BIOMARIN PHARMACEUTICAL INC
Form 10-K
February 26, 2013
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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, 2012

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	**(I.R.S. Employer**

incorporation or organization)	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: 126,101,610 shares common stock, par value $0.001, outstanding as of February 15, 2013. The aggregate market value of the voting and non-voting stock held by non-affiliates of the registrant as of June 30, 2012 was $2,740.8 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 May 15, 2013, are incorporated by reference into Part III.

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BIOMARIN PHARMACEUTICAL INC.

2012 FORM 10-K ANNUAL REPORT

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Part I

FORWARD LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements as defined under securities laws. Many of these statements can be identified by the use of terminology such as believes, expects, anticipates, plans, may, will, projects, continues, estimates, pote and similar expressions. These forward-looking statements may be found in *Risk Factors*, *Business*, and other sections of this Annual Report on Form 10-K. Our actual results or experience could differ significantly from the forward-looking statements. Factors that could cause or contribute to these differences include those discussed in *Risk Factors*, as well as those discussed elsewhere in this Annual Report on Form 10-K. You should carefully consider that information before you make an investment decision.

You should not place undue reliance on these statements, which speak only as of the date that they were made. These cautionary statements should be considered in connection with any written or oral forward-looking statements that we may issue in the future. We do not undertake any obligation to release publicly any revisions to these forward-looking statements after completion of the filing of this Annual Report on Form 10-K to reflect later events or circumstances or to reflect the occurrence of unanticipated events.

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

Item 1. Business

Overview

BioMarin Pharmaceutical Inc. (BioMarin, we, us or our) develops and commercializes innovative pharmaceuticals for serious diseases and medical conditions. We select product candidates for diseases and conditions that represent a significant unmet medical need, have well-understood biology and provide an opportunity to be first-to-market or offer a significant benefit over existing products. Our product portfolio is comprised of four approved products and multiple investigational product candidates. Approved products include Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (laronidase) and Firdapse (amifampridine phosphate).

Naglazyme received marketing approval in the United States (U.S.) in May 2005, in the European Union (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 (EMEA) 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., EU and subsequently other countries. Net product revenues during 2012 for our approved products, Naglazyme, Kuvan, Firdapse and Aldurazyme were $257.0 million, $143.1 million, $14.2 million and $82.2 million, respectively.

We are conducting clinical trials on several investigational product candidates for the treatment of various diseases including: Vimizim (formerly referred to as GALNS), an enzyme replacement therapy for the treatment of Mucopolysaccharidosis Type IV or Morquio Syndrome Type A, or MPS IV A, 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, or PARP inhibitor for the treatment of patients with certain cancers and BMN-111, a peptide therapeutic for the treatment of achondroplasia.

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We are conducting or planning to conduct preclinical development of several other enzyme product candidates for genetic and other metabolic diseases, including BMN-190 for the treatment of late infantile neuronal ceroid lipofuscinosis, or LINCL, a form of Batten disease. We expect to initiate a Phase 1 clinical trial of BMN-190 in the first half of 2013.

A summary of our various commercial products and major development programs, including key metrics as of December 31, 2012, is provided below:

Program	Indication	Orphan Drug Designation	Stage	2012 Total Net Product Revenues (in millions)	2012 Research & Development Expense (in millions)
Naglazyme	MPS VI (1)	Yes	Approved	$ 257.0	$ 12.4
Aldurazyme (2)	MPS I (3)	Yes	Approved	$ 82.2	$ 1.3
Kuvan	PKU (4)	Yes	Approved	$ 143.1	$ 14.1
Firdapse (5)	LEMS (6)	Yes	Approved in the EU only	$ 14.2	$ 5.4
Vimizim for MPS IV A	MPS IVA(7)	Yes	Clinical Phase 3	N/A	$ 97.0
PEG-PAL	PKU	Yes	Clinical Phase 2	N/A	$ 26.7
BMN-701 for Pompe disease	POMPE (8)	Yes	Clinical Phase 1/2	N/A	$ 31.6
BMN-673, PARP inhibitor for the treatment of patients with cancer	Not yet determined	Not yet determined	Clinical Phase 1/2	N/A	$ 9.7
BMN-673, PARP inhibitor for the treatment of patients with hematological malignancies	Not yet determined	Not yet determined	Clinical Phase 1/2	N/A	$ 1.7
BMN-111, peptide therapeutic for the treatment of Achondroplasia	Achondroplasia	Yes	Clinical Phase 1	N/A	$ 12.1

(1) Mucopolysaccharidosis VI, or MPS VI
(2) 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.
(3) Mucopolysaccharidosis I, or MPS I
(4) Phenylketonuria, or PKU
(5) Marketing approval from the EMEA for Firdapse was granted in December 2009. We launched Firdapse in the EU in April 2010.
(6) Lambert Eaton Myasthenic Syndrome, or LEMS
(7) Morquio A Syndrome, or MPS IVA
(8) Pompe disease, a glycogen storage disorder

Commercial Products

Naglazyme

Naglazyme is a recombinant form of N-acetylgalactosamine 4-sulfatase (arylsulfatase B) indicated for patients with mucopolysaccharidosis VI, or 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, or 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 Russia 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 sales for 2012 totaled $257.0 million, as compared to $224.9 million for 2011. Naglazyme net product sales for 2010 were $192.7 million.

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Kuvan

Kuvan is a proprietary synthetic oral form of 6R-BH4, a naturally occurring enzyme co-factor for phenylalanine hydroxylase, or 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, or 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.

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 2014. We expect that our patents will provide market exclusivity beyond the expiration of orphan status. Kuvan net product sales for 2012 were $143.1 million, as compared to $116.8 million for 2011. Kuvan net product sales for 2010 were $99.4 million.

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 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. 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 right 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. In 2012, we earned $1.9 million in net royalties on net sales of $46.8 million of Kuvan by Merck Serono, compared to 2011 when we earned $1.6 million in net royalties on net sales of $40.4 million. In 2010, we earned $0.9 million in net royalties on net sales of $23.7 million. We recorded collaborative agreement revenue associated with shared Kuvan development costs in the amounts of $2.0 million in 2012, $0.5 million in 2011 and $0.7 million in 2010.

On February 19, 2013, we announced results from our PKU-016 ASCEND study, a randomized controlled trial evaluating neuropsychiatric outcomes in PKU patients treated with Kuvan. The study evaluated medically important symptoms similar to attention deficit hyperactivity disorder (ADHD) in PKU patients whose blood levels of Phe are reduced by Kuvan. The primary endpoint of the study was evaluated using an attention deficit hyperactivity rating scale (ADHD-RS), commonly used to evaluate symptoms of inattentiveness and hyperactivity. Kuvan improved the ADHD-RS ($p=0.085$), driven by a statistically significant change in the inattention component of the score ($p=0.036$).

Aldurazyme

Aldurazyme has been approved for marketing in the U.S., EU and other countries for patients with mucopolysaccharidosis I, or 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

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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. 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.

Our Aldurazyme net product revenues totaled $82.2 million for 2012 as compared to $82.8 million for 2011 and $71.2 million for 2010. The net product revenues for 2012, 2011 and 2010 include $80.4 million, $74.2 million and $68.0 million, respectively, of royalty revenue on net Aldurazyme sales by Genzyme. Net sales of Aldurazyme by Genzyme totaled $193.1 million for 2012, $185.2 million for 2011 and $166.8 million for 2010. Incremental Aldurazyme net product transfer revenue of $1.8 million, $8.6 million, and $3.2 million for 2012, 2011 and 2010, respectively, reflect incremental shipments of Aldurazyme to Genzyme to meet future product demand. In the future, to the extent that Genzyme Aldurazyme inventory quantities on hand remain consistent, we expect that our total Aldurazyme revenues will approximate the 39.5% to 50% royalties on net product sales by Genzyme.

Firdapse

Firdapse is a form of 3, 4-diaminopyridine (amifampridine phosphate), or 3, 4-DAP for the treatment of LEMS. Firdapse was originally developed by AGEPS, the pharmaceutical unit of the Paris Public Hospital Authority, or 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 in 2012 were $14.2 million, compared to $13.1 million and $6.4 million in 2011 and 2010, 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.

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

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consist of strategies directed at the underlying malignancy, if one is present. Therapy of small cell lung cancer is limited and outcomes are generally poor. Immunosuppressive agents have been tried but success is limited by toxicity and difficulty administering the regimens. A mainstay of therapy has been 3, 4-DAP, but its use in practice has been limited by the drug s availability.

Products in Clinical Development

We are developing Vimizim, an enzyme replacement therapy for the treatment of MPS IV A, a lysosomal storage disorder. In November 2012, we announced the top-line results of a pivotal Phase 3 clinical trial for Vimizim for the treatment of MPS IV A. The top-line results demonstrated that the study met the primary endpoint of change in six-minute walk distance compared with a placebo at 24 weeks in subjects receiving weekly infusions of Vimizim at the dose of two milligrams per kilogram per week. This Phase 3 trial was a randomized, double-blind, placebo-controlled study designed to evaluate the efficacy and safety of Vimizim in patients with MPS IV A. The trial was conducted at 31 centers worldwide including Brazil, Japan, Taiwan, most Western European countries, Canada and the U.S. We enrolled 176 patients in this trial. The trial explored doses of two milligrams per kilogram per week and two milligrams per kilogram every other week for a treatment period of 24 weeks.

In addition, in November 2011, we announced the initiation of a Phase 2 study for Vimizim in patients with MPS IVA who are under five years of age. The primary objective of the Phase 2, open-label, multinational clinical study is to evaluate the safety and tolerability of infusions of Vimizim at a dose of 2.0 milligrams per kilogram per week over a 52-week period in 10 to 15 patients with MPS IVA who are under five years of age. The secondary objectives are to evaluate urinary keratin sulfate levels and growth velocity. This study is ongoing.

PEG-PAL is an investigational enzyme substitution therapy that we are developing as a subcutaneous injection for the treatment of PKU. In preclinical models, PEG-PAL produced a rapid, dose-dependent reduction in blood phenylalanine, or Phe levels, the same endpoint that was used in the Kuvan studies. 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 is to optimize the dose and schedule that produces the most favorable safety profile and Phe reduction. The secondary objectives of the clinical trial are 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 the quickest and safest induction dosing regimen to an efficacious maintenance dose. This study is ongoing. We expect to initiate a Phase 3 clinical trial of PEG-PAL in the second quarter of 2013.

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. The 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 primary objective of the study is to establish the maximum tolerated dose of daily oral BMN-673. The secondary objective of the study is to establish the safety, pharmacokinetic profile and recommended Phase 2 dose. The study, to date, has established a preliminary dose that is generally well-tolerated and reaches steady state with repeated daily doses, and we are currently expanding the number of patients treated at that dose. The expansion phase of the study will focus on cancers characterized by BRCA mutations, Ewing s sarcoma and small cell lung cancer.

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In July 2011, we initiated a Phase 1/2 clinical trial for BMN-673 for the treatment of patients with advanced hematological malignancies. This clinical trial is a two-arm, open-label dose escalation study to determine the maximum tolerated dose and to assess the safety, pharmacokinetics, pharmacodynamics and preliminary efficacy of once daily, orally administered BMN-673 in patients with acute myeloid leukemia, myelodsplastic syndrome, chronic lymphocytic leukemia or mantle cell lymphoma. This study will enroll approximately 80 patients. Currently, the study is in a dose-escalation phase.

BMN-701 is a novel fusion of insulin-like growth factor 2 and alpha glucosidase (IGF2-GAA) in development for Pompe disease. 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 is 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, which 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. We expect to report top-line results from this study in the first quarter of 2013.

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. We expect to start the Phase 2 study in pediatric patients in mid-2013.

Manufacturing

We manufacture Naglazyme, Aldurazyme, Vimizim, PEG-PAL and BMN-111 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 clinical requirements and initial launch of Vimizim, if approved.

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. The addition of the Shanbally facility will increase our operating capacity to support the commercial demand of Vimizim, if approved.

Our Novato, California facilities have been licensed by the Food and Drug Administration (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 be GMP certified before we can manufacture our drugs for commercial sales.

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Kuvan is 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 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 small salesforce 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 the size of our sales force is appropriate to effectively reach our target audience in markets where Naglazyme, Kuvan and Firdapse are directly marketed. We utilize third-party logistics companies to store and distribute our products.

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

Customers

Our 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 2012, 43% 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.

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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 or 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, or 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 however, has yet been approved by the FDA or any other regulatory agency.

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

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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.

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 213, including approximately 62 patents issued by the U.S. Patent and Trademark Office (USPTO). Furthermore, our portfolio of pending patent applications totals approximately 354 applications, including approximately 58 pending U.S. applications.

