BIOMARIN PHARMACEUTICAL INC
Form 424B5
March 04, 2014
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The information in this prospectus is not complete and may be changed. A registration statement relating to these securities has been declared effective by the Securities and Exchange Commission. This preliminary prospectus supplement and the accompanying prospectus are not an offer to sell these securities and we are not soliciting an offer to buy these securities in any state where the offer or sale is not permitted.

Subject to Completion

Preliminary Prospectus Supplement dated March 4, 2014

PROSPECTUS SUPPLEMENT

(To prospectus dated October 7, 2013)

1,500,000 Shares

Common Stock

We are selling 1,500,000 shares of our common stock.

Our shares trade on the NASDAQ Global Select Market under the symbol BMRN. On March 3, 2014, the last sale price of the shares as reported on the NASDAQ Global Select Market was $79.72 per share.

Investing in our common stock involves risks, including those described in the Risk Factors section beginning on page S-14 of this prospectus supplement and in our Annual Report on Form 10-K for the fiscal year ended December 31, 2013, which is incorporated herein by reference.

The underwriter has agreed to purchase the common stock from us at a price of $ per share, which will result in $ of proceeds to us before expenses. The underwriter may offer the shares of common stock from time to time for sale in one or more transactions on the NASDAQ Global Select Market, in the over-the-counter market, through negotiated transactions or otherwise at market prices prevailing at the time of sale, at prices related to prevailing market prices or at negotiated prices.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus supplement or the accompanying prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

The shares will be ready for delivery on or about March , 2014.

BofA Merrill Lynch

The date of this prospectus supplement is March , 2014.

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You should rely only on the information contained or incorporated by reference in this prospectus supplement, the accompanying prospectus, the documents incorporated by reference therein and any free writing prospectus we provide you. We have not, and the underwriter has not, authorized anyone to provide you with different information. If anyone provides you with different or inconsistent information, you should not rely on it. We are not, and the underwriter is not, making an offer to sell these securities in any jurisdiction where the offer or sale is not permitted. You should assume that the information appearing in this prospectus supplement, the accompanying prospectus, the documents incorporated by reference in this prospectus supplement and the accompanying prospectus and any free writing prospectus we provide you is accurate only as of the date on those respective documents. Our business, financial condition, results of operations and prospects may have changed since those dates. You should read this prospectus supplement and the accompanying prospectus, including the documents incorporated by reference in this prospectus supplement and the accompanying prospectus, when making your investment decision. You should also read and consider the information in the documents we have referred you to in the sections of this prospectus supplement entitled Where You Can Find More Information and Information Incorporated by Reference.

General information about us can be found on our website at *www.bmrn.com*. The information on our website is for information only and should not be relied on for investment purposes. The information on our website is not incorporated by reference into either this prospectus supplement or the accompanying prospectus and should not be considered part of this or any other report filed with the Securities and Exchange Commission (the SEC).

BioMarin®, Naglazyme® and Kuvan® are registered trademarks of BioMarin Pharmaceutical Inc., or its affiliates. Aldurazyme® is a registered trademark of BioMarin/Genzyme LLC. VIMIZIM and Firdapse are trademarks of BioMarin Pharmaceutical Inc., or its affiliates.

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ABOUT THIS PROSPECTUS SUPPLEMENT

This prospectus supplement and the accompanying prospectus are part of a registration statement that we filed with the SEC, utilizing a shelf registration process. This prospectus supplement provides you with the specific details regarding this offering. The accompanying prospectus provides you with more general information, some of which does not apply to the offering of our common stock. To the extent information in this prospectus supplement is inconsistent with the accompanying prospectus or any of the documents incorporated by reference into this prospectus supplement and the accompanying prospectus, you should rely on this prospectus supplement. You should read and consider the information in both this prospectus supplement and the accompanying prospectus together with the additional information described under the headings Where You Can Find More Information and Information Incorporated by Reference .

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CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus supplement, the accompanying prospectus and the documents incorporated by reference in this prospectus supplement and the accompanying prospectus contain forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the Securities Act), and Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act), that involve substantial risks and uncertainties. All statements, other than statements of historical facts, included in this prospectus supplement, the accompanying prospectus or any document incorporated by reference in this prospectus supplement and the accompanying prospectus regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects and plans and objectives of management are forward-looking statements.

Forward-looking statements include, but are not limited to, statements about:

- our expectations with respect to regulatory submissions and approvals and our clinical trials;
- any projection or expectation of earnings, revenue or other financial items;
- the plans, strategies and objectives of management for future operations;
- factors that may affect our operating results;
- new products or services;
- the demand for our products;
- our ability to consummate acquisitions and successfully integrate them into our operations;
- future capital expenditures;
- effects of current or future economic conditions on performance;
- industry trends and other matters that do not relate strictly to historical facts or statements of assumptions underlying any of the foregoing;
- our success in any future litigation; and
- our estimates regarding our capital requirements and our need for additional financing.

The words anticipates, believes, estimates, expects, intends, may, plans, projects, will, would and similar expressions are inte forward-looking statements, although not all forward-looking statements contain these identifying words. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements and you should not place undue reliance on our forward-looking

statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements that we make. We have identified some of the important factors that could cause future events to materially differ from our current expectations and they are described in this prospectus supplement under the caption Risk Factors as well as in our most recent Annual Report on Form 10-K. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make. We do not assume any obligation to update any forward-looking statement.

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PROSPECTUS SUPPLEMENT SUMMARY

This summary highlights selected information contained elsewhere or incorporated by reference in this prospectus supplement. This summary does not contain all the information that you should consider before investing in our common stock. You should read the entire prospectus supplement and the accompanying prospectus carefully, including Risk Factors, the financial statements and related footnotes thereto and other information included or incorporated by reference in this prospectus supplement and the accompanying prospectus before making an investment decision. This prospectus supplement contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from the results anticipated in these forward- looking statements as a result of factors described under the Risk Factors section and elsewhere in this prospectus supplement. Unless the context otherwise requires, any reference to BioMarin, the Company, we, our and us in this prospectus supplement refers to BioMarin Pharmaceutical Inc. and its subsidiaries.

BioMarin Pharmaceutical Inc.

Overview

We develop and commercialize innovative pharmaceuticals for serious diseases and medical conditions. We select product candidates for diseases and conditions that represent a significant unmet medical need, have well-understood biology and provide an opportunity to be first-to-market or offer a significant benefit over existing products. Our product portfolio is comprised of five approved products and multiple investigational product candidates. Approved products include Naglazyme® (galsulfase), Kuvan® (sapropterin dihydrochloride), Aldurazyme® (laronidase), Firdapse (amifampridine phosphate) and VIMIZIM (elosulfase alpha).

Naglazyme received marketing approval in the United States (the U.S.) in May 2005, in the European Union (the EU) in January 2006 and subsequently in other countries. Kuvan was granted marketing approval in the U.S. and EU in December 2007 and December 2008, respectively. In December 2009, the European Medicines Agency (the EMA) granted marketing approval for Firdapse, which was launched in the EU in April 2010. Aldurazyme, which was developed in collaboration with Genzyme Corporation (Genzyme), was approved in 2003 for marketing in the U.S. and the EU, and subsequently in other countries. VIMIZIM received marketing approval in the U.S. on February 14, 2014.

We are conducting clinical trials on several investigational product candidates for the treatment of various diseases including: PEG PAL, an enzyme substitution therapy for the treatment of phenylketonuria or PKU, BMN 701, an enzyme replacement therapy for Pompe disease, a glycogen storage disorder, BMN 673, an orally available poly (ADP-ribose) polymerase (PARP) inhibitor for the treatment of patients with certain cancers, BMN 111, a peptide therapeutic for the treatment of achondroplasia and BMN 190, an enzyme replacement therapy for the treatment of late infantile neuronal ceroid lipofuscinosis (CLN2), a form of Batten disease. We are conducting or planning to conduct preclinical development of several other product candidates for genetic and other metabolic diseases and recently announced the selection of two new drug development candidates, BMN 270 and BMN 250. BMN 270 is a Factor VIII gene therapy drug development candidate, an AAV VIII vector, for the treatment of hemophilia A. BMN 250 is a novel fusion of alpha-N-acetyglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo B syndrome or Mucopolysaccharidosis type IIIB (MPS IIIB).

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Summary of Commercial Products and Major Development Programs

A summary of our various commercial products and major development programs, including key metrics as of December 31, 2013, is provided below:

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

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

(1) Mucopolysaccharidosis VI, or MPS VI
(2) Phenylketonuria, or PKU
(3) The Aldurazyme total product revenue noted above is the total product revenue recognized by us in accordance with the terms of our agreement with Genzyme Corporation. See Commercial Products Aldurazyme below for further discussion.
(4) Mucopolysaccharidosis I, or MPS I
(5) Lambert Eaton Myasthenic Syndrome, or LEMS
(6) Mucopolysaccharidosis IV Type A, or MPS IVA
(7) Pompe disease, a glycogen storage disorder
(8) The Phase 2 clinical trial began in January 2014.
(9) BMN 673 is an orally available poly (ADP-ribose) polymerase, or PARP inhibitor for the treatment of patients with certain cancers.
(10) CLN2, or late infantile neuronal ceroid lipofuscinosis, is a lysosomal storage disorder primarily affecting the brain.
(11) Firdapse has not received marketing approval in the U.S. and we have the North American rights to develop and market Firdapse to a third party.
(12) Merck Serono markets Kuvan in the EU.
(13) We anticipate receiving marketing approval in the EU in the second quarter of 2014.

Recent Developments

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

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

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

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

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

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

In February 2014, we announced the selection of a new drug development candidate, BMN 250, a novel fusion of alpha-N-acetyglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo B syndrome or Mucopolysaccharidosis type IIIB (MPS IIIB). We have initiated IND-enabling studies and expect to initiate clinical studies with BMN 250 in mid-2015. Discovered by BioMarin, BMN 250 is an enzyme replacement therapy using recombinant human NAGLU with an IGF2, or Glycosylation Independent Lysosomal Targeting (GILT) tag. BMRN 250 is delivered directly to the brain using our patented technology.

Contract to Purchase San Rafael Corporate Center

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

Convertible Debt Offering

On October 15, 2013, we completed a convertible debt offering of $750.0 million of our senior subordinated convertible notes consisting of $375.0 million 0.75% senior subordinated convertible notes due 2018 (the 2018 Notes) and $375.0 million 1.50% senior subordinated convertible notes due 2020 (the 2020 Notes and together with the 2018 Notes, the Notes).The Notes will be convertible, under certain circumstances, into cash, shares of our common stock or a combination of cash and common stock at our election. The initial conversion rate will be 10.6213 shares of common stock per $1,000 principal amount of Notes (representing an initial conversion price of approximately $94.15 per common share), subject to customary adjustments. The initial conversion rate represents approximately a 40% premium to the last reported sale price of our common stock on the NASDAQ Global Select Market on October 8, 2013. We also entered into privately-negotiated capped call transactions with respect to 50% of the principal amount of the Notes with three of the underwriters or their affiliates. The capped call transactions are generally expected to reduce potential dilution to our common stock upon conversion of the relevant Notes in excess of the principal amount of such converted Notes. The cap price of the capped call transactions entered into with respect to 50% of the Notes will initially be, in each case, approximately $121.05, which represents a premium of approximately 80% over the NASDAQ closing price of a share of our common stock on October 8, 2013 and is subject to certain adjustments under the terms of such capped call transactions. We received net proceeds after fees, transaction costs and the purchase of the related capped calls of approximately $696.4 million, which we intend to use for general corporate purposes.

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Commercial Products

Naglazyme

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

Naglazyme was granted marketing approval in the U.S. in May 2005 and in the EU in January 2006. We market Naglazyme in the U.S., EU, Canada, Latin America, Turkey and other areas using our own sales force and commercial organization. Additionally, we use local distributors in several other regions to help us pursue registration and/or market Naglazyme on a named patient basis. Naglazyme net product revenues for the years ended December 31, 2013, 2012 and 2011 totaled $271.2 million, $257.0 million and $224.9 million, respectively.

Kuvan

Kuvan is a proprietary synthetic oral form of 6R-BH4, a naturally occurring enzyme co-factor for phenylalanine hydroxylase (PAH), indicated for patients with PKU. Kuvan is the first drug for the treatment of PKU, which is an inherited metabolic disease that affects at least 50,000 diagnosed patients under the age of 40 in the developed world. We believe that approximately 30% to 50% of those with PKU could benefit from treatment with Kuvan. PKU is caused by a deficiency of activity of an enzyme, PAH, which is required for the metabolism of phenylalanine (Phe). Phe is an essential amino acid found in all protein-containing foods. Without sufficient quantity or activity of PAH, Phe accumulates to abnormally high levels in the blood, resulting in a variety of serious neurological complications, including severe mental retardation and brain damage, mental illness, seizures and other cognitive problems. As a result of newborn screening efforts implemented in the 1960s and early 1970s, virtually all PKU patients under the age of 40 in developed countries have been diagnosed at birth. Currently, PKU can be managed by a Phe-restricted diet, which is supplemented by nutritional replacement products, like formulas and specially manufactured foods; however, it is difficult for most patients to adhere to the strict diet to the extent needed for achieving adequate control of blood Phe levels. Kuvan has been demonstrated to reduce blood Phe levels 30% in approximately 30% of patients.

In December 2013, the FDA approved the use of Kuvan powder for oral solution which will be provided in a dose sachet packet allowing faster dissolution of powder in solution compared to the current tablet form. This new dosage form is expected to have increasing appeal for young patients in the 1-7 year age range. We commenced the commercial launch of this new form of Kuvan on February 28, 2014.

Kuvan was granted marketing approval for the treatment of PKU in the U.S. in December 2007. We market Kuvan in the U.S. and Canada using our own sales force and commercial organization. Kuvan has been granted orphan drug status in the U.S., which confers seven years of market exclusivity in the U.S. for the treatment of PKU, expiring in December 2014. We expect that our patents will provide market exclusivity beyond the expiration of orphan status. Kuvan net product revenues for the years ended December 31, 2013, 2012 and 2011 totaled $167.4 million, $143.1 million and $116.8 million, respectively.

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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 and PEG PAL in Japan. Merck Serono markets Kuvan in the EU and several other countries outside the U.S., Canada and Japan. Under the agreement with Merck Serono, we are entitled to receive royalties, on a country-by-country basis, until the later of the expiration of patent rights licensed to Merck Serono or ten years after the first commercial sale of the licensed product in such country. Over the next several years, we expect a royalty of approximately four percent on net sales of Kuvan by Merck Serono. We also sell Kuvan to Merck Serono at or near cost, and Merck Serono resells the product to end-users outside the U.S., Canada and Japan. The royalty earned from Kuvan product sold by Merck Serono in the EU is included as a component of net product revenues in the period earned. During 2013, 2012 and 2011 we earned $2.0 million, $1.9 million and $1.6 million, respectively, in net royalties on net sales of $51.0 million, $46.8 million and $40.4 million of Kuvan by Merck Serono, respectively. We recorded collaborative agreement revenue associated with shared Kuvan development costs in the amounts of $1.0 million, $1.8 million, and $0.5 million in 2013, 2012 and 2011, respectively.

Aldurazyme

Aldurazyme has been approved for marketing in the U.S., EU and other countries for patients with mucopolysaccharidosis I (MPS I). MPS I is a progressive and debilitating life-threatening genetic disease, for which no other drug treatment currently exists, that is caused by the deficiency of alpha-L-iduronidase, a lysosomal enzyme normally required for the breakdown of GAGs. Patients with MPS I typically become progressively worse and experience multiple severe and debilitating symptoms resulting from the build-up of carbohydrate residues in all tissues in the body. These symptoms include: inhibited growth, delayed and regressed mental development (in the severe form of the disease), enlarged liver and spleen, joint deformities and reduced range of motion, impaired cardiovascular function, upper airway obstruction, reduced pulmonary function, frequent ear and lung infections, impaired hearing and vision, sleep apnea, malaise and reduced endurance.

We developed Aldurazyme through collaboration with Genzyme, now a wholly-owned subsidiary of Sanofi. Under our collaboration agreement, we are responsible for manufacturing Aldurazyme and supplying it to Genzyme. Genzyme records sales of Aldurazyme and is required to pay us, on a quarterly basis, a 39.5% to 50% royalty on worldwide net product sales. We recognize a portion of this royalty as product transfer revenue when product is released to Genzyme and all of our obligations have been fulfilled. Genzyme s return rights for Aldurazyme are limited to defective product. The product transfer revenue represents the fixed amount per unit of Aldurazyme that Genzyme is required to pay us if the product is unsold by Genzyme. The amount of product transfer revenue will eventually be deducted from the calculated royalty when the product is sold by Genzyme. Additionally, Genzyme and we are members of a 50/50 limited liability company that: (1) holds the intellectual property relating to Aldurazyme and other collaboration products and licenses all such intellectual property on a royalty-free basis to us and Genzyme to allow us to exercise our rights and perform our obligations under the agreements related to the restructuring, and (2) engages in research and development activities that are mutually selected and funded by Genzyme and us.

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

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revenue of $1.8 million, and $8.6 million, in 2012 and 2011, respectively. Incremental/previously recognized product transfer revenue reflects incremental shipments of Aldurazyme to Genzyme to meet future product demand. In the future, to the extent that Genzyme Aldurazyme inventory quantities on hand remain consistent, we expect that our total Aldurazyme revenues will approximate the 39.5% to 50% royalties on net product sales by Genzyme.

Firdapse

Firdapse is a form of 3, 4-diaminopyridine (amifampridine phosphate or 3, 4-DAP) for the treatment of Lambert Myasthenic Syndrome (LEMS). Firdapse was originally developed by AGEPS, the pharmaceutical unit of the Paris Public Hospital Authority (AP-HP). Firdapse was granted marketing approval in the EU in December 2009. In addition, Firdapse has been granted orphan drug status in the EU, which confers ten years of market exclusivity in the EU. We launched Firdapse on a country-by-country basis in Europe beginning in April 2010. Firdapse net product revenues for the years ended December 31, 2013, 2012 and 2011 totaled $16.1 million, $14.2 million and $13.1 million, respectively. In October 2012, we licensed to Catalyst Pharmaceutical Partners, Inc. the North American rights to develop and market Firdapse. In exchange for the North American rights to Firdapse, we may receive royalties of 7% to 10% on net product sales of Firdapse in North America. For the year ended December 31, 2013 we recognized collaborative revenue of $2.9 million related to our agreement with Catalyst.

LEMS is a rare autoimmune disease with the primary symptoms of muscle weakness. Muscle weakness in LEMS is caused by autoantibodies to voltage gated calcium channels leading to a reduction in the amount of acetylcholine released from nerve terminals. The prevalence of LEMS is estimated at four to ten per million, or approximately 2,000 to 5,000 patients in the EU and 1,200 to 3,100 patients in the U.S. Approximately 50% of LEMS patients diagnosed have small cell lung cancer. Patients with LEMS typically present with fatigue, muscle pain and stiffness. The weakness is generally more marked in the proximal muscles particularly of the legs and trunk. Other problems include reduced reflexes, drooping of the eyelids, facial weakness and problems with swallowing. Patients often report a dry mouth, impotence, constipation and feelings of light headedness on standing. On occasion, these problems can be life threatening when the weakness involves respiratory muscles. A diagnosis of LEMS is generally made on the basis of clinical symptoms, electromyography testing and the presence of auto antibodies against voltage gated calcium channels. Currently approved treatments of LEMS can consist of strategies directed at the underlying malignancy, if one is present. Therapy of small cell lung cancer is limited and outcomes are generally poor. Immunosuppressive agents have been tried but success is limited by toxicity and difficulty administering the regimens. A mainstay of therapy has been 3, 4-DAP, but its use in practice has been limited by the drug s availability.

VIMIZIM

VIMIZIM is an enzyme replacement therapy for the treatment of MPS IV A, a lysosomal storage disorder. MPS IV A is a disease characterized by deficient activity of Nacetylgalactosamine- 6-sulfatase (GALNS) causing excessive lysosomal storage of glycosaminoglycans such as keratan sulfate and chondroitin sulfate. This excessive storage causes a systemic skeletal dysplasia, short stature, and joint abnormalities, which limit mobility and endurance. Malformation of the chest impairs respiratory function, and looseness of joints in the neck cause spinal instability and potentially spinal cord compression. Other symptoms may include hearing loss, corneal clouding, and heart disease. Initial symptoms often become evident in the first five years of life. The disease substantially limits both the quality and length of life of those affected. We have identified approximately 1,500 patients worldwide, including approximately 200 patients in the U.S., suffering from MPS IV A and if approved in the EU and other countries, we expect that VIMIZIM could be our largest commercial product to date.

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VIMIZIM was granted marketing approval in the U.S. on February 14, 2014. We immediately began marketing VIMIZIM in the U.S. using our own existing sales force and commercial organization and we completed our first commercial sale in the U.S. Now that we have received approval for VIMIZIM in the U.S., we plan to pursue registration and/or market VIMIZIM on a named patient basis in other regions. Additionally, many countries allow for named patient or other early access sales based on the FDA approval. We plan to institute sales in these countries where appropriate. The EMA has validated the MAA, for VIMIZIM and has recently moved from an accelerated assessment to a standard assessment for this MAA. On February 20, 2014, the CHMP of the EMA adopted a positive opinion for our MAA for VIMIZIM. The EC is expected to render an approval decision for VIMIZIM in the second quarter of 2014.

Products in Clinical Development

PEG PAL

PEG PAL is an investigational enzyme substitution therapy that we are developing as a subcutaneous injection for the treatment of PKU. In June 2009, we announced results from a Phase 1 open-label, single-dose, dose-escalation clinical trial of PEG PAL for PKU. Significant reductions in blood Phe levels were observed in all patients in the fifth dosing cohort of the Phase 1 trial. In addition, there were no serious immune reactions observed and mild to moderate injection-site reactions were in line with our expectations. In September 2009, we initiated a Phase 2, open-label dose finding clinical trial of PEG PAL. The primary objective of this clinical trial was to optimize the dose and schedule that produces the most favorable safety profile and Phe reduction. The secondary objectives of the clinical trial were to evaluate the safety and tolerability of multiple dose levels of PEG PAL, to evaluate the immune response to PEG PAL, and to evaluate steady-state phamacokinetics in all patients and accumulation of PEG PAL in a subset of patients enrolled in this clinical trial. Preliminary results from this clinical trial were presented in August 2010 and showed that of the seven patients who received at least one milligram per kilogram per week of PEG PAL for at least four weeks, six patients have achieved Phe levels below 600 micromoles per liter. Mild to moderate self-limiting injection site reactions are the most commonly reported toxicity. In April 2011, we initiated an extension of the Phase 2 study to find a shorter induction and titration dosing regimen to an efficacious maintenance dose. This study is fully enrolled and ongoing with 24 subjects. A Phase 3 clinical trial of PEG PAL was initiated in May 2013. This Phase 3 clinical trial includes an open-label study to evaluate safety and blood Phe levels in naïve patients and a randomized controlled study of the Phase 2 extension study patients and patients from the open-label trial to evaluate blood Phe levels and neurocognitive endpoints. This ongoing Phase 2 study has enrolled 24 patients to date and has demonstrated Phe reduction using the standard indication period we are using for the Phase 3 study. The FDA has indicated that lowering Phe blood levels in adults could support accelerated approval, even if neurocognitive endpoints are not demonstrated. We expect to report results from these trials in the fourth quarter of 2014.

BMN 673

BMN 673 is a PARP inhibitor, a class of molecules that has shown clinical activity against cancers involving defects in DNA repair that we are investigating for the treatment of certain cancers. In January 2011, we announced the initiation of a Phase 1/2 clinical trial for BMN 673 for the treatment of patients with solid tumors. This clinical trial is an open-label study of once daily, orally administered BