual limit of $25,000. The ESPP is intended to qualify as an employee stock purchase plan under Section 423 of the Internal Revenue Code. During 2013, the Company issued 253,710 shares under the ESPP.

As of December 31, 2013 there were approximately 0.4 million shares reserved for future issuance under the ESPP.

Board of Director Grants

An initial option is granted to each new outside member of BioMarin s Board of Directors to purchase 30,000 shares of common stock at the fair value on the date of the grant. Until January 2007, on each anniversary date of becoming a director, each outside director was granted options to purchase 30,000 shares of common

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

stock at the fair market value on such date. Currently, on the date of each annual meeting of stockholders, other than newly elected directors, each outside director is granted options for the purchase of 15,000 shares of common stock and 2,500 RSUs. The options vest over one year and have a term of ten years. The RSUs vest on the one year anniversary of the date of grant.

Stockholders Rights Plan

The Company s Rights Plan expired on May 30, 2012. As a result, each outstanding share of the Company s common stock is no longer accompanied by a Right. The holders of common stock were not entitled to any payment as a result of the expiration of the Rights Agreement and the Rights issued thereunder.

At December 31, 2013, an aggregate of approximately 23.2 million unissued shares was authorized for future issuance under the Company s stock plans, which includes shares issuable under the Share Incentive Plan and the ESPP. Under the Share Incentive Plan awards that expire or are cancelled without delivery of shares generally become available for issuance under the respective plan. Awards that expire or are cancelled under the Company s suspended 1997 Stock Plan, 1998 Director Option Plan or 2012 Inducement Plan may not be reissued.

(17) STOCK-BASED COMPENSATION

The following table summarizes activity under the Company s stock option plans, including the 2012 Inducement Plan and those suspended upon the adoption of the Share Incentive Plan for the year ended December 31, 2013. All option grants presented in the table had exercise prices not less than the fair value of the underlying common stock on the grant date:

	Shares	Weighted-Average Exercise Price	Weighted Average Remaining Years	Aggregate Intrinsic Value (1)
Options outstanding as of December 31, 2012	13,865,151	$ 25.69		
Granted	2,555,122	$ 66.75		
Exercised	(2,885,052)	$ 22.73		
Expired and forfeited	(377,938)	$ 34.43		
Options outstanding as of December 31, 2013	13,157,283	$ 34.06	6.7	$ 477,618
Options expected to vest at December 31, 2013	4,156,902	$ 47.23		96,260
Exercisable at December 31, 2013	8,394,774	$ 26.33	5.7	$ 369,564

(1) The aggregate intrinsic value for outstanding options is calculated as the difference between the exercise price of the underlying awards and the quoted price of the Company s common stock as of the last trading day of fiscal 2013. The aggregate intrinsic value of options outstanding and exercisable includes options with an exercise price below $70.35, the closing price of the Company s common stock on December 31, 2013.

The weighted-average fair value per option granted in the years ended December 31, 2013, 2012 and 2011 was $30.77, $37.70 and $27.89, respectively. The total intrinsic value of options exercised during the years ended December 31, 2013, 2012 and 2011 was $119.2 million, $94.6 million and $25.1 million, respectively. The aggregate intrinsic value of options exercised was determined as of the date of option exercise. Upon the exercise of the options, the Company issues new common stock from its authorized shares.

There were 13.1 million options that were in-the-money at December 31, 2013.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

Determining the Fair Value of Stock Options and Stock Purchase Rights

The fair value of each option award is estimated on the date of grant using the Black-Scholes valuation model and the assumptions noted in the tables below. The expected life of options is based on observed historical exercise patterns. Groups of employees that have similar historical exercise patterns were considered separately for valuation purposes, but none were identified that had distinctly different exercise patterns as of December 31, 2013. The expected volatility of stock options is based upon the weighted average of the historical volatility of the Company s common stock and the implied volatility of traded options on the Company s common stock for fiscal periods in which there is sufficient trading volume in options on the Company s common stock. The risk-free interest rate is based on the implied yield on a U.S. Treasury zero-coupon issue with a remaining term equal to the expected term of the option. The dividend yield reflects that the Company has not paid any cash dividends since inception and does not intend to pay any cash dividends in the foreseeable future. The assumptions used to estimate the per share fair value of stock options granted under the 2012 Inducement Plan and the 2006 Share Incentive Plan were as follows:

	Years Ended December 31,		
	2013	2012	2011
Expected volatility	44 47%	45 46%	46 50%
Dividend yield	0.0%	0.0%	0.0%
Expected life	6.6 6.8 years	6.5 years	6.3 6.4 years
Risk-free interest rate	1.0 2.4%	0.8 1.1%	1.2 2.7%

The Company recorded $37.0 million, $32.8 million and $31.7 million of compensation costs related to current period vesting of stock options for the years ended December 31, 2013, 2012 and 2011, respectively. As of December 31, 2013, the total unrecognized compensation cost related to unvested stock options was $102.1 million. These costs are expected to be recognized over a weighted average period of 2.8 years.

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

	Years Ended December 31,		
	2013	2012	2011
Expected volatility	37%	31%	32-48%
Dividend yield	0.0%	0.0%	0.0%
Expected life	6-24 months	6-24 months	6-24 months
Risk-free interest rate	0.1-0.3%	0.2-0.3%	0.1-0.6%

The Company recorded $3.6 million, $2.9 million and $2.4 million of compensation costs related to options granted under the ESPP for the years ended December 31, 2013, 2012 and 2011, respectively. As of December 31, 2013, there was $5.0 million of total unrecognized compensation cost related to unvested stock options issuable under the ESPP. These costs are expected to be recognized over a weighted average period of 1.5 years.

Restricted Stock Unit Awards with Service-Based Vesting Conditions

RSUs are generally subject to forfeiture if employment terminates prior to the release of vesting restrictions. The Company expenses the cost of the RSUs, which is determined to be the fair market value of the shares of common stock underlying the RSUs at the date of grant, ratably over the period during which the vesting restrictions lapse.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

A summary of non-vested RSU activity under the plan for the year ended December 31, 2013 as follows:

	Shares	Weighted Average Grant Date Fair Value	Weighted Average Remaining Years	Aggregate Intrinsic Value
Non-vested units as of December 31, 2012	898,949	$ 33.10		
Granted	592,001	$ 66.81		
Vested	(300,968)	$ 30.69		
Forfeited	(56,147)	$ 40.80		
Non-vested units as of December 31, 2013	1,133,835	$ 50.97	8.7	$ 79,765
Non-vested units expected to vest at December 31, 2013	1,039,520	$ 50.62		$ 73,130

The weighted-average grant date fair value per share of RSUs granted during the years ended December 31, 2013, 2012 and 2011, was $66.81, $37.81 and $27.47, respectively. The total fair value of restricted stock that vested and was released in the years ended December 31, 2013, 2012 and 2011 was $19.7 million, $7.7 million and $4.2 million, respectively.

The Company recorded $13.0 million, $7.3 million and $4.5 million of compensation costs related to RSUs with service-based vesting conditions for the years ended December 31, 2013, 2012 and 2011, respectively. As of December 31, 2013, there was $46.6 million of total unrecognized compensation cost related to unvested RSUs with service-based vesting conditions. These costs are expected to be recognized over a weighted average period of 3.0 years.

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

Pursuant to the approval of the Board the Company granted RSU awards with performance and market-based vesting conditions to certain executive officers that provide for a base award of 860,000 RSUs in total (Base RSUs) that may be adjusted to 75% to 125% depending on the performance of the Company s stock as discussed further below. A summary of non-vested Base RSU activity under the plans for the year ended December 31, 2013 is as follows:

	Base Awards	Weighted Average Grant Date Fair	Weighted Average Remaining	Aggregate Intrinsic Value

			Value		Years		
Non-vested units with performance and market vesting conditions as of December 31, 2012	875,000	$	33.83				
Granted	0						
Vested	0						
Forfeited	(15,000)	$	32.61				
Non-vested units with performance and market vesting conditions as of December 31, 2013	860,000	$	34.66		2.2	$	60,501

The number of RSUs that could potentially vest from the Base RSUs granted is contingent upon achievement of specific performance goals and will be multiplied by the Total Shareholder Return (the TSR) multiplier which could range from 75% to 125% to determine the number of earned RSUs.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

The vesting of the Base RSUs under these specific grants is contingent upon the achievement of multiple performance conditions, as follows:

Strategic Performance Goals	Percentage of Base RSUs to Vest Upon Achievement of Goal	Base Number of RSUs Granted Before TSR Multiplier
Product Goals		
Approval of VIMIZIM in the U.S. or EU prior to December 31, 2015	35%	301,000
Approval of PEG PAL or any other non-VIMIZIM product in the U.S. or EU prior to December 31, 2015	25%	215,000
Financial Goal		
Total revenues of at least $775.0 million in fiscal 2015	40%	344,000
		860,000

The number of RSUs that could potentially vest from the Base RSUs granted is contingent upon achievement of specific performance goals and will be multiplied by the TSR multiplier which could range from 75% to 125% to determine the number of earned RSUs. The TSR multiplier will be determined based on the Company s TSR percentile ranking relative to the TSR of the NASDAQ Biotechnology Index on December 31, 2015. TSR is calculated based on the 20-trading day average prices before the beginning and end of the performance period of the Company s common stock and each comparator company in the NASDAQ Biotechnology Index. The measurement period for the performance and TSR conditions is from the grant date through December 31, 2015, subject to certain change of control provisions (the Performance Period). The RSUs earned at the end of the Performance Period will vest on the filing date of the Company s Annual Report on Form 10-K for the 2015 fiscal year, subject to certain holding periods. The maximum number of RSUs that could vest if all performance conditions are achieved and a TSR multiplier of 125% is applied would be 1,075,000 RSUs.

The Company utilized a Monte Carlo simulation model to estimate the TSR multiplier and determined the grant date fair value on each of the grant dates. The assumptions used to estimate the fair value of the RSUs with performance and market vesting conditions were as follows:

	Grant Date		
	September 5, 2012	May 29, 2012	June 1, 2011
Fair value of the Company s common stock on grant date	$ 37.45	$ 39.06	$ 28.11
Expected volatility	31.73%	44.87%	47.95%

Risk-free interest rate	0.37%	0.52%	1.42%
Dividend yield	0.0%	0.0%	0.0%

The Monte Carlo simulation model also assumed correlations of returns of the stock prices of the Company s common stock and the common stock of a peer group of companies and historical stock price volatilities of the peer group of companies. The valuation model also used terms based on the length of the performance period and compound annual growth rate goals for total stockholder return based on the provisions of the award.

Stock-based compensation expense for this award will be recognized over the remaining service period beginning in the period the Company determines the strategic performance goal or goals is probable of achievement. During 2013, management concluded that regulatory approval of VIMIZIM was probable and the Company recorded $6.5 million of compensation expense related to the performance based RSUs allocated to this performance goal. The Company did not recognize compensation expense for these awards for the years

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

ended December 31, 2012 and 2011 because the Company s management had not yet determined the goals were probable of achievement. As of December 31, 2013, there was $6.3 million of total unrecognized compensation cost related to the unvested awards allocated to the VIMIZIM performance goal. These costs are expected to be recognized over a weighted average period of 2.2 years.

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

	Years Ended December 31,		
	2013	2012	2011
Cost of sales	$ 4,860	$ 4,890	$ 5,171
Research and development	27,763	20,736	16,365
Selling, general and administrative	31,753	22,346	22,283
Total stock-based compensation expense	$ 64,376	$ 47,972	$ 43,819

Stock-based compensation of $6.1 million, $4.3 million and $5.3 million was capitalized into inventory, for the years ended December 31, 2013, 2012 and 2011, respectively. Capitalized stock-based compensation is recognized as cost of sales when the related product is sold.

(18) COMPREHENSIVE INCOME

The following table summarizes amounts reclassified out of Accumulated Other Comprehensive Income/(Loss) (AOCI) and their effect on the Company s Consolidated Statements of Operations for the year ended December 31, 2013.

Details about AOCI Components	Amount Reclassified from AOCI (Gain) Loss Year Ended December 31, 2013	Consolidated Statement of Operations Classification
Gains on cash flow hedges:		
Forward foreign currency exchange contracts	$ (37)	Net product revenues
Forward foreign currency exchange contracts	(40)	Selling, general and administrative
	28	Provision for income taxes
	$ (49)	Net loss

The following table summarizes changes in the accumulated balances for each component, of other comprehensive income/(loss), including current period other comprehensive income and reclassifications out of AOCI, for the year ended December 31, 2013.

	Gains (Losses) on Cash Flow Hedges	Unrealized Gain (Losses) on Available-for-sale Securities	Foreign Currency Translation Adjustments	Total
AOCI balance, net of tax at December 31, 2012	$ (97)	$ 133	$ (238)	$ (202)
Other comprehensive income (loss) before reclassifications	(1,366)	6,275	361	5,270
Less amounts reclassified from AOCI	49	1	0	50
Net increase in other comprehensive income (loss)	(1,415)	6,274	361	5,220
AOCI balance, net of tax at December 31, 2013	$ (1,512)	$ 6,407	$ 123	$ 5,018

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

(19) REVENUE AND CREDIT CONCENTRATIONS

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

The table below summarizes net product revenue concentrations based on patient location for Naglazyme, Kuvan and Firdapse and Genzyme s headquarters for Aldurazyme. Although Genzyme sells Aldurazyme worldwide, the royalties earned by the Company on Genzyme s net sales are included in the U.S. region, as the transactions are with Genzyme whose headquarters are located in the U.S.

	For the Years Ended December 31,		
	2013	2012	2011
Region:			
United States	52%	50%	51%
Europe	22%	22%	23%
Latin America	13%	15%	13%
Rest of world	13%	13%	13%
Total net product revenue	100%	100%	100%

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

	For the Years Ended December 31,		
	2013	2012	2011
Customer A	15%	15%	17%
Customer B (1)	16%	16%	19%
Customer C	9%	12%	10%
Customer D	11%	9%	8%
Total	51%	52%	54%

(1) Genzyme is the Company s sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third-parties. Net product revenues from Genzyme are comprised of royalties on worldwide net

Aldurazyme sales and incremental product transfer revenue.

The accounts receivable balances at December 31, 2013 and 2012 were comprised of amounts due from customers for net product sales of Naglazyme, Kuvan and Firdapse and Aldurazyme product transfer and royalty revenues. On a consolidated basis, the Company s two largest customers accounted for 45% and 15% of the December 31, 2013 accounts receivable balance, respectively, compared to December 31, 2012 when the two largest customers accounted for 51% and 13% of the accounts receivable balance, respectively. As of December 31, 2013 and December 31, 2012, accounts receivable for the Company s largest customer balance included $26.3 million and $32.4 million, respectively, of unbilled accounts receivable related to net incremental Aldurazyme product transfers to Genzyme. The Company does not require collateral from its customers, but does perform periodic credit evaluations of its customers financial condition and requires immediate payment in certain circumstances.

The Company s product sales to government-owned or government-funded customers in certain European countries, including Italy, Spain, Portugal and Greece, are subject to payment terms that are statutorily

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

determined. Because these customers are government-owned or government-funded, the Company may be impacted by declines in sovereign credit ratings or sovereign defaults in these countries. A significant or further decline in sovereign credit ratings or a default in these countries may decrease the likelihood that the Company will collect accounts receivable or may increase the discount rates and the length of time until receivables are collected, which could result in a negative impact to the Company s operating results. In the year ended December 31, 2013, approximately 4% of the Company s net product revenues were from these countries. Additionally, approximately 16% of the Company s outstanding accounts receivable at December 31, 2013 related to such countries.

The following table summarizes the accounts receivable by country that were past due related to Italy, Spain, Portugal and Greece, the number of days past due and the total allowance for doubtful accounts related to each of these countries at December 31, 2013.

	Days Past Due				
	< 180 Days	180 360 Days	> 360 Days	Total Amount Past Due	Allowance for Doubtful Accounts
Italy	$ 0	$ 0	$ 0	$ 0	$ 0
Spain	2,031	1,443	2,166	5,640	0
Portugal	0	0	0	0	0
Greece	0	0	352	352	352
Total	$ 2,031	$ 1,443	$ 2,518	$ 5,992	$ 352

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

(20) INCOME TAXES

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

	Years Ended December 31,		
	2013	2012	2011
U.S. Source	$ 46,675	$ 45,422	$ 63,640

Non-U.S. Source	(223,178)	(163,700)	(107,267)
Loss before income taxes	$ (176,503)	$ (118,278)	$ (43,627)

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

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

	Years Ended December 31,		
	2013	2012	2011
Provision for current income tax expense:			
Federal	$ 5,060	$ 2,253	$ 2,766
State and local	1,496	1,879	1,439
Foreign	2,199	1,858	1,641
	$ 8,755	$ 5,990	$ 5,846
Provision for deferred income tax expense (benefit):			
Federal	$ (6,084)	$ (6,055)	$ 7,398
State and local	(2,658)	(3,891)	(2,957)
Foreign	(163)	25	(78)
	$ (8,905)	$ (9,921)	$ 4,363
Provision for (benefit from) income taxes	$ (150)	$ (3,931)	$ 10,209

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

	Years Ended December 31,		
	2013	2012	2011
Federal statutory income tax rate	35.0%	35.0%	35.0%
State and local taxes	(0.3)	(1.3)	(1.9)
Orphan Drug & General Business Credit	14.7	27.6	43.9
Stock compensation expense	(1.7)	(1.6)	(8.2)
Changes in the fair value of contingent acquisition consideration payable	(2.9)	(2.6)	1.5
Foreign tax rate differential	(45.4)	(50.0)	(86.7)
Other	1.6	(3.2)	(2.0)
Valuation allowance/Deferred benefit	(0.9)	(0.6)	(5.0)
Effective income tax rate	0.1%	3.3%	(23.4)%

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

	December 31,	
	2013	2012
Net deferred tax assets:		
Net operating loss carryforwards	$ 22,890	$ 20,431
Credit carryforwards	176,226	170,322
Property, plant and equipment	504	1,791
Accrued expenses, reserves, and prepaids	21,071	18,770
Intangible assets	8,255	6,161
Stock-based compensation	29,603	22,634
Inventory	12,417	17,074
Capital loss carryforwards	3,071	3,083
Other	799	764
Gross deferred tax assets	$ 274,836	$ 261,030
Joint venture basis difference	(1,806)	(1,801)
Acquired Intangibles	(34,091)	(31,420)
Convertible notes discount	(46,029)	0
Other comprehensive loss	(3,611)	(75)
Valuation allowance	(8,347)	(6,075)
Net deferred tax assets	$ 180,952	$ 221,659

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

As of December 31, 2013, the Company had federal net operating loss carryforwards of $29.1 million and state net operating loss carryforwards of $184.1 million. The Company also had federal research and development and orphan drug credit carryforwards of $250.4 million and state research credit carryovers of $39.9 million. The Company has elected to recognize the excess benefits related to the exercise of employee stock options under a with and without approach, which will be accounted for as an increase to additional paid-in-capital if and when realized. As of December 31, 2013, the Company had unrecognized federal and state stock option benefits of $199.6 million and $71.2 million, respectively.

The federal net operating loss carryforwards will expire at various dates beginning in 2026 through 2033 if not utilized. The federal credit carryforward will expire at various dates beginning in 2020 through 2033 if not utilized. The state net operating loss carryforwards will expire at various dates beginning in 2015 through 2033 if not utilized. Certain state research credit carryovers will begin to expire in 2017 if not utilized, with others carrying forward indefinitely. The Company also has Canadian net operating loss carryforwards of $1.8 million and research credit carryovers of $0.6 million that it currently does not expect to fully utilize and therefore the Company carries a full valuation allowance on all but $0.2 million of the research credit carryforward. The Canadian net operating loss carryforwards and research credit carryovers will expire from 2014 to 2027 and from 2018 to 2022, respectively.

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

In 2013, the valuation allowance increased by $2.3 million primarily due to state net operating losses and credits that are not more likely than not to be realized. In 2012, the valuation allowance increased by $0.6 million primarily due to investment impairments that are not more likely than not to be realized.

The financial statement recognition of the benefit for a tax position is dependent upon the benefit being more likely than not to be sustainable upon audit by the applicable taxing authority. If this threshold is met, the tax benefit is then measured and recognized at the largest amount that is greater than 50% likely of being realized upon ultimate settlement. A reconciliation of the beginning and ending amount of unrecognized tax benefits for the years ended December 31, 2013 is as follows:

	December 31,	
	2013	2012
Balance at beginning of period	$ 43,531	$ 36,350
Additions based on tax positions related to the current year	7,478	7,190
Additions for tax positions of prior years	(194)	(9)
Balance at end of period	$ 50,815	$ 43,531

Included in the balance of unrecognized tax benefits at December 31, 2013 are potential benefits of $50.8 million that, if recognized, would affect the effective tax rate. The Company s policy for classifying interest and penalties associated with unrecognized income tax benefits is to include such items in the income tax expense. No interest or penalties have been recorded by the Company to date through December 31, 2013.

The Company files income tax returns in the U.S. federal jurisdiction and various states and foreign jurisdictions. For income tax returns filed before 2010, the Company is no longer subject to audit by the U.S.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

federal, state, local or non-U.S. tax authorities. However, carryforward tax attributes that were generated prior to 2010 may still be adjusted upon examination by tax authorities. Currently, the Company has an open tax return audit with the state of California for tax years 2010 and 2011.

U.S. income and foreign withholding taxes have not been recognized on the excess of the amount for financial reporting over the tax basis of investments in foreign subsidiaries that are essentially permanent in duration. This excess totaled approximately $4.7 million as of December 31, 2013, which will be indefinitely reinvested; therefore, deferred income taxes of approximately $1.7 million have not been provided on such foreign earnings.

(21) COLLABORATIVE AGREEMENTS

Merck Serono

In May 2005, the Company entered into an agreement with Merck Serono for the further development and commercialization of BH4, both in Kuvan for PKU and for other indications, and PEG PAL (phenylalanine ammonia lyase). Through the agreement and subsequent amendment, Merck Serono acquired exclusive rights to market these products in all territories outside the U.S., Canada and Japan, and BioMarin retained exclusive rights to market these products in the U.S. and Canada. The Company and Merck Serono may collaborate on the development of Kuvan and PEG PAL. If they agree to collaborate Merck Serono will generally share equally all development costs following successful completion of Phase 2 trials for such product candidate in such indication. Merck Serono has opted-out of the PEG PAL development program, a decision that does not affect its exclusive rights to PEG PAL in its territory. Unless or until Merck Serono elects to opt-in, it is not obligated to pay any of the milestones related to the program or to reimburse the Company for any of the PEG PAL development costs. Merck Serono may elect to opt in at any time. If it elects to opt in prior to the unblinding of the first Phase 3 trial, it must pay 75% of the Phase 3 costs incurred prior to opting in and a $7.0 million development milestone if the Phase 3 trial has started. If Merck Serono opts in after the unblinding of the first Phase 3 trial for PEG PAL, it must pay 100% of the Phase 3 costs incurred prior to opting in and a $7.0 million development milestone.

BioMarin and Merck Serono are individually responsible for the costs of commercializing the products within their respective territories. Merck Serono will also pay BioMarin royalties on its net sales of these products. The term of the agreement is the later of 10 years after the first commercial sale of the products or the period through the expiration of all related patents within the territories. As of December 31, 2013 and 2012, amounts due from Merck Serono for reimbursable development costs for Kuvan totaled $0.3 million and $0.4 million, respectively.

Other Agreements

The Company is engaged in research and development collaborations with various other entities. These provide for sponsorship of research and development by the Company and may also provide for exclusive royalty-bearing intellectual property licenses or rights of first negotiation regarding licenses to intellectual property development under the collaborations. Typically, these agreements can be terminated for cause by either party upon 90 days written notice.

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

In October 2012, the Company licensed to Catalyst Pharmaceutical Partners, Inc., (Catalyst) the North American rights to develop and market Firdapse. In consideration of this licensing arrangement, the Company

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

received from Catalyst a $5.0 million convertible promissory note. Under the terms of the note agreement, the Company received 6.7 million shares of Catalyst common stock upon the automatic conversion of the convertible promissory note on December 10, 2012. The conversion price was based on $0.75 per share, which resulted in a $2.0 million loss on conversion, which was included as a component of Other Income (Expense) on the Company s Consolidated Statement of Operations for the year ended December 31, 2012. In exchange for the North American rights to Firdapse the Company may receive royalties of 7% to 10% on net product sales of Firdapse in North America. As of December 31, 2013 and 2012, amounts due from Catalyst for reimbursable development costs totaled $0.8 million and $43, respectively.

In May 2013, the Company entered into a non-exclusive royalty bearing license with Shire Human Genetic Therapies, Inc, (Shire). Under the terms of the agreement, Shire was granted the right to use patents related to the intrathecal delivery of lysosomal enzymes that are within the Company s control. In consideration of this licensing agreement, the Company received a $3.0 million non-refundable upfront payment, future milestone payments of up to $18.0 million if certain development and commercial milestones are attained by Shire and royalties ranging from 3% to 5% on Shire net sales of the product. The milestone payments to be made by Shire are based solely upon Shire s performance; therefore the Company expects to recognize the payments as revenue upon receipt, provided that the other revenue recognition criteria have been satisfied.

Other Commitments

In the normal course of business, the Company enters into various firm purchase commitments primarily related to active pharmaceutical ingredients and certain inventory related items. As of December 31, 2013, these commitments for the next five years were approximately $38.2 million in 2013. The amounts primarily related to active pharmaceutical ingredients represent minimum purchase requirements and post marketing commitments related to the Company s approved products.

(22) COMPENSATION AGREEMENTS AND PLANS

Employment Agreements

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

401(k) Plan

The Company sponsors the BioMarin Retirement Savings Plan (the 401(k) Plan). Most employees (Participants) are eligible to participate following the start of their employment, at the beginning of each calendar month. Participants may contribute to the 401(k) Plan up to the lesser of 100% of their current compensation or an amount up to a statutorily prescribed annual limit. The Company pays the direct expenses of the 401(k) Plan and matched 100% of each Participant s contributions, up to a maximum of the lesser of 2% of the employee s annual compensation or $4,000 per year through December 31, 2013. In 2014, the Company s 401(k) match was increased to the lesser of 3% of the

employee s annual compensation or $6,000 per year. The Company s matching contribution vests over four years from employment commencement and was approximately $3.4 million, $2.8 million and $2.2 million for the years ended December 31, 2013, 2012 and 2011, respectively. Employer contributions not vested upon employee termination are forfeited.

Deferred Compensation Plan

In December 2005, the Company adopted the Deferred Compensation Plan. The Deferred Compensation Plan allows eligible employees, including members of the Board, management and certain highly-compensated

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

employees as designated by the Deferred Compensation Plan s Administrative Committee, the opportunity to make voluntary deferrals of compensation to specified future dates, retirement or death. Participants are permitted to defer portions of their salary, annual cash bonus and restricted stock. The Company may not make additional direct contributions to the Deferred Compensation Plan on behalf of the participants, without further action by the Board. Deferred compensation is held in trust and generally invested to match the investment benchmarks selected by participants. The recorded cost of any investments will approximate fair value. Company stock issued into the Deferred Compensation Plan is recorded and accounted for similarly to treasury stock in that the value of the employer stock is determined on the date the restricted stock vests and the shares are issued into the Deferred Compensation Plan. The restricted stock issued into the Deferred Compensation Plan upon vesting is recorded in stockholders equity. As of December 31, 2013 and 2012, the fair value of Company stock held by the Deferred Compensation Plan was $13.6 million and $11.5 million, respectively. The change in market value amounted to a loss of approximately $4.2 million in 2013, compared to losses of $3.2 million and $1.3 million in 2012 and 2011, respectively. See Note 15 to these Consolidated Financial Statements for additional discussion regarding the fair value of the Deferred Compensation Plan assets and liabilities.

(23) JOINT VENTURE

Effective January 2008, the Company and Genzyme restructured BioMarin/Genzyme LLC. Under the revised structure, the operational responsibilities for the Company and Genzyme did not significantly change, as Genzyme continues to globally market and sell Aldurazyme and the Company continues to manufacture Aldurazyme.

Genzyme records sales of Aldurazyme to third-party customers and pays the Company a tiered payment ranging from approximately 39.5% to 50% of worldwide net product sales depending on sales volume, which is recorded by the Company as product revenue. The Company recognizes a portion of this amount as product transfer revenue when the product is released to Genzyme because all of the Company s performance obligations are fulfilled at this 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 is deducted from the calculated royalty rate when the product is sold by Genzyme. Genzyme s contractual return rights for Aldurazyme are limited to defective product. Certain research and development activities and intellectual property related to Aldurazyme continue to be managed in the joint venture with the costs shared equally by the Company and Genzyme.

The Company presents the related cost of sales and its Aldurazyme-related operating expenses as operating expenses in the Company s Consolidated Statements of Operations. Equity in the loss of BioMarin/Genzyme LLC subsequent to the restructuring includes BioMarin s 50% share of the net income (loss) of BioMarin/Genzyme LLC related to intellectual property management and ongoing research and development activities.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

The results of the joint venture s operations are presented in the table below.

	Years Ended December 31,		
	2013 (unaudited)	2012 (unaudited)	2011 (unaudited)
Revenue	$ 0	$ 0	$ 0
Cost of goods sold	0	0	0
Gross profit	0	0	0
Operating expenses	2,221	2,534	4,855
Loss from operations	(2,221)	(2,534)	(4,855)
Other income	3	4	5
Net loss	$ (2,218)	$ (2,530)	$ (4,850)
Equity in the loss of BioMarin/Genzyme LLC	$ (1,149)	$ (1,221)	$ (2,426)

The summarized assets and liabilities of the joint venture and the components of the Company s investment in the joint venture are as follows:

	December 31,	
	2013 (unaudited)	2012 (unaudited)
Assets	$ 1,770	$ 3,343
Liabilities	(136)	(1,747)
Net equity	$ 1,634	$ 1,596
Investment in BioMarin/Genzyme LLC (50% share of net equity)	$ 816	$ 1,080

(24) COMMITMENTS AND CONTINGENCIES

Lease Commitments

The Company leases office space and research, testing and manufacturing laboratory space in various facilities under operating agreements expiring at various dates through 2022. Certain of the leases provide for options by the Company to extend the lease for multiple five-year renewal periods and also provide for annual minimum increases in

rent, usually based on a consumer price index or annual minimum increases. Minimum lease payments for future years are as follows:

2014	$ 10,897
2015	10,059
2016	8,907
2017	8,343
2018	8,045
Thereafter	20,280
Total	$ 66,531

At December 31, 2013, the Company s annual minimum lease obligations included $35.9 million related to its leases for SRCC which will be terminated upon closing of the purchase of SRCC during the first quarter of 2014.

Table of Contents

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

Rent expense for the years ended December 31, 2013, 2012 and 2011 was $10.4 million, $10.1 million, and $6.0 million, respectively. Deferred rent accruals at December 31, 2013 totaled $9.9 million, of which $0.9 million was current. The December 31, 2013 deferred rent accruals include $8.8 million related to SRCC which will be released upon the completion of the purchase of SRCC. Deferred rent accruals at December 31, 2012 totaled $10.0 million, of which $1.0 million was current.

See Note 11 to these Consolidated Financial Statements for additional discussion regarding the purchase of SRCC.

Research and Development Funding and Technology Licenses

The Company uses experts and laboratories at universities and other institutions to perform certain research and development activities. These amounts are included as research and development expenses as services are provided.

The Company has also licensed technology, for which it is required to pay royalties upon future sales, subject to certain annual minimums. As of December 31, 2013, such minimum annual commitments were approximately $1.2 million.

Contingencies

From time to time the Company is involved in legal actions arising in the normal course of its business. The Company is not presently subject to any material litigation nor, to management s knowledge, is any litigation threatened against the Company that collectively is expected to have a material adverse effect on the Company s consolidated cash flows, financial condition or results of operations.

As of December 31, 2013 the Company is also subject to contingent payments totaling approximately $422.2 million upon achievement of certain regulatory and licensing milestones if they occur before certain dates in the future. Of this amount, $56.4 million relates to programs that are no longer being developed.

As of December 31, 2013, the Company has recorded $42.7 million of contingent acquisition consideration payable on its Consolidated Balance Sheet, of which $11.9 million current.

(25) SUBSEQUENT EVENTS

On February 14, 2014, the FDA granted marketing approval for VIMIZIM for the treatment of mucopolysaccharidosis Type IV A (Morquio Syndrome Type A or MPS IV A). The Company immediately began marketing VIMIZIM in the U.S. using its existing sales force and commercial organization and completed the first commercial sale in the U.S.

On February 20, 2014 the Committee for CHMP of the EMA adopted a positive opinion for the Company s MAA for VIMIZIM for the treatment of MPS IV A. The CHMP s recommendation is now referred to the European Commission (EC). The EC is expected to render a final decision for VIMIZIM in the second quarter of 2014.