With respect to Naglazyme, we have 17 issued patents, including three U.S. patents. Claims cover our ultrapure *N* -acetylgalactosamine-4-sulfatase compositions of Naglazyme, methods of treating deficiencies of *N* -acetylgalactosamine-4-sulfatase, including MPS VI, methods of producing and purifying such ultrapure *N* -acetylgalactosamine-4-sulfatase compositions, and methods of detecting lysosomal enzyme-specific antibodies. These patents will expire between 2022 (compositions of matter, methods of use) and 2028 (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 31 issued patents including 12 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 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 and we have no issued patents in the U.S. for Firdapse for the treatment of LEMS.

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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.

Government Regulation

We operate in a highly regulated industry, which 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 including the Federal Food, Drug and Cosmetic Act, or FDC Act, the Public Health Service Act, the Medicaid rebate program, the Veterans Health Care Act of 1992, and the Occupational Safety and Health Act, among others.

The FDC Act and other federal and state statutes and regulations govern, among other things, the testing, research, development, manufacture, safety, effectiveness, labeling, storage, record keeping, approval, advertising and promotion, import and export of our products. As a result of these laws and regulations, product development and product approval processes are very expensive and time consuming.

FDA Approval Process

Pharmaceutical product development in the U.S. typically involves preclinical laboratory and animal tests, the submission to the FDA of an investigational new drug application, or IND, 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 FDA approval is sought. Satisfaction of FDA pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity and novelty of the product or disease.

Preclinical tests include laboratory evaluation, as well as animal trials, to assess the characteristics and potential pharmacology and toxicity of the product. The conduct of the preclinical tests must comply with federal regulations and requirements, including good laboratory practices. The results of preclinical testing are submitted to the FDA as part of an IND along with other information, including information about product chemistry, manufacturing and controls and a proposed clinical trial protocol. Long term preclinical tests, such as animal tests of reproductive toxicity and carcinogenicity, may continue after the IND is submitted.

A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If the FDA has not objected to the IND within this 30-day period, the clinical trial proposed in the IND may begin.

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 federal regulations, good clinical practices, or GCP, as well as under protocols detailing the objectives of the trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol involving testing on U.S. patients and subsequent protocol amendments must be submitted to the FDA as part of the IND.

The FDA may order the temporary or permanent discontinuation of a clinical trial at any time or impose other sanctions if it believes that the clinical trial is not being conducted in accordance with FDA 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, or IRB, for approval. An IRB may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB s requirements, or may impose other conditions.

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Clinical trials to support new drug applications, or NDAs, or biological product licenses, or BLAs, for marketing approval are typically conducted in three sequential phases, but the phases may overlap. 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. FDA approval of the NDA or BLA is required before marketing of the product may begin in the U.S. The NDA or BLA 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, proposed labeling and a payment of a significant user fee (currently exceeding $1,958,000), among other things. The manufacturer and/or sponsor under an approved NDA or BLA are also subject to annual product and establishment user fees, currently exceeding $98,000 per product and $526,000 per establishment. These fees are typically increased by the FDA annually.

The FDA has 60 days from its receipt of an NDA or BLA to determine whether the application will be accepted for filing based on the agency s threshold determination that it is sufficiently complete to permit substantive review. The FDA may request additional information rather than accepting an NDA or BLA for filing. Once the submission is accepted for filing, the FDA begins an in-depth review. The FDA has agreed to certain performance goals in the review of NDAs or BLAs. Most such applications for non-priority drug products are reviewed within ten to twelve months. The goal for initial review of most applications for priority review of drugs, that is, drugs that the FDA determines represent a significant improvement over existing therapy, is six months to eight months. The review process may be extended by the FDA for three additional months to consider new information submitted during the review or clarification regarding information already provided in the submission. The FDA may also refer applications for novel products or products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations. Before approving an NDA or BLA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP. Additionally, the FDA will inspect the facility or the facilities at which the drug is manufactured. The FDA will not approve the product unless compliance with current good manufacturing practices, or cGMPs, is satisfactory and the NDA or BLA contains data that provide substantial evidence that the drug is safe and effective in the indication studied.

After the FDA evaluates the NDA or BLA, including the manufacturing procedures and facilities, 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.

An approval letter 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, or 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, or 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

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requirement for REMS can materially affect the potential market and profitability of the drug. Moreover, product approval may require substantial post-approval testing and surveillance to monitor the drug s safety or efficacy. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

Disclosure of Clinical Trial Information

Sponsors of clinical trials of FDA-regulated products, including drugs and biologics, are required to register and disclose certain clinical trial information. Information related to the product, patient population, phase of investigation, study sites and investigators, and other aspects of the clinical trial are then made public as part of the registration. Sponsors are also obligated to discuss the results of their clinical trials after completion. Disclosure of the results of these trials can be delayed until the new product or new indication being studied has been approved. Competitors may use this publicly-available information to gain knowledge regarding the progress of development programs.

The 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 FDA s 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

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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.

Section 505(b)(2) New Drug Applications

Most drug products (other than biological products) obtain FDA marketing approval pursuant to an NDA or an ANDA. A third alternative is a special type of NDA, commonly referred to as a Section 505(b)(2) NDA, which enables the applicant to rely, in part, on the FDA s finding of safety and efficacy data for an existing product, or published literature, in support of its application.

Section 505(b)(2) NDAs often provide an alternate path to FDA approval for new or improved formulations or new uses of previously approved products. Section 505(b)(2) permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference. The applicant may rely upon certain preclinical or clinical studies conducted for an approved product. The FDA may also require companies to perform additional studies or measurements to support the change from the approved product. The FDA may then approve the new product candidate for all or some of the labeled indications for which the referenced product has been approved, as well as for any new indication for which the Section 505(b)(2) NDA applicant has submitted data.

To the extent that the Section 505(b)(2) applicant is relying on prior FDA findings of safety and efficacy, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would. Thus, approval of a Section 505(b)(2) NDA can be delayed until all the listed patents claiming the referenced product have expired, 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, and, in the case of a Paragraph IV certification and subsequent patent infringement suit, until the earlier of 30 months, settlement of the lawsuit or a decision in the infringement case that is favorable to the Section 505(b)(2) NDA applicant.

Orphan Drug Designation

Naglazyme, Aldurazyme, Kuvan and Firdapse have received orphan drug designations from the FDA. Orphan drug designation is granted by the FDA to drugs intended to treat a rare disease or condition, which for this program is defined as having a prevalence of less than 200,000 individuals in the U.S. Orphan drug designation must be requested before submitting a marketing application. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. 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.

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. 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.

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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, or 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.

Accelerated Approval

Under the FDA s accelerated approval regulations, the FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit. In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, will allow the FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by the FDA.

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 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.

Priority Review

Under the FDA policies, a drug candidate is eligible for priority review, or review within a six to eight month time frame from the time a complete NDA is submitted, if the drug candidate provides a significant improvement compared to marketed drugs in the treatment, diagnosis or prevention of a disease. A fast track designated drug candidate would ordinarily meet the FDA s criteria for priority review. For biologics, priority

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review is further limited only for drugs intended to treat a serious or life-threatening disease relative to the currently approved products.

Post-Approval Regulatory Requirements

Following FDA approval, a product is subject to certain post-approval requirements. 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 FDA before the change can be implemented. An NDA or BLA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA or BLA supplements as it does in reviewing NDAs and BLAs.

Adverse event reporting and submission of periodic reports is required following FDA approval of an NDA or BLA. The FDA also may 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 required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA 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 (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 (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

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and no application for a biosimilar can be submitted for four years from the date of licensure of the reference product. The first biologic product submitted under the abbreviated approval pathway that is determined to be interchangeable with the reference product has exclusivity against a finding of interchangeability for other biologics for the same condition of use for the lesser of (i) one year after first commercial marketing of the first interchangeable biosimilar, (ii) eighteen months after the first interchangeable biosimilar is approved if there is not patent challenge, (iii) eighteen months after resolution of a lawsuit over the patents of the reference biologic in favor of the first interchangeable biosimilar applicant, or (iv) 42 months after the first interchangeable biosimilar s application has been approved if a patent lawsuit is ongoing within the 42-month period.

The PPACA also imposes a new fee on certain manufacturers and importers of branded prescription drugs (excluding orphan drugs under certain conditions). The annual fee will be apportioned among the participating companies based on each company s sales of qualifying products to, or use by, certain U.S. government programs during the preceding year. Other provisions of the new law, which have varying effective dates, may also affect us and will likely increase certain of our costs. For example, the Medicaid rebate rate was increased and the volume of rebated drugs has been expanded to include beneficiaries in Medicaid managed care organizations. Among other things, the PPACA also expands the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance and includes a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or donut hole . The law also revised the definition of average manufacturer price for reporting purposes, which could increase the amount of the Medicaid drug rebates paid to states. Substantial new provisions affecting compliance also have been added, which may require us to modify our business practices with health care practitioners.

In addition, drug manufacturers will be 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. Although the statute requires reporting by March 31, 2013 of payments and other transfers of value made in calendar year 2012, the Centers for Medicare & Medicaid Services (CMS), has issued a final rule that will go into effect in April 2013 and will require manufacturers to begin collecting required information on August 1, 2013, with the first reports due March 31, 2014. Further, the PPACA 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 these statutes or specific intent to violate them. In addition, 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.

Other Regulatory Requirements

In addition to FDA restrictions on marketing of pharmaceutical products, several other types of state and federal laws have been applied to restrict certain marketing practices in the pharmaceutical industry in recent years. These laws include anti-kickback statutes and false claims statutes. The federal healthcare program anti-kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. 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.

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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. 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.

Regulation in the European Union

Drugs are also subject to extensive regulation outside of the U.S. In the EU, for example, there is a centralized approval procedure that authorizes marketing of a product in all countries of the EU (which includes most major countries in Europe). If this procedure is not used, approval in one country of the EU can be used to obtain approval in another country of the EU under two simplified application processes, the mutual recognition procedure or the decentralized procedure, both of which rely on the principle of mutual recognition. After receiving regulatory approval through any of the European registration procedures, pricing and reimbursement approvals are also required in most countries.

A similar system for orphan drug designation exists in the EU. Naglazyme, Aldurazyme and Kuvan received orphan medicinal product designation by the European Committee for Orphan Medicinal Products. Orphan designation does not shorten the regulatory review and approval process for an orphan drug, nor does it give that drug 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 ten years in the EU.

Anti-Corruption Legislation

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

Employees

As of January 4, 2013, we had 1,089 full-time employees, 457 of whom are in operations, 315 of whom are in research and development, 149 of whom are in sales and marketing and 168 of whom are in administration.

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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 2012, 2011 and 2010, see Item 7, *Management Discussion and Analysis of Financial Condition and Results of Operations Research and Development Expense* .

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. There are no segment managers who are held accountable by the chief operating decision maker, or anyone else, for operations, operating results and planning for levels or components below the consolidated unit level. 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. as our transactions are with Genzyme.

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

	Years Ended December 31,		
	2012	2011	2010
Net product revenues:			
United States	$ 249,745	$ 224,630	$ 196,979
Europe	108,138	100,348	90,321
Latin America	74,390	56,950	41,581
Rest of the World	64,224	55,719	40,820
Total net product revenues	$ 496,497	$ 437,647	$ 369,701

Total revenue generated outside the U.S. was $251.0 million, $217.1 million and $173.9 million, in the years ended December 31, 2012, 2011 and 2010, respectively.

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

	Years Ended December 31,	
	2012	2011
Non-monetary long-lived assets:		
United States	$ 649,172	$ 652,207
International	80,067	80,459
Total long-lived assets	$ 729,252	$ 732,666

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

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

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(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 and statements filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act, are available free of charge at *www.bmrn.com* as soon as reasonably practicable after we electronically file such reports with the U.S. Securities and Exchange Commission, or SEC. Such reports, statements and other information may be obtained by visiting the SEC s Public Reference Room at 100 F Street, NE, Washington, DC 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 trading price 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. 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. Although we announced in November 2012 that our Phase 3 study of Vimizim , an enzyme replacement therapy for patients with MPS IVA (Morquio Syndrome), had met its primary endpoint, Vimizim has not received regulatory approval in the U.S., EU or any other jurisdiction and may never receive approval. Also, even if we receive priority review timelines from the FDA for Vimizim, there is no assurance that the FDA will comply with such timelines and there may be delays and ultimately the FDA may decide not to approve Vimizim.

As part of the recent reauthorization of PDUFA, 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 no 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, including Vimizim, we will be unable to market and sell those drug products. Because of the risks and uncertainties in pharmaceutical development, our product candidates could take a significantly longer time to gain regulatory approval than we expect or may never gain approval. We also rely on independent third-party contract research organizations, or CROs, to file

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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.

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

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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 and Aldurazyme products, as well as certain of our product candidates, including Vimizim, are regulated by the FDA as biologics under the Federal Food, Drug and Cosmetics Act, or the FDC Act, and the Public Health Service 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 Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively, the PPACA, created a regulatory pathway 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 increases 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;

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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.

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 contract research organizations, or 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 and Firdapse, the successful continued commercialization of Aldurazyme by Genzyme, the receipt of regulatory approval of our product candidates, our ability to successfully manufacture and market any approved drugs, either by ourselves or jointly with others, our spending on our development programs and the impact of any possible future business development transactions. The extent of our future losses and the timing of profitability are highly uncertain. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

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

Before we can begin commercial manufacture of our products, 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 European Commission (EC), and health agencies in other countries for the manufacture of Aldurazyme and Naglazyme. The manufacturing facility located in Shanbally, Cork, Ireland that we purchased in 2011has not yet been approved by the FDA or EMA. In addition, our third-party manufacturers facilities involved with the manufacture of Naglazyme, Kuvan, Firdapse and Aldurazyme have also been inspected and approved by various regulatory authorities.

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 and Firdapse 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.

If we, or third-party manufacturers with whom we contract, are unable to comply with manufacturing regulations, we may be subject to 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.

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

As of December 31, 2012, we had cash, cash equivalents and short and long-term investments totaling $566.7 million. We may require additional financing to fund our future operations, including the commercialization of our approved drugs and drug product candidates currently under development, preclinical studies and clinical trials, and potential licenses and acquisitions. We may be unable to raise additional financing, if needed, due to a variety of factors, including our financial condition, the status of our product programs, and the general condition of the financial markets. If we fail to raise additional financing if we need such funds, 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 and Firdapse;
- Genzyme s ability to continue to successfully commercialize Aldurazyme;
- the progress and success of our preclinical studies and clinical trials (including studies and the manufacture of materials);
- the timing, number, size and scope of our preclinical studies and clinical trials;
- the time and cost necessary to obtain regulatory approvals and the costs of post-marketing studies which may be required by regulatory authorities;
- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities;
- the progress of research programs carried out by us;
- our possible achievement of milestones identified in our purchase agreements with the former stockholders of LEAD Therapeutics, Inc., ZyStor, Huxley Pharmaceuticals, Inc., and Zacharon Pharmaceuticals Inc. that trigger related milestone payments;
- any changes made to, or new developments in, our existing collaborative, licensing and other commercial relationships or any new collaborative, licensing and other commercial relationships that we may establish; and
- whether our convertible debt is converted to common stock in the future.

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

additional licenses and collaborative agreements;

additional contracts for product manufacturing; and

additional financing facilities.

We may need to raise additional funds from equity or debt securities, loans or collaborative agreements if we are unable to satisfy our liquidity requirements. The sale of additional securities may result in additional dilution to our stockholders. Furthermore, additional financing may not be available in amounts or on terms satisfactory to us or at all. This could result in the delay, reduction or termination of our research, which could harm our business.

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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. Also, if we contract for manufacturing services with an unproven process, our contractor is subject to the same uncertainties, high standards and regulatory controls, and may therefore experience difficulty if further process development is necessary.

Even a developed manufacturing process can encounter difficulties. Problems may arise during manufacturing for a variety of reasons, including human error, mechanical breakdowns, problems with raw materials and cell banks, malfunctions of internal information technology systems, and other events that cannot always be prevented or anticipated. Many of the processes include biological systems, which add significant complexity, as compared to chemical synthesis. We expect that, from time to time, consistent with biotechnology industry expectations, certain production lots will fail to produce product that meets our quality control release acceptance criteria. To date, our historical failure rates for all of our product programs, including Naglazyme, Aldurazyme and Vimizim, have been within our expectations, which are based on industry norms. If the failure rate increased substantially, we could experience increased costs, lost revenue, damage to customer relations, time and expense investigating the cause and, depending upon the cause, similar losses with respect to other lots or products. If problems are not discovered before the product is released to the market, recall and product liability costs may also be incurred.

In order to produce product within our time and cost parameters, we must continue to produce product within our expected success rate and yield expectations. Because of the complexity of our manufacturing processes, it may be difficult or impossible for us to determine the cause of any particular lot failure and we must effectively take corrective action in response to any failure in a timely manner.

Although we have entered into contractual relationships with third-party manufacturers to produce the active ingredient in Kuvan and Firdapse, if those manufacturers are unwilling or unable to fulfill their contractual obligations, we may be unable to meet demand for these products or sell these products at all and we may lose potential revenue. We have contracts for the production of final product for Kuvan and Firdapse. We also rely on third-parties for portions of the manufacture of Naglazyme and Aldurazyme. If those manufacturers are unwilling or unable to fulfill their contractual obligations or satisfy demand outside of or in excess of the contractual obligations, we may be unable to meet demand for these products or sell these products at all and we may lose potential revenue. Further, the availability of suitable contract manufacturing capacity at scheduled or optimum times is not certain.

In addition, our manufacturing processes subject us to a variety of federal, state and local laws and regulations governing the use, generation, manufacture, storage, handling and disposal of hazardous materials and wastes resulting from their use. We may incur significant costs in complying with these laws and regulations.

If we are unable to effectively address manufacturing issues, we may be unable to meet demand for our products and lose potential revenue, have reduced margins, or be forced to terminate a program.

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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, and 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.

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.

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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 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.

For our future products, we will not know what the reimbursement rates will be until we are ready to market the product and we actually negotiate the rates. If we are unable to obtain sufficiently high reimbursement rates for our products, they may not be commercially viable or our future revenues and gross margins may be adversely affected.

A significant portion of our international sales are made based on special access programs, and changes to these programs could adversely affect our product sales and revenue in these countries.

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

These programs are not well defined in some countries and are subject to changes in requirements and funding levels. Any change to these programs could adversely affect our ability to sell our products in those countries and delay sales. If the programs are not funded by the respective government, there could be insufficient funds to pay for all patients. Further, governments have in the past undertaken and may in the future undertake, unofficial measures to limit purchases of our products, including initially denying coverage for purchasers, delaying orders and denying or taking excessively long to approve customs clearance. Any such actions could materially delay or reduce our revenues from such countries.

Without the special access programs, we would need to seek full product approval to commercially market and sell our products. This can be an expensive and time-consuming process and may subject our products to additional price controls. Because the number of patients is so small in some countries, it may not be economically feasible to seek and maintain a full product approval, and therefore the sales in such country would be permanently reduced or eliminated. For all of these reasons, if the special access programs that we are currently using are eliminated or restricted, our revenues could be adversely affected.

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If we fail to compete successfully with respect to product sales, we may be unable to generate sufficient sales to recover our expenses related to the development of a product program or to justify continued marketing of a product and our revenue could be adversely affected.

Our competitors may develop, manufacture and market products that are more effective or less expensive than ours. They may also obtain regulatory approvals for their products faster than we can obtain them (including those products with orphan drug designation) or commercialize their products before we do. If we do not compete successfully, our revenue would be adversely affected, and we may be unable to generate sufficient sales to recover our expenses related to the development of a product program or to justify continued marketing of a product.

Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our current and future products, which would adversely affect our revenue and results of operations.

We expect that coverage and reimbursement may be increasingly restricted both in the U.S. and internationally. The escalating cost of health care has led to increased pressure on the health care industry to reduce costs. Governmental and private third-party payers have proposed health care reforms and cost reductions. A number of federal and state proposals to control the cost of health care, including the cost of drug treatments, have been made in the U.S. In some international markets, the government controls the pricing, which can affect the profitability of drugs. Current government regulations and possible future legislation regarding health care may affect coverage and reimbursement for medical treatment by third-party payers, which may render our products not commercially viable or may adversely affect our future revenues and gross margins.

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

We cannot predict the extent to which our business may be affected by these or other potential future legislative or regulatory developments. However, future price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our current and future products, which would adversely affect our revenue and results of operations.

Government health care reform could increase our costs, and would adversely affect our revenue and results of operations.

Our industry is highly regulated and changes in law may adversely impact our business, operations or financial results. The PPACA is a sweeping measure intended to expand healthcare coverage within the U.S., primarily through the imposition of health insurance mandates on employers and individuals and expansion of the Medicaid program.

Several provisions of the 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 expands the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance; includes a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or donut hole, and imposes 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

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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. For example, beginning in 2013, drug manufacturers will be required to 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. Although the statute requires reporting by March 31, 2013 of payments and other transfers of value made in calendar year 2012, the CMS has issued a final rule that will go into effect in April 2013 and will require manufacturers to begin collecting required information on August 1, 2013 with the first reports due March 31, 2014.

The reforms imposed by the new law will significantly impact the pharmaceutical industry; however, the full effects of the PPACA cannot be known until these provisions are implemented and the CMS and other federal and state agencies issue applicable regulations or guidance. Moreover, in the coming years, additional changes could be made to governmental healthcare programs that could significantly impact the success of our products or product candidates. We will continue to evaluate the PPACA, as amended, the implementation of regulations or guidance related to various provisions of the PPACA by federal agencies, as well as trends and changes that may be encouraged by the legislation and that may potentially have an impact on our business over time. The cost of implementing more detailed record keeping systems and otherwise complying with these regulations could substantially increase our costs. The changes to the way our products are reimbursed by the CMS could reduce our revenues. Both of these situations could adversely affect our results of operations.

We face credit risks from customers that may adversely affect our results of operations.

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

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

We are subject to various federal and state health care fraud and abuse laws, including anti-kickback laws, false claims laws and laws related to ensuring compliance. The federal health care program anti-kickback statute makes it illegal for any person, including a pharmaceutical company, to knowingly and willfully offer, solicit, pay or receive any remuneration, directly or indirectly, in exchange for or to induce the referral of business, including the purchase, order or prescription of a particular drug, for which payment may be made under federal health care programs, such as Medicare and Medicaid. Under federal government regulations, certain arrangements, or safe harbors, are deemed not to violate the federal anti-kickback statute. However, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration not intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor. Our practices may not in all cases meet all of the criteria for safe harbor protection from anti-kickback liability, although we seek to comply with these safe harbors. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs.

Federal and state false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. Under the Health Insurance Portability and Accountability Act of 1996, we

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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. In addition, the state of California and several other states have passed laws that require pharmaceutical companies to comply with both the April 2003 Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers and the PhRMA Code on Interactions with Healthcare Professionals.

Neither the government nor the courts have provided definitive guidance on the application of some of these laws to our business. Law enforcement authorities are increasingly focused on enforcing these laws, and it is possible that some of our practices may be challenged under these laws. While we believe we have structured our business arrangements to comply with these laws, it is possible that the government could allege violations of, or convict us of violating, these laws. If we are found in violation of one of these laws, we are required to pay a penalty or are suspended or excluded from participation in federal or state health care programs and our business, financial condition and results of operation may be adversely affected.

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 Asia. 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 Foreign Corrupt Practices Act.

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

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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.

We own or have licensed patents and patent applications related to Naglazyme, Kuvan, Aldurazyme and Firdapse and certain of our product candidates, including 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 U.S. Patent Office after grant.

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In addition, competitors may also seek intellectual property protection for their technology. 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 their intellectual property, we would face a number of issues, including the following:

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

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 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.

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 (MMS 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 (MMS Agreement), between Genzyme and us related to Aldurazyme for specified reasons, including if the other party is in material breach of the MMS, 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. Although we are not currently in breach of the MMS, there is a risk that either party could breach the MMS in the future. Either party may also terminate the MMS 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, or the LLC, to the non-breaching party, and the non-breaching party will pay a specified buyout

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amount for the breaching party s interest in Aldurazyme and in the LLC. If we are the breaching party, we would lose our rights to Aldurazyme and the related intellectual property and regulatory approvals. If the MMS Agreement is terminated without cause, the non-terminating party would have the option, exercisable for one year, to buy out the terminating party s interest in Aldurazyme and in the LLC at a specified buyout amount. If such option is not exercised, all rights to Aldurazyme will be sold and the LLC will be dissolved. In the event of termination of the buyout option without exercise by the non-terminating party as described above, all right and title to Aldurazyme is to be sold to the highest bidder, with the proceeds to be split between Genzyme and us in accordance with our percentage interest in the LLC.

If the MMS Agreement is terminated by either party because the other party declared bankruptcy, the terminating party would be obligated to buy out the other party and would obtain all rights to Aldurazyme exclusively. If the MMS Agreement is terminated by a party because the other party experienced a change of control, the terminating party shall notify the other party, the offeree, of its intent to buy out the offeree s interest in Aldurazyme and the LLC for a stated amount set by the terminating party at its discretion. The offeree must then either accept this offer or agree to buy the terminating party s interest in Aldurazyme and the LLC on those same terms. The party who buys out the other party would then have exclusive worldwide rights to Aldurazyme. The Amended and Restated Collaboration Agreement between us and Genzyme will automatically terminate upon the effective date of the termination of the MMS Agreement and may not be terminated independently from the MMS Agreement.

If we were obligated, or given the option, to buy out Genzyme s interest in Aldurazyme and the LLC, and thereby gain exclusive rights to Aldurazyme, we may not have sufficient funds to do so and we may not be able to obtain the financing to do so. If we fail to buy out Genzyme s interest, we may be held in breach of the agreement and may lose any claim to the rights to Aldurazyme and the related intellectual property and regulatory approvals. We would then effectively be prohibited from developing and commercializing Aldurazyme. If this happened, not only would our product revenues decrease, but our share price would also decline.

Based on our strategic alliance with Merck Serono, unless Merck Serono opts in to the 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, 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 the trial has started. 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.

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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 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 abbreviated new drug applications, or 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 no 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 FDA s Approved Drug Products with Therapeutic Equivalence Evaluations (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 (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.

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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 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.

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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.

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 maintain insurance against product liability lawsuits for 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 and Firdapse, or our clinical trials for PEG-PAL, Vimizim, BMN-701, BMN-673 or BMN-111 for which our insurance coverage may not be adequate.

The product liability insurance we will need to obtain in connection with the commercial sales of our product candidates if and when they receive regulatory approval may be unavailable in meaningful amounts or at a reasonable cost. In addition, while we continue to take what we believe are appropriate precautions, 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 any such failure, problem or breach, all of which could adversely affect our business, financial condition and results of operations.

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Our business is affected by macroeconomic conditions.

Various macroeconomic factors could adversely affect our business and the results of our operations and financial condition, including changes in inflation, interest rates and foreign currency exchange rates and overall economic conditions and uncertainties, including those resulting from the current and future conditions in the global financial markets. For instance, if inflation or other factors were to significantly increase our business costs, it may not be feasible to pass through price increases on to our customers due to the process by which health care providers are reimbursed for our products by the government. Interest rates, the liquidity of the credit markets and the volatility of the capital markets could also affect the value of our investments and our ability to liquidate our investments in order to fund our operations. We purchase or enter into a variety of financial instruments and transactions, including investments in commercial paper, the extension of credit to corporations, institutions and governments and hedging contracts. If any of the issuers or counter parties to these instruments were to default on their obligations, it could materially reduce the value of the transaction and adversely affect our cash flows.

For the year ended December 31, 2012, approximately 4% of our net product revenues were from the Southern European countries of Italy, Spain, Portugal and Greece. Approximately 8% of our total accounts receivable as of December 31, 2012 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.

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 and Firdapse;
- manufacture, supply or distribution of Naglazyme, Aldurazyme, Kuvan and Firdapse;
- progress of our product candidates through the regulatory process;
- 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;

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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 our 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, the current decline in the financial markets and related factors beyond our control, including the credit and mortgage crisis in the U.S. and worldwide, may cause our stock price to decline rapidly and unexpectedly.

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 additional shares of preferred stock and to determine the terms of those shares of stock without any further action by our stockholders, which would allow our Board of Directors to implement a stockholder rights plan without approval by our stockholders. The rights of holders of our common stock are subject to the rights of the holders of any preferred stock that may be issued. The issuance of preferred stock could make it more difficult for a third-party to acquire a majority of our outstanding voting stock. Delaware law also prohibits corporations from engaging in a business combination with any holders of 15% or more of their capital stock until the holder has held the stock for three years unless, among other possibilities, our Board of Directors approves the transaction. Our Board of Directors may use these provisions to prevent changes in the management and control of our company. Also, under applicable Delaware law, our Board of Directors may adopt additional anti-takeover measures in the future.

Item 1B. Unresolved Staff Comments

None.

Item 2. Properties

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

Location	Approximate Square Feet	Use	Lease Expiration Date
San Rafael facility, San Rafael, California	120,400	Corporate headquarters, office	2022
Several locations in Novato, California	273,000	Office, laboratory and warehouse	2011-2020
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

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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

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
2011	First Quarter	$ 28.29	$ 23.46
2011	Second Quarter	$ 28.46	$ 24.93
2011	Third Quarter	$ 31.87	$ 24.02
2011	Fourth Quarter	$ 35.38	$ 30.07

On February 15, 2013, the last reported sale price on the NASDAQ Global Select Market for our common stock was $56.28. 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, 2012.

Holders

As of February 15, 2013, there were 54 holders of record of 126,101,610 outstanding shares of our common stock. Additionally, on such date, options to acquire 13.7 million shares of our common stock were outstanding.

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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, 2007 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, 2007 in stock or index, including reinvestment of dividends.

	Fiscal Year Ending December 31,					
	2007	**2008**	**2009**	**2010**	**2011**	**2012**
BioMarin Pharmaceutical Inc.	100.00	50.28	53.14	76.07	97.12	138.98
NASDAQ Composite Index	100.00	59.03	82.25	97.32	98.63	110.78
NASDAQ Biotechnology Index	100.00	93.40	103.19	113.89	129.12	163.33

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Item 6. Selected Consolidated Financial Data

The information set forth below for the five years ended December 31, 2012 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)				
	2012	2011	2010	2009	2008
Consolidated statements of operations data:					
REVENUES:					
Net product revenues	$ 496,497	$ 437,647	$ 369,701	$ 315,721	$ 251,851
Collaborative agreement revenues	1,955	468	682	2,379	38,907
Royalty and license revenues	2,271	3,243	5,884	6,556	5,735
Total revenues	500,723	441,358	376,267	324,656	296,493
OPERATING EXPENSES:					
Cost of sales (excludes amortization of certain acquired intangible assets)	91,830	84,023	70,285	65,909	52,509
Research and development	302,218	214,374	147,309	115,116	93,291
Selling, general and administrative	198,173	175,423	151,723	124,290	106,566
Intangible asset amortization and contingent consideration	18,717	1,428	6,406	2,914	4,371
Total operating expenses	610,938	475,248	375,723	308,229	256,737
INCOME (LOSS) FROM OPERATIONS	(110,215)	(33,890)	544	16,427	39,756
Equity in the loss of BioMarin/Genzyme LLC	(1,221)	(2,426)	(2,991)	(2,594)	(2,270)
Interest income	2,584	2,934	4,112	5,086	16,695
Interest expense	(7,639)	(8,409)	(10,818)	(14,404)	(16,394)
Debt conversion expense	0	(1,896)	(13,728)	0	0
Impairment loss on equity investments	0	0	0	(5,848)	(4,056)
Net gain from sale of investments	0	0	902	1,585	0
Other income (expense)	(1,787)	60	489	314	(307)
INCOME (LOSS) BEFORE INCOME TAXES	(118,278)	(43,627)	(21,490)	566	33,424
Provision for (benefit from) income taxes	(3,931)	10,209	(227,309)	1,054	2,593
NET INCOME (LOSS)	$ (114,347)	$ (53,836)	$ 205,819	$ (488)	$ 30,831
NET INCOME (LOSS) PER SHARE, BASIC	$ (0.95)	$ (0.48)	$ 2.00	$ (0.00)	$ 0.31
NET INCOME (LOSS) PER SHARE, DILUTED	$ (0.95)	$ (0.48)	$ 1.73	$ (0.00)	$ 0.29
Weighted average common shares outstanding, basic	120,271	112,122	103,093	100,271	98,975
Weighted average common shares outstanding, diluted	120,271	112,122	125,674	100,271	103,572

	December 31, (in thousands)				
	2012	2011	2010	2009	2008
Consolidated balance sheet data:					
Cash, cash equivalents and investments	$ 566,731	$ 289,477	$ 402,283	$ 470,526	$ 561,425
Total current assets	743,462	469,802	504,260	467,727	737,696
Total assets	1,601,643	1,305,709	1,262,623	917,163	906,695
Long-term liabilities, net of current portion	415,447	438,536	461,522	516,824	499,939
Total stockholders equity	1,015,763	773,048	717,257	322,185	276,675

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

	Three Months Ended (In thousands, except per share data, unaudited)			
	March 31,	June 30,	September 30,	December 31,
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)
2011:				
Total revenue	$ 109,456	$ 110,631	$ 113,425	$ 107,846
Net loss	(4,371)	(5,077)	(17,653)	(26,735)
Net loss per share, basic and diluted	(0.04)	(0.05)	(0.16)	(0.23)

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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 include the following (in millions):

	Years Ended December 31,		
	2012	2011	2010
Total net product revenues	$ 496.5	$ 437.6	$ 369.7
Cost of sales	91.8	84.0	70.3
Research and development expense	302.2	214.4	147.3
Selling, general and administrative expense	198.2	175.4	151.7
Intangible asset amortization and contingent consideration	18.7	1.4	6.4
Provision for (benefit from) income taxes	(3.9)	10.2	(227.3)
Net income (loss)	(114.3)	(53.8)	205.8
Stock-based compensation expense	48.0	43.8	37.5

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

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

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 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, 2012, totaled $257.0 million, compared to $224.9 million and $192.7 million, respectively, for the years ended December 31, 2011 and 2010.

Kuvan was granted marketing approval for the treatment of phenylketonuria (PKU) in the U.S. and in the EU in December 2007 and December 2008, respectively. Our Kuvan net product revenues for the year ended December 31, 2012 totaled $143.1 million, compared to $116.8 million and $99.4 million, respectively, for the years ended December 31, 2011 and 2010.

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, 2012 totaled $14.2 million, compared to $13.1 million and $6.4 million, respectively, for the years ended December 31, 2011 and 2010.

Aldurazyme (laronidase), which was developed in collaboration with Genzyme Corporation (Genzyme), was approved in 2003 for marketing in the U.S., the EU and subsequently in other countries for patients with

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

mucopolysaccharidosis I (MPS I). Our Aldurazyme net product revenues for the year ended December 31, 2012 totaled $82.2 million, compared to $82.8 million and $71.2 million, respectively for the years ended December 31, 2011 and 2010.

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

- Vimizim™, formerly referred to as GALNS, an enzyme replacement therapy for the treatment of mucopolysaccharidosis Type IV or Morquio Syndrome Type A, a lysosomal storage disorder;
- 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; and
- BMN-111, a peptide therapeutic for the treatment of achondroplasia, the leading cause of dwarfism.

We are conducting preclinical development of several other product candidates for genetic and other metabolic diseases, including BMN-190 for late infantile neuronal ceroid lipofuscinosis (LINCL), a lysosomal storage disorder primarily affecting the brain.

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

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 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.

Our cash, cash equivalents, short-term investments and long-term investments totaled $566.7 million as of December 31, 2012, compared to $289.5 million as of December 31, 2011. We have historically financed our operations primarily through 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. Further, depending on market conditions, our

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

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 SEC, we make assumptions, judgments and estimates that can have a significant impact on our net income/(loss) and affect the reported amounts of certain assets, liabilities, revenue and expenses, and related disclosures. We base our assumptions, judgments and estimates on historical experience and various other factors that we believe to be reasonable under the circumstances. Actual results could differ materially from these estimates under different assumptions or conditions. On a regular basis, we evaluate our assumptions, judgments and estimates. We also discuss our critical accounting policies and estimates with the Audit Committee of our Board of Directors.

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

Business Combinations

We allocate the purchase price of acquired businesses to the tangible and intangible assets acquired and liabilities assumed based upon their estimated fair values on the acquisition date. The purchase price allocation process requires management to make significant estimates and assumptions, especially at the acquisition date with respect to intangible assets and in-process research and development (IPR&D). In connection with the purchase price allocations for acquisitions, we estimate the fair value of contingent acquisition consideration payments utilizing a probability-based income approach inclusive of an estimated discount rate.

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

- the feasibility and timing of achievement of development, regulatory and commercial milestones;
- expected costs to develop the 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.

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

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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 re-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 our investment in BioMarin/Genzyme LLC, long-term investments, property, plant and equipment, intangible assets and goodwill. We regularly review long-lived assets for impairment. The recoverability of our debt and equity investments is measured by available external market data, including quoted prices on public exchanges and other relevant information. If the carrying amount of the asset is not recoverable, an impairment loss is recorded for the amount that the carrying value of the asset exceeds its fair value.

The recoverability of long-lived assets, other than goodwill, indefinite-lived intangible assets and our long-term investments is measured by comparing the asset s carrying amount to the expected undiscounted future cash flows that the asset is expected to generate. Determining whether an impairment has occurred typically requires various estimates and assumptions, including determining which cash flows are directly related to the potentially impaired asset, the useful life over which cash flows will occur, their amount, and the asset s residual value, if any. In turn, measurement of an impairment loss requires a determination of fair value, which is based on the best information available. We use internal cash flow estimates, quoted market prices when available and independent appraisals as appropriate to determine fair value. We derive the required cash flow estimates from our historical experience and our internal business plans and apply an appropriate discount rate.

The recoverability of the carrying value of buildings, leasehold improvements for our facilities and equipment will depend on the successful execution of our business initiatives and our ability to earn sufficient returns on our approved products and product candidates. We continually monitor events and changes in circumstances that could indicate carrying amounts of our fixed assets may not be recoverable. When such events or changes in circumstances occur, we assess 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, we recognize an impairment loss based on the excess of the carrying amount over the fair value of the assets. Based on management s current estimates, we expect to recover the carrying value of such assets.

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

We have recorded intangible assets, primarily related to IPR&D, and goodwill as part of our recognition and measurement of assets acquired and liabilities assumed in conjunction with our business combinations. Goodwill and intangible assets determined to be indefinite-lived assets are not amortized, but are required to be reviewed annually for impairment or more frequently if events and circumstances indicate that the carrying value may not be recoverable. We perform our annual impairment test of indefinite-lived intangible assets in the fourth quarter of each fiscal year and in between annual tests if we become aware of any events or changes in circumstances that would indicate a reduction in the fair value of the assets below their carrying values. As of December 31, 2012, we had $63.7 million of indefinite-lived assets related to IPR&D projects acquired from our business combinations. We performed a qualitative assessment of events and circumstances that could affect the fair value of our indefinite-lived intangible assets. Our assessment included consideration of various factors including costs associated with the underlying development programs, expected future revenues and cash flows, legal, regulatory and other entity specific factors that may have a significant impact on the inputs used to determine fair value of our indefinite-lived intangible assets. Based on our qualitative assessment, we determined that it is not more likely than not that the fair value of our indefinite-lived intangible assets is less than their carrying amounts at December 31, 2012.

At December 31, 2012, the net book value of our intangible assets whose lives are considered finite in nature was $99.3 million. These intangible assets are related to marketing rights for Naglazyme, Kuvan and Firdapse, which are being amortized over their estimated useful lives using the straight-line method. We review these intangible assets for impairment when facts or circumstances indicate a reduction in the fair value below their carrying amount.

As of December 31, 2012, we had goodwill of $51.5 million resulting from our business combinations. We currently operate in one business segment, the biopharmaceutical development and commercialization segment. 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 it is not more likely than not that the fair value of our goodwill is less than its carrying amount at December 31, 2012.

Revenue Recognition

We recognize revenue in accordance with FASB ASC Subtopics ASC 605-15, *Revenue Recognition Products* and ASC 605-25, *Revenue Recognition Multiple-Element Arrangements.* Our revenues consist of net product revenues from commercial products, revenues from our collaborative agreement with Merck Serono and other license and royalty revenues. Milestone payments are recognized in full when the related milestone performance goal is achieved and we have no future performance obligations related to that payment.

Net Product Revenues We recognize net product revenue when persuasive evidence of an arrangement exists, the product has been delivered to the customer, title and risk of loss have passed to the customer, the price to the buyer is fixed or determinable and collection from the customer is reasonably assured. 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 Naglazyme and Firdapse 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.

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

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 the 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, 2012 and 2011, accounts receivable included $32.4 million and $31.0 million, respectively, of unbilled accounts receivable related to net incremental Aldurazyme product transfers to Genzyme.

We sell Naglazyme worldwide, Kuvan in the U.S. and Canada and Firdapse in the EU. In the U.S., Naglazyme and Kuvan 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 four percent. Outside the U.S., Naglazyme and Firdapse are sold to our authorized distributors or directly to government purchasers or hospitals, which act as the end-users. 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 Naglazyme, Kuvan and Firdapse 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, 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.

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

The nature and amount of our current estimates of the applicable revenue dilution items that are currently applied to aggregate world-wide gross 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, 2012	2011	Description
Rebates	0.9-5.0%	0.9-3.2%	Rebates payable to state Medicaid, other government programs and certain managed care providers
Distributor Fees	0.3-3.8%	0.3-2.9%	Fees paid to authorized distributors
Cash Discounts	0.5-1.9%	0.5-1.9%	Discounts offered to customers for prompt payment of accounts receivable
Total	1.7-10.7%	1.7-8.0%	

We maintain a policy to record allowances for doubtful accounts for estimated losses resulting from our customers inability to make required payments. As of December 31, 2012, our allowance for doubtful accounts was $0.3 million, compared to $0.5 million as of December 31, 2011.

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 inventories at the lower of cost or net realizable value. We determine the cost of inventory using the average-cost method. 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. Expired inventory is disposed of and the related costs are recognized as cost of sales on the Consolidated Statements of Operations.

Inventories consist of currently marketed products and 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. When regulatory approval and the likelihood of future revenues for a product candidate are less certain, the related manufacturing costs expensed as research and development expenses.

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 on our consolidated results of operations and financial condition.

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

Results of Operations

Net Income (Loss)

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 gross profit from product sales	51.0
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)
Decreased income tax expense	14.1
Loss on conversion of promissory note	(2.0)
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.

Our net loss for the year ended December 31, 2011 was $53.8 million, compared to net income of $205.8 million for the year ended December 31, 2010. The change in net income (loss) was primarily a result of the following (in millions):

Net income for the year ended December 31, 2010	$ 205.8
Absence of benefit from the reversal of deferred tax asset valuation allowance	(230.6)
Increased gross profit from product sales	54.2
Increased research and development expense	(67.1)
Increased selling, general and administrative expense	(23.7)
Decreased intangible asset amortization and contingent consideration expense	5.0
Decreased debt conversion expense	11.9
Increased income tax expense, excluding valuation allowance reversal	(6.9)
Other individually insignificant fluctuations	(2.4)
Net loss for the year ended December 31, 2011	$ (53.8)

The increase in gross profit from product sales during the year ended December 31, 2011 as compared to the year ended December 31, 2010 was primarily a result of additional Naglazyme patients initiating therapy, additional Kuvan patients initiating therapy in the U.S. and increased Firdapse sales in Europe. The increase in research and development expense was primarily attributed to increased development expenses for our Vimizim,

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

PEG-PAL, Firdapse, BMN-701 and BMN-673 programs. The increase in selling, general and administrative expense was primarily due to increased facility and employee related costs, continued international expansion of Naglazyme, U.S. commercialization activities related to Kuvan, the commercialization of Firdapse in Europe and increased bad debt expense.

See below for additional information related to the primary net income/(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):

	Year Ended December 31,				
	2012	2011	2010	2012 v. 2011	2011 v. 2010
Naglazyme	$ 257.0	$ 224.9	$ 192.7	$ 32.1	$ 32.2
Kuvan	143.1	116.8	99.4	26.3	17.4
Firdapse	14.2	13.1	6.4	1.1	6.7
Aldurazyme	82.2	82.8	71.2	(0.6)	11.6
Total net product revenues	$ 496.5	$ 437.6	$ 369.7	$ 58.9	$ 67.9

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

	Year Ended December 31,				
	2012	2011	2010	2012 v. 2011	2011 v 2010
Naglazyme	$ 218.5	$ 186.9	$ 158.3	$ 31.6	$ 28.6
Kuvan	118.9	98.1	82.7	20.8	15.4
Firdapse	11.4	10.8	5.0	0.6	5.8
Aldurazyme	55.8	57.8	53.4	(2.0)	4.4
Total gross profit	$ 404.6	$ 353.6	$ 299.4	$ 51.0	$ 54.2

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

	Year Ended December 31,				
	2012	2011	2010	2012 v. 2011	2011 v. 2010
Aldurazyme revenue reported by Genzyme	$ 193.1	$ 185.2	$ 166.8	$ 7.9	$ 18.4
Royalties earned from Genzyme	$ 80.4	$ 74.2	$ 68.0	$ 6.2	$ 6.2
Incremental (previously recognized) Aldurazyme product transfer revenue	1.8	8.6	3.2	(6.8)	5.4
Total Aldurazyme net product revenues	$ 82.2	$ 82.8	$ 71.2	$ (0.6)	$ 11.6

2012 compared to 2011

Naglazyme net product revenues 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. 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

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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. For additional discussion of the transaction see Note 6 to the Consolidated Financial Statements.

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.

The Hatch Waxman Act permits the FDA to approve abbreviated new drug applications, (ANDA), for generic versions of branded drugs. See Part I. Item 1 Business Government Regulation for details related to the Hatch-Waxman Act. Pursuant to the Hatch-Waxman Act, other companies were able to file an ANDA for the active ingredient in Kuvan at any time after December 2011. If a generic competitor were to enter the market following the expiration of orphan exclusivity it would have an adverse effect on our sales of Kuvan.

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.

2011 compared to 2010

Naglazyme net product revenues for the year ended December 31, 2011 totaled $224.9 million, of which $194.2 million was earned from customers 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 $0.2 million for 2011. Gross margins from Naglazyme sales during 2011 were 83%, compared to 82% during 2010. Naglazyme gross margins for the year ended December 31, 2011 were consistent with expectations and were expected to improve slightly in 2012 as a result of our purchase of the Naglazyme intellectual property from SA Pathology in November 2011. Prior to the purchase, we licensed the intellectual property from SA Pathology and paid them a 5% royalty on net sales of Naglazyme. See Note 6 to our accompanying Consolidated Financial Statements for additional discussion of the transaction.

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

Net product revenue for Kuvan for the year ended December 31, 2011 was $116.8 million, compared to $99.4 million for the year ended December 31, 2010. Gross margins from Kuvan during 2011 were approximately 84%, compared to 83% during 2010. The increase in gross margins was primarily attributed to price increases at the end of 2010. Cost of goods sold for the years ended December 31, 2011 and 2010 reflect royalties paid to third parties of 10% and 11%, respectively. The 4% royalties from Merck Serono s net sales of Kuvan during 2011 were $1.6 million, compared to $0.9 million in 2010. Kuvan gross margins for the year ended December 31, 2011 were consistent with expectations.

We launched Firdapse in Europe on a country-by-country basis beginning in April 2010. Net product revenue for Firdapse for the year ended December 31, 2011 was $13.1 million, compared to $6.4 million for the year ended December 31, 2010. Gross margins from Firdapse during 2011 were 82% compared to 79% during 2010. Cost of goods sold for the years ended December 31, 2011 and 2010 reflect royalties paid to third parties of approximately 8%.

During the year ended December 31, 2011, Aldurazyme gross margins were 70%, compared to the year ended December 31, 2010 when gross margins were 75%. 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, 2011 was $84.0 million, compared to $70.3 million for the year ended December 31, 2010. The increase in cost of sales during 2011 compared to 2010 was primarily attributed to the increase in product sales 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 perform during a given period and then prioritize 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.

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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 in 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 was attributed to increased clinical trial and manufacturing activities related to the product candidate. The increase in BMN-673 and BMN-701 development expenses were attributed to increased clinical trial activities related to these product candidates. The increase in BMN-190 development expense was attributed to increased pre-clinical activities related to this product candidate. The decrease in PEG-PAL development expense was attributed to the timing of purchases of materials to produce the drug substance for the clinical trial. The decrease in BMN-111 development expense 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 expense primarily includes increased research and development personnel and facility costs that are not allocated to specific programs.

In 2013, we expect research and development spending to increase over 2012 levels due to our Vimizim, PEG-PAL, BMN-673, BMN-701, BMN-111 and BMN-190 programs progressing to more advanced phases of clinical studies as well as increased spending on pre-clinical and clinical activities for our early development stage programs. 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 prelaunch 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 prelaunch 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 expensed as research and development expenses.

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

Research and development expense increased to $214.4 million for the year ended December 31, 2011, from $147.3 million for the year ended December 31, 2010. 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, 2010	$ 147.3
Increased Vimizim development expenses	26.4
Increased BMN-701 development expenses	15.0
Increased PEG-PAL development expenses	11.3
Increased BMN-111 development expenses	11.3
Increased ongoing development expenses related to commercial products	2.8
Increased stock-based compensation expense related to research and development	2.6
Decreased BMN-195 for Duchenne muscular dystrophy development expenses	(3.3)
Decreased BMN-673 development expenses	(0.9)
Increase in non-allocated research and development expenses and other net changes	1.9
Research and development expense for the year ended December 31, 2011	$ 214.4

The increase in Vimizim, PEG-PAL, BMN-673 and BMN-701 development expenses were attributed to increased clinical trial activities related to these product candidates. The increase in research and development expenses related to commercial products was primarily attributed to long-term Firdapse clinical activities related to post-approval regulatory commitments in the EU. The decrease in development expense related to BMN-195 was attributed to the termination of our license agreement with Summit plc in October 2010. The increase in stock-based compensation expense was a result of an increased number of options outstanding due to an increased number of employees. The increase in non-allocated research and development expense primarily included increased research and development personnel costs that were not allocated to specific programs.

Selling, General and Administrative

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
Increased foreign exchange losses on unhedged transactions	(2.3)
Selling, general and administrative expense for the year ended December 31, 2012	$ 198.2

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

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. 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 and the administrative support of our expanding operations.

Selling, general and administrative expense increased to $175.4 million for the year ended December 31, 2011, from $151.7 million for the year ended December 31, 2010. 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, 2010	$ 151.7
Net increase in corporate overhead and other administrative expenses	12.4
Increased sales and marketing expenses related to commercial products	8.4
Increased foreign exchange loss on unhedged transactions	1.9
Increased Vimizim pre-commercial expense	1.7
Increased bad debt expense	1.1
Absence of transaction costs related to the acquisition of ZyStor Therapeutics, Inc (ZyStor)	(1.8)
Selling, general and administrative expense for the year ended December 31, 2011	$ 175.4

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, and spending related to the European commercialization of Firdapse, which launched in the EU in April 2010. The increase in corporate overhead and other administrative costs during 2011 was primarily comprised of increased employee related costs, legal costs, accounting costs and facility costs.

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 assumed probability of achievement or timing of milestones and adjustments to the discount periods and rates. Intangible asset amortization and contingent consideration expense consisted of the following (in millions):

	Year Ended December 31,			Change	
	2012	2011	2010	2012 v. 2011	2011 v 2010
Amortization of Firdapse European marketing rights	$ 3.2	$ 3.2	$ 2.4	$ 0	$ 0.8
Impairment loss on intangible assets	6.7	0	0	6.7	0
Changes in the fair value of contingent acquisition consideration payable	8.8	(1.8)	4.0	10.6	(5.8)
Total intangible asset amortization and contingent consideration	$ 18.7	$ 1.4	$ 6.4	$ 17.3	$ (5.0)

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

In the first quarter of 2012, we recorded an impairment charge of $6.7 million related to the U.S. Firdapse in-process research and development (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. The increase in the fair value of the contingent acquisition consideration payable was primarily attributed to increases in the assumed probability of achieving development milestones based on the current status of the related development programs.

The 2011 increase in the amortization of the Firdapse European marketing rights was attributed to the timing of the European commercial launch of Firdapse which occurred in April 2010.

See Note 6 to our accompanying Consolidated Financial Statements for additional discussion.

Equity in the Loss of BioMarin/Genzyme LLC

Equity in the loss of BioMarin/Genzyme LLC 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 $1.2 million for the year ended December 31, 2012 compared to $2.4 million and $3.0 million for the years ended December 31, 2011 and 2010, 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 $2.6 million for the year ended December 31, 2012, compared to $2.9 million and $4.1 million for the years ended December 31, 2011 and 2010, respectively. The reduction in interest income during 2012, as compared to 2011 and 2010 was primarily due to lower market interest rates. We expect that interest income will increase during 2013 as compared to 2012 due to higher cash and investment balances resulting from the net proceeds received from the sale of 6.5 million shares of our common stock in June 2012.

Interest Expense and Debt Conversion Expense

We incur interest expense on our convertible debt and our capital leases. Interest expense for the year ended December 31, 2012 was $7.6 million, compared to $8.4 million and $10.8 million for the years ended December 31, 2011 and 2010, respectively. The decrease in interest expense was attributed to the early conversion of $29.2 million and $119.6 million in aggregate principal of our 2013 Notes in September 2011 and November 2010, respectively. In connection with the early conversion of the 2013 Notes, we recognized debt conversion expense of $1.9 million and $13.7 million in 2011 and 2010, respectively. We expect interest expense to decrease in 2013, compared to 2012 as our senior subordinated convertible notes mature in March 2013. See Note 11 to our accompanying Consolidated Financial Statements for additional discussion.

Provision for (Benefit from) Income Taxes

During the year ended December 31, 2012 we recognized an income tax benefit of $3.9 million, compared to an income tax expense of $10.2 million and an income tax benefit of $227.3 million for the years ended December 31, 2011 and 2010, respectively. The provision for (benefit from) income taxes for 2012 and 2011 consisted of state, federal and foreign current tax expense of $6.0 million and $5.8 million, respectively. The provision for (benefit from) income taxes also consisted of deferred tax expense related to the utilization of our

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federal net operating loss carryforwards and a portion of our credit carryforwards which were offset by deferred tax benefits from federal orphan drug credits and California R&D credits of $32.6 million and $19.2 million earned during 2012 and 2011, respectively. The benefit from income tax during 2010 consisted of foreign and state current tax expense and deferred tax benefit related to the release of $230.6 million of our valuation allowance during 2010. See Note 19 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, *Income Taxes,* we calculate our provision for (benefit from) income taxes on an entity-by-entity and jurisdiction-by-jurisdiction basis as adjusted for differences between book-basis income and tax-basis income, which results in certain foreign entities being profitable and incurring foreign current income tax expense. Certain foreign entities incur significant amounts of 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 $168.0 million of foreign net losses. Other foreign operations generated U.S. GAAP income of approximately $5.0 million with an effective tax rate of approximately 38%.

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. We expect our current cash, cash equivalents and short-term and long-term investments will meet our operating and capital requirements for at least the next twelve months based on our current business plans. This expectation could also change depending on how much we elect to spend on our development programs and for potential licenses and acquisitions of complementary technologies, products and companies. In June 2012, we sold 6.5 million shares of our common stock at a price of $36.28 per share in an underwritten public offering pursuant to an effective registration statement. We received net cash proceeds of $235.5 million from the public offering. 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.

We consider the unrepatriated cumulative earnings of certain of our foreign subsidiaries to be invested indefinitely outside the U.S. As of December 31, 2012, $17.1 million of our $566.7 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.

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

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

	2012	2011	2010	2012 v. 2011	2011 v. 2010
Cash and cash equivalents	$ 180.5	$ 46.3	$ 88.1	$ 134.2	$ (41.8)
Short-term investments	270.2	148.8	186.0	121.4	(37.2)
Long-term investments	116.0	94.4	128.2	21.6	(33.8)
Cash, cash equivalents and investments	$ 566.7	$ 289.5	$ 402.3	$ 277.2	$ (112.8)
Current assets	$ 743.5	$ 469.8	$ 504.3	$ 273.7	(34.5)
Current liabilities	170.4	94.1	83.8	76.3	10.3
Working capital	$ 573.1	$ 375.7	$ 420.5	$ 197.4	$ (44.8)
Convertible debt	$ 348.2	$ 348.3	$ 377.5	$ (0.1)	$ (29.2)

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

	2012	2011	2010	2012 v. 2011	2011 v. 2010
Cash and cash equivalents at the beginning of the period	$ 46.3	$ 88.1	$ 167.2	$ (41.8)	$ (79.1)
Net cash provided by operating activities	17.6	18.8	18.7	(1.2)	0.1
Net cash used in investing activities	(195.6)	(89.6)	(101.3)	(106.0)	11.7
Net cash provided by financing activities	312.2	29.0	3.5	283.2	25.5
Cash and cash equivalents at the end of the period	$ 180.5	$ 46.3	$ 88.1	$ 134.2	$ (41.8)
Short-term and long-term investments	386.2	243.2	314.2	143.0	(71.0)
Cash, cash equivalents and investments	$ 566.7	$ 289.5	$ 402.3	$ 277.2	$ (112.8)

Cash, cash equivalents and investments

The increase in cash, cash equivalents and investments in 2012 from December 31, 2011 was primarily attributed to the net proceeds of $235.5 million from our public offering of our common stock in June 2012, proceeds from employee stock purchases under the Employee Stock Purchase Plan (ESPP) and employee stock option exercises of $81.4 million.

The decrease in cash, cash equivalents and investments in 2011 from December 31, 2010 was primarily attributed to the $49.7 million of cash used in the purchase of the Shanbally facility and the $81.0 million purchase of the Naglazyme intellectual property (Naglazyme IP), partially offset by proceeds from employee stock purchases under the ESPP and employee stock option exercises of $33.6 million.

Working Capital

Working capital was $573.1 million at December 31, 2012, an increase of $197.4 million from working capital of $375.7 million at December 31, 2011. The increase in working capital was attributed to the following:

Working capital at December 31, 2011	$ 375.7

Increased cash, cash equivalents and short-term investments	255.6
Increased current deferred tax assets	8.3
Increased accounts payable and accrued liabilities	(52.9)
Reclassification of 2013 Notes from long-term convertible debt	(23.4)
Net increase in other current operating assets	9.8
Working capital at December 31, 2012	$ 573.1

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

The increase in cash, cash equivalents and short-term investments resulted from net proceeds of $235.5 million from the public offering of our common stock in June 2012. The increase in other current assets is primarily attributed to the increases in prepaid expenses, current deferred tax assets and short-term restricted investments. The restricted investments secure our irrevocable standby letter of credit obtained in connection with our new corporate facility lease agreements and certain other commercial arrangements. The classification of the 2013 Notes as a current liability from long-term convertible debt was due to their maturity in March 2013.

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, 2012, approximately 8% of our outstanding accounts receivable relate to such countries. See Note 18 of our accompanying Consolidated Financial Statements for additional discussion.

Cash Provided by Operating Activities

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.

Cash provided by operating activities of $18.8 million for the year ended December 31, 2011 primarily related to net loss of $53.8 million, adjusted for non-cash items such as $36.1 million of depreciation and amortization expenses, $43.9 million of stock-based compensation expense, $4.4 million of deferred income taxes and $7.2 million of unrealized foreign exchange gains on forward foreign currency exchange contracts and $25.1 million of net cash outflows related to changes in operating assets and liabilities.

Cash Used in Investing Activities

Net cash used in investing activities during the year ended December 31, 2012 was $195.6 million, compared to net cash used in investing activities of $89.6 million and $101.3 million for the years ended December 31, 2011 and 2010, respectively. Our investing activities have consisted primarily of purchases and sales and maturities of investments and capital expenditures. 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. The decrease in net cash used in investing activities for the year

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ended December 31, 2011 compared to the year ended December 31, 2010 was primarily due to increased capital expenditures of $23.8 million and lower spending on business acquisitions of $33.0 million, partially offset by the $81.0 million purchase of Naglazyme IP and increased net settlements of investment securities of $81.9 million. In 2011, capital expenditures were primarily comprised of our purchase of the Shanbally facility for a total purchase price of $49.7 million.

Cash Provided by Financing Activities

Net cash provided by financing activities for the year ended December 31, 2012 was $312.2 million, compared to net cash provided by financing activities of $29.0 million and $3.5 million for the years ended December 31, 2011 and 2010, respectively. Historically, our financing activities primarily included payments related to our contingent acquisition obligations, payments related to our convertible debt obligations and proceeds from employee stock purchases under the ESPP and employee stock option exercises. The 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 decrease in payments of contingent acquisition consideration. The increase in net cash provided by financing activities for the year ended December 31, 2011, was primarily attributed to the decrease in payments of contingent acquisition consideration of $14.0 million and lower induced debt conversion payments of $11.9 million. See Notes 11 and 13 to our accompanying Consolidated Financial Statements for additional discussion regarding our convertible debt and the June 2012 public offering of our common stock, respectively.

Other Information

In March 2006, we sold approximately $172.5 million of senior subordinated convertible notes due March 2013 (the 2013 Notes) of which $23.4 million remains outstanding at December 31, 2012. The debt was issued at face value and bears interest at the rate of 2.5% per annum, payable semi-annually in cash. The debt does not contain a call provision included and we are unable to unilaterally redeem the remaining debt prior to maturity in 2013. The remaining $23.4 million of the 2013 Notes is 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 $16.58 per share, subject to adjustment in certain circumstances. However, we must repay the remaining debt prior to maturity 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 notes.

In April 2007, we sold approximately $324.9 million of senior subordinated convertible notes due April 2017 (the 2017 Notes). The debt was issued at face value and bears interest at the rate of 1.875% per annum, payable semi-annually in cash. The debt is 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 notes. See Note 11 to our accompanying Consolidated Financial Statements for additional discussion.

Our $348.2 million of total convertible debt as of December 31, 2012 will impact our liquidity due to the semi-annual cash interest payments and will impact our liquidity if the holders 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.

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

On October 23, 2009, we acquired Huxley Pharmaceuticals Inc. (Huxley), which has rights to Firdapse for a total purchase price of $37.2 million, of which $15.0 million was paid in cash and $22.2 million represented the acquisition date fair value of contingent acquisition consideration payable. In connection with the acquisition, we agreed to pay the Huxley stockholders additional consideration in future periods of up to $41.9 million (undiscounted) in milestone payments if certain annual sales, cumulative sales and U.S. development milestones are met. During 2011, 2010 and 2009 we made milestone payments of $3.0 million, $6.5 million and $1.0 million, respectively, related to the attainment of development milestones.

On February 10, 2010, we acquired LEAD Therapeutics, Inc. (LEAD), which had the key compound now referred to as BMN-673, for a total purchase price of $39.1 million, of which $18.6 million was paid in cash and $20.5 million represented the acquisition date fair value of contingent acquisition consideration payable. We paid $3.0 million of the $18.6 million in cash during December 2009. In connection with the acquisition, we agreed to pay the LEAD stockholders additional consideration in future periods of up to $68.0 million (undiscounted) in milestone payments if certain clinical, development and sales milestones are met. During 2012 and 2010, we paid the former LEAD stockholders $6.0 million and $11.0 million for the attainment of a clinical milestone and regulatory milestone, respectively.

On August 17, 2010, we acquired ZyStor, which had the compound now referred to as BMN-701, for a total purchase price of $35.9 million, of which $20.3 million was paid in cash and $15.6 million represented the acquisition date fair value of contingent acquisition consideration payable. In connection with the acquisition, we agreed to pay ZyStor stockholders additional consideration in future periods of up to $93.0 million (undiscounted) in milestone payments if certain clinical, development and sales milestones are met.

On January 4, 2013, we acquired Zacharon Pharmaceuticals, Inc. (Zacharon), which focused on developing small molecules targeting pathways of glycan and glycolipid metabolism, for an upfront payment of $10.0 million, of which $1.7 million was held in escrow. 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 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 included in this Annual Report on Form 10-K:

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.

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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 during the three years ended December 31, 2012, 2011 and 2010 and the period since inception (March 1997 for the portion not allocated to any major program) were as follows (in millions):

	Year Ended December 31,			Since Program Inception
	2012	2011	2010	
Vimizim	$ 97.0	$ 54.5	$ 28.1	$ 211.8
Naglazyme	12.4	10.3	9.7	164.8
Kuvan	14.1	12.6	12.8	140.8
Firdapse	5.4	11.0	8.8	25.7
BMN-673	11.4	7.4	8.3	27.1
BMN-701	31.6	17.5	2.5	51.6
BMN-111	12.1	13.6	2.3	31.9
BMN-190	11.1	1.2	2.4	17.7
PEG-PAL	26.7	27.7	16.4	113.2
Not allocated to specific major current projects	80.4	58.6	56.0	Not meaningful
Totals	$ 302.2	$ 214.4	$ 147.3	

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 and Firdapse; preclinical studies and clinical trials for our other product candidates; potential licenses and other acquisitions of complementary technologies, products and companies; and general corporate purposes.

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

- our ability to successfully market and sell Naglazyme, Kuvan and Firdapse;
- Genzyme s ability to continue to successfully commercialize Aldurazyme;
- the progress and success of our preclinical studies and clinical trials (including studies and the manufacture of materials);
- the timing, number, size and scope of our preclinical studies and clinical trials;
- the time and cost necessary to obtain regulatory approvals and the costs of post-marketing studies which may be required by regulatory authorities;
- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities;

the progress of research programs carried out by us;

our possible achievement of milestones identified in our purchase agreements with the former stockholders of LEAD, ZyStor, Huxley and Zacharon 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.

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.

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

Contractual and Commercial Obligations

We have contractual and commercial obligations under our debt, operating leases and other obligations related to research and development activities, purchase commitments, licenses and sales royalties with annual minimums. Information about these obligations as of December 31, 2012 is presented in the table below (in millions).

	Payments Due by Period					
	2013	2014	2015-2016	2017-2018	2019 and Thereafter	Total
Convertible debt and related interest	$ 29,749	$ 6,091	$ 12,182	$ 327,905	$ 0	$ 375,927
Operating leases	9,015	6,749	13,486	11,582	16,424	57,256
Research and development and purchase commitments	6,378	2,992	2,620	0	0	11,990
Total	$ 45,142	$ 15,832	$ 28,288	$ 339,487	$ 16,424	$ 445,173

We are also subject to contingent payments related to various development activities totaling approximately $285.5 million as of December 31, 2012, which are due upon achievement of certain development and commercial milestones, and if they occur before certain dates in the future. As of December 31, 2012 $41.4 million of the contingent payments are included in contingent acquisition consideration payable on our accompanying Consolidated Balance Sheets, of which $10.8 million is current.

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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.

As of December 31, 2012, 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, 2012, 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 $5.0 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.

The table below presents the carrying value of our cash and investment portfolio, which approximates fair value at December 31, 2012 (in millions):

	Carrying Value
Cash and cash equivalents	$ 180.5*
Short-term investments	270.2**
Long-term investments	116.0***
Total	$ 566.7

* 70% of cash and cash equivalents are invested in money market instruments and 30% in cash.

** 82% of short-term investments are invested in corporate debt securities, 14% in certificates of deposit, 3% in U.S. government agency securities and 1% in corporate equity securities.

*** 82% of long-term investments are invested in corporate debt securities, 10% in certificates of deposit and 8% in U.S. government agency securities.

Our debt obligations consist of our convertible debt, which carries a fixed interest rate and, as a result, we are not exposed to interest rate market risk on our convertible debt. The carrying value of our convertible debt approximates its fair value at December 31, 2012.

Foreign Currency Exchange Rate Risk

We transact business in various foreign currencies, primarily in Euros 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. Our operating expenses in the United Kingdom, other European countries and Brazil are in British Pounds, Euros and Real, respectively, which serve to mitigate a portion of the exposure related to the above-mentioned revenue in both markets.

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

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short-term foreign currency exposure resulting from currency fluctuations by entering into forward foreign currency exchange contracts. These contracts have maturities of less than 17 months.

As of December 31, 2012, we had forward foreign currency exchange contracts to sell approximately 83.0 million Euros and to buy approximately 6.0 million Brazilian Reais. As of December 31, 2012, our outstanding forward foreign currency exchange contracts had a net fair value of $0.1 million, of which $1.5 million was included in other current assets, $1.0 million was included in accounts payable and accrued expenses and $0.4 million was included in other long-term liabilities 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, 2012, 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 $11.4 million. We expect to enter into new transactions based in foreign currencies that could be impacted by changes in exchange rates.

At December 31, 2012, we had cash of approximately $25.4 million denominated in foreign currencies, which represented approximately 14% of the 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-46 of this report.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

None.

Item 9A. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

An evaluation was carried out, under the supervision of and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, of the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act as of the end of the period covered by this report. Based on the evaluation, our Chief Executive Officer and our Chief Financial Officer have concluded that our disclosure controls and procedures are effective to ensure that the information required to be disclosed by us in the reports we file or submit under the Exchange Act was recorded, processed, summarized and reported within the time periods specified in the SEC s rules and forms.

Management s Annual Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining an adequate internal control structure and procedures for financial reporting. Under the supervision of and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, our management has assessed the effectiveness of our internal control over financial reporting as defined in Rule 13a-15(f) under the Exchange Act as of December 31, 2012. Our management s assessment was based on criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission, or COSO, Internal Control-Integrated Framework.

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Based on the COSO criteria, we believe our internal control over financial reporting as of December 31, 2012 was effective.

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

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting during our most recently completed quarter that have materially affected or are reasonably likely to materially affect our internal control over financial reporting, as defined in Rule 13a-15(f) under the Exchange Act.

Scope of the Effectiveness of Controls

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

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

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

Item 9B. Other Information

None

Part III

Item 10. Directors, Executive Officers and Corporate Governance

We incorporate information regarding our directors, executive officers and corporate governance into this section by reference from sections captioned Election of Directors and Executive Officers in the proxy statement for our 2013 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 2013 annual meeting of stockholders.

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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 2013 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 2013 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 2013 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 2013 annual meeting of stockholders.

Part IV

Item 15. Exhibits, Financial Statement Schedules

Financial Statements

In accordance with Rule 3-09 of Regulation S-X, the comparative unaudited 2012 and 2011 and audited 2010 Consolidated Financial Statements and accompanying notes of BioMarin/Genzyme LLC, which constituted a significant subsidiary in 2010 are filed herewith as Exhibit 99.1 to this Annual Report on Form 10-K.

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Exhibit Index

3.1	Amended and Restated Certificate of Incorporation, as amended June 12, 2003, previously filed with the Commission 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 Commission 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 Commission 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 Commission on December 23, 2010 as Exhibit 3.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
3.5	Certificate of Elimination of Series B Junior Participating Preferred Stock, dated May 30, 2012, previously filed with the Commission on May 31, 2012 as Exhibit 3.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
4.1	Indenture dated June 23, 2003, by and between BioMarin Pharmaceutical Inc. and Wilmington Trust Company, previously filed with the Commission on August 12, 2003 as Exhibit 4.1 to the Company s Quarterly report on Form 10-Q, which is incorporated herein by reference.
4.2	Indenture dated March 29, 2006, by and between BioMarin Pharmaceutical Inc. and Wilmington Trust Company, previously filed with the Commission on March 29, 2006 as Exhibit 4.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
4.3	First Supplemental Indenture dated March 29, 2006, by and between BioMarin Pharmaceutical Inc. and Wilmington Trust Company, previously filed with the Commission on March 29, 2006 as Exhibit 4.2 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
4.4	Form of 2.5% Senior Subordinated Convertible Notes due 2013, previously filed with the Commission on March 29, 2006 as Exhibit 4.3 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
4.5	Second Supplemental Indenture, dated April 23, 2007, by and between BioMarin Pharmaceutical Inc. and Wilmington Trust Company, previously filed with the Commission on April 23, 2007 as Exhibit 4.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
4.6	Form of 1.875% Senior Subordinated Convertible Notes due 2017, previously filed with the Commission on April 23, 2007 as Exhibit 4.2 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 Commission on October 19, 2010 as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.

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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, previously filed with the Commission on July 31, 2009 as Exhibit 10.1 to the Company s Quarterly Report on Form 10-Q, which is incorporated by reference herein.
10.3	Amendment to 1997 Stock Plan, as amended, as adopted March 20, 2002, previously filed with the Commission 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 Commission 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 Commission 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 Commission 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 Commission 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 Commission 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 Commission on August 3, 2006 as Exhibit 10.3 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference.
10.10	Amended and Restated BioMarin Pharmaceutical Inc. 2006 Share Incentive Plan as adopted on adopted on May 12, 2010, incorporated by reference to Appendix A of the Company s Definitive Proxy Statement on Schedule 14A, as filed with the Commission on March 26, 2010.
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 Commission 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 Commission 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 Stephen Aselage dated January 1, 2009 previously filed with the Commission on December 23, 2008 as Exhibit 10.2 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.

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10.15	Amended and Restated Employment Agreement with Robert A. Baffi dated January 1, 2009 previously filed with the Commission on December 23, 2008, as Exhibit 10.3 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.16	Amended and Restated Employment Agreement with Jeffrey H. Cooper dated January 1, 2009 previously filed with the Commission on December 23, 2008 as Exhibit 10.5 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.17	Amended and Restated Employment Agreement with G. Eric Davis dated January 1, 2009, previously filed with the Commission on December 23, 2008 as Exhibit 10.6 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.18	Amended and Restated Employment Agreement with Mark Wood dated January 1, 2009 previously filed with the Commission on December 23, 2008 as Exhibit 10.7 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.19	Employment Agreement with Henry Fuchs, dated March 18, 2009, previously filed with the Commission on March 23, 2009 as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.20	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 Commission 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 Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.21	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 Commission 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 Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.22	Development, License and Commercialization Agreement dated May 13, 2005, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the Commission 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 Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.23	Operating Agreement with Genzyme Corporation, previously filed with the Commission 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.
10.24	License Agreement between BioMarin Pharmaceutical Inc. and Women s and Children s Hospital dated February 7, 2007, previously filed with the Commission 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 Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.25	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 Commission on February 22, 2012 as Exhibit 10.25 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference.

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10.26	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 Commission 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 Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.27	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 Commission 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 Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.28	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 Commission 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 Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.29	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 Commission 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 Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.30	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 Commission 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 Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.31	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 Commission 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 Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.32	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 Commission 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 Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.
10.33	Asset Purchase Agreement dated June 22, 2011 between BioMarin Manufacturing Ireland Limited and Pfizer Biotechnology Ireland, previously filed with the Commission 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 Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Commission.

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10.34	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 Commission on February 22, 2012 as Exhibit 10.34 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference.
10.35	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 Commission on February 22, 2012 as Exhibit 10.35 to the Company s Annual Report on Form 10-K, which is incorporated herein by reference.
10.36	Severance Agreement and Release of All Claims with Jeffrey H. Cooper, dated February 21, 2012, previously filed with the Commission on February 22, 2012 as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.37	Employment Agreement with Daniel Spiegelman dated May 8, 2012, previously filed with the Commission on May 9, 2012 as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.38	Amendment No. 1 to Employment Agreement with Stephen Aselage dated May 8, 2012, previously filed with the Commission on May 9, 2012 as Exhibit 10.3 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.39	Amendment No. 1 to Employment Agreement with Robert A. Baffi dated May 8, 2012, previously filed with the Commission on May 9, 2012 as Exhibit 10.4 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.40	Amendment No. 1 to Employment Agreement with G. Eric Davis dated May 8, 2012, previously filed with the Commission on May 9, 2012 as Exhibit 10.5 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.41	Amendment No. 1 to Employment Agreement with Henry J. Fuchs dated May 8, 2012, previously filed with the Commission on May 9, 2012 as Exhibit 10.6 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.42	Amendment No. 1 to Employment Agreement with Mark Wood dated May 8, 2012, previously filed with the Commission on May 9, 2012 as Exhibit 10.7 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.43	Amendment No. 2 to Employment Agreement with Robert A. Baffi dated May 24, 2012, previously filed with the Commission on May 24, 2012 as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.44	Amendment No. 2 to Employment Agreement with Henry J. Fuchs dated May 24, 2012, previously filed with the Commission on May 24, 2012 as Exhibit 10.2 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.45	BioMarin Pharmaceutical Inc 2012 Inducement Plan, adopted May 8, 2012, previously filed with the Commission on May 9, 2012 as Exhibit 10.2 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.46	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 Commission 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 Commission has granted confidential treatment with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Comission.

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10.47	Form of Stock Option Agreement for the BioMarin Pharmaceutical Inc. 2006 Share Incentive Plan. (As Amended and Restated 2010), previously filed with the Commission on August 2, 2012 as Exhibit 10.11 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference.
10.48	Form of Restricted Stock Unit Agreement for the BioMarin Pharmaceutical 2006 Share Incentive Plan. (As Amended and Restated 2010), previously filed with the Commission on August 2, 2012 as Exhibit 10.12 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference.
10.49	Form of Stock Option Agreement for the BioMarin Pharmaceutical Inc. 2012 Inducement Plan, previously filed with the Commission on August 2, 2012 as Exhibit 10.13 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference.
10.50	Form of Restricted Stock Unit Agreement for the BioMarin Pharmaceutical Inc. 2012 Inducement Plan, previously filed with the Commission on August 2, 2012 as Exhibit 10.14 to the Company s Quarterly Report on Form 10-Q, which is incorporated herein by reference.
10.51	Employment Agreement with Jeffrey R. Ajer dated September 5, 2012, previously filed with the Commission on September 5, 2012 as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.52	Severance Agreement and Release of All Claims with Stephen Aselage, dated September 4, 2012, previously filed with the Commission on September 5, 2012 as Exhibit 10.2 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.53	Amendment No. 1 to Employment Agreement with Daniel Spiegelman dated December 17, 2012, previously filed with the Commission on December 18, 2012 as Exhibit 10.1 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.54	Amendment No. 1 to Amended and Restated Employment Agreement with Jean-Jacques Bienaime dated December 17, 2012, previously filed with the Commission on December 18, 2012 as Exhibit 10.2 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.55	Amendment No. 1 to Employment Agreement with Jeffery R. Ajer dated December 17, 2012, previously filed with the Commission on December 18, 2012 as Exhibit 10.3 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.56	Amendment No. 3 to Employment Agreement with Robert A. Baffi dated December 17, 2012, previously filed with the Commission on December 18, 2012 as Exhibit 10.4 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.57	Amendment No. 3 to Employment Agreement with Henry J. Fuchs dated December 17, 2012, previously filed with the Commission on December 18, 2012 as Exhibit 10.5 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.58	Amendment No. 2 to Employment Agreement with G. Eric Davis dated December 17, 2012, previously filed with the Commission on December 18, 2012 as Exhibit 10.6 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.
10.59	Amendment No. 2 to Employment Agreement with Mark Wood dated December 17, 2012, previously filed with the Commission on December 18, 2012 as Exhibit 10.7 to the Company s Current Report on Form 8-K, which is incorporated herein by reference.

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10.60*	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. Portions of this document have been redacted pursuant to a Request for Confidential Treatment filed pursuant to the Freedom of Information Act.
21.1*	Subsidiaries of BioMarin Pharmaceutical Inc.
23.1*	Consent of KPMG LLP, Independent Registered Public Accounting Firm for BioMarin Pharmaceutical Inc.
23.2*	Consent of PricewaterhouseCoopers LLP, Independent Accountants for BioMarin/Genzyme LLC.
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, 2012 and 2011, and for the three years ended December 31, 2012.
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase
101.LAB	XBRL Taxonomy Extension Labels Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Link Document

* Filed herewith
Management contract or compensatory plan or arrangement

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SIGNATURES

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

BIOMARIN PHARMACEUTICAL INC.

Dated: February 26, 2013

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

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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, 2013
/s/ DANIEL SPIEGELMAN **Daniel Spiegelman**	Executive Vice President and Chief Financial Officer (Principal Financial Officer)	February 26, 2013
/s/ BRIAN R. MUELLER **Brian R. Mueller**	Vice President, Corporate Controller and Chief Accounting Officer (Principal Accounting Officer)	February 26, 2013
/s/ PIERRE LAPALME **Pierre LaPalme**	Chairman and Director	February 26, 2013
/s/ KENNETH BATE **Kenneth Bate**	Director	February 26, 2013
/s/ MICHAEL G. GREY **Michael G. Grey**	Director	February 26, 2013
/s/ ELAINE HERON **Elaine Heron**	Director	February 26, 2013
/s/ V. BRYAN LAWLIS **V. Bryan Lawlis**	Director	February 26, 2013
/s/ ALAN J. LEWIS **Alan J. Lewis**	Director	February 26, 2013
/s/ RICHARD A. MEIER **Richard A. Meier**	Director	February 26, 2013

/s/ WILLIAM YOUNG	Director	February 26, 2013

William Young

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BIOMARIN PHARMACEUTICAL INC.

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

The Board of Directors and Stockholders

BioMarin Pharmaceutical Inc.:

We have audited the accompanying consolidated balance sheets of BioMarin Pharmaceutical Inc. and subsidiaries (the Company) as of December 31, 2012 and 2011, and the related consolidated statements of operations, comprehensive income (loss), stockholders equity, and cash flows for each of the years in the three-year period ended December 31, 2012. 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, 2012 and 2011, and the results of their operations and their cash flows for each of the years in the three-year period ended December 31, 2012, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), BioMarin Pharmaceutical Inc. s internal control over financial reporting as of December 31, 2012, based on criteria established in *Internal Control Integrated Framework* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) , and our report dated February 26, 2013 expressed an unqualified opinion on the effectiveness of the Company s internal control over financial reporting.

/s/ KPMG LLP

San Francisco, California

February 26, 2013

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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, 2012, based on criteria established in *Internal Control Integrated Framework* 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, 2012, based on criteria established in *Internal Control Integrated Framework* 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, 2012 and 2011, and the related consolidated statements of operations, comprehensive income (loss), stockholders equity, and cash flows for each of the years in the three-year period ended December 31, 2012, and our report dated February 26, 2013 expressed an unqualified opinion on those consolidated financial statements.

/s/ KPMG LLP

San Francisco, California

February 26, 2013

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BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED BALANCE SHEETS

December 31, 2012 and 2011

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

	December 31, 2012	December 31, 2011
ASSETS		
Current assets:		
Cash and cash equivalents	$ 180,527	$ 46,272
Short-term investments	270,211	148,820
Accounts receivable, net (allowance for doubtful accounts: $348 and $513, respectively)	109,066	104,839
Inventory	128,695	130,118
Current deferred tax assets	29,454	21,115
Other current assets	25,509	18,638
Total current assets	743,462	469,802
Noncurrent assets:		
Investment in BioMarin/Genzyme LLC	1,080	559
Long-term investments	115,993	94,385
Property, plant and equipment, net	284,473	268,971
Intangible assets, net	162,980	180,277
Goodwill	51,543	51,543
Long-term deferred tax assets	225,501	224,677
Other assets	16,611	15,495
Total assets	$ 1,601,643	$ 1,305,709
LIABILITIES AND STOCKHOLDERS EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 147,068	$ 94,125
Convertible debt	23,365	0
Total current liabilities	170,433	94,125
Noncurrent liabilities:		
Long-term convertible debt	324,859	348,329
Long-term contingent acquisition consideration payable	30,618	33,059
Long-term deferred tax liabilities	33,296	37,155
Other long-term liabilities	26,674	19,993
Total liabilities	585,880	532,661
Stockholders equity:		
Common stock, $0.001 par value: 250,000,000 shares authorized at December 31, 2012 and 2011: 125,809,162 and 114,789,732 shares issued and outstanding at December 31, 2012 and 2011, respectively.	126	115
Additional paid-in capital	1,561,890	1,197,082
Company common stock held by Nonqualified Deferred Compensation Plan	(6,603)	(3,935)
Accumulated other comprehensive income (loss)	(202)	4,887
Accumulated deficit	(539,448)	(425,101)

Total stockholders equity	1,015,763	773,048
Total liabilities and stockholders equity	$ 1,601,643	$ 1,305,709

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

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BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF OPERATIONS

Years Ended December 31, 2012, 2011 and 2010

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

	2012	2011	2010
REVENUES:			
Net product revenues	$ 496,497	$ 437,647	$ 369,701
Collaborative agreement revenues	1,955	468	682
Royalty and license revenues	2,271	3,243	5,884
Total revenues	500,723	441,358	376,267
OPERATING EXPENSES:			
Cost of sales (excludes amortization of certain acquired intangible assets)	91,830	84,023	70,285
Research and development	302,218	214,374	147,309
Selling, general and administrative	198,173	175,423	151,723
Intangible asset amortization and contingent consideration	18,717	1,428	6,406
Total operating expenses	610,938	475,248	375,723
INCOME (LOSS) FROM OPERATIONS	(110,215)	(33,890)	544
Equity in the loss of BioMarin/Genzyme LLC	(1,221)	(2,426)	(2,991)
Interest income	2,584	2,934	4,112
Interest expense	(7,639)	(8,409)	(10,818)
Debt conversion expense	0	(1,896)	(13,728)
Net gain from sale of investments	0	0	902
Other income (expense)	(1,787)	60	489
INCOME (LOSS) BEFORE INCOME TAXES	(118,278)	(43,627)	(21,490)
Provision for (benefit from) income taxes	(3,931)	10,209	(227,309)
NET INCOME (LOSS)	$ (114,347)	$ (53,836)	$ 205,819
NET INCOME (LOSS) PER SHARE, BASIC	$ (0.95)	$ (0.48)	$ 2.00
NET INCOME (LOSS) PER SHARE, DILUTED	$ (0.95)	$ (0.48)	$ 1.73
Weighted average common shares outstanding, basic	120,271	112,122	103,093
Weighted average common shares outstanding, diluted	120,271	112,122	125,674

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

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BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME (LOSS)

Years Ended December 31, 2012, 2011 and 2010

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

	2012	2011	2010
NET INCOME (LOSS)	$ (114,347)	$ (53,836)	$ 205,819
OTHER COMPREHENSIVE INCOME (LOSS):			
Net foreign currency gain (loss)	(301)	6	(2)
Available-for-sale securities:			
Unrealized holding gain (loss) arising during the period, net of tax impact of $(140), $(229) and $390 for the years ended December 31, 2012, 2011 and 2010, respectively.	388	(508)	(1,983)
Reclassifications to net income (loss), net of tax impact of $40, $12 and $(148) for the years ended December 31, 2012, 2011 and 2010, respectively.	(110)	27	755
Net Change	278	(481)	(1,228)
Cash flow hedges:			
Unrealized holding gain (loss) arising during the period, net of tax impact of $5,114, $(4,500) and $418 for the years ended December 31, 2012, 2011 and 2010, respectively.	(8,749)	8,163	(3,726)
Reclassifications to net income (loss), net of tax impact of $(2,153), $1,648 and $(473) for the years ended December 31, 2012, 2011 and 2010, respectively.	3,683	(2,989)	4,211
Net Change	(5,066)	5,174	485
OTHER COMPREHENSIVE INCOME (LOSS), NET OF TAX	(5,089)	4,699	(745)
COMPREHENSIVE INCOME (LOSS)	$ (119,436)	$ (49,137)	$ 205,074

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

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BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF STOCKHOLDERS EQUITY

Years Ended December 31, 2012, 2011 and 2010

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

	Common stock			Company Common Stock Held by Nonqualified Deferred			
	Shares	Amount	Additional Paid-in Capital	Compensation Plan	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders Equity
Balance at December 31, 2009	100,962	$ 101	$ 899,950	$ (1,715)	$ 933	$ (577,084)	$ 322,185
Net income						205,819	205,819
Other comprehensive loss					(745)		(745)
Issuance of common stock under Employee Stock Purchase Plan (ESPP)	317		3,777				3,777
Exercise of common stock options	2,040	2	29,461				29,463
Excess tax benefit from stock option exercises			541				541
Conversion of convertible notes	7,213	8	118,234				118,242
Restricted stock vested during the period, net	102		(137)				(137)
Common stock held by Nonqualified Deferred Compensation Plan				(250)			(250)
Stock-based compensation			38,362				38,362
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 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
Restricted stock vested during the period, net	162		(1,659)				(1,659)
Common stock held by Nonqualified Deferred Compensation Plan				(2,668)			(2,668)
Stock-based compensation			47,340				47,340

Balance at December 31, 2012	125,809	$ 126	$ 1,561,890	$ (6,603)	$ (202)	$ (539,448)	$ 1,015,763

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

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BIOMARIN PHARMACEUTICAL INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS

Years Ended December 31, 2012, 2011 and 2010

(In thousands of U.S. dollars)

	2012	2011	2010
CASH FLOWS FROM OPERATING ACTIVITIES:			
Net income (loss)	$ (114,347)	$ (53,836)	$ 205,819
Adjustments to reconcile net income (loss) to net cash provided by operating activities:			
Depreciation and amortization	45,295	36,094	27,737
Accretion of discount on investments	4,469	4,036	4,453
Equity in the loss of BioMarin/Genzyme LLC	1,221	2,426	2,991
Stock-based compensation	47,340	43,909	38,362
Impairment of intangible assets	6,707	0	0
Loss on conversion of convertible promissory note	2,000	0	0
Net gain from sale of investments	0	0	(902)
Deferred income taxes	(9,921)	4,363	(230,577)
Excess tax benefit from stock option exercises	(473)	(415)	(541)
Unrealized foreign exchange (loss) gain on forward contracts	(6,529)	7,174	(4,220)
Changes in the fair value of contingent acquisition consideration payable	8,788	(1,795)	3,989
Debt conversion expense	0	1,896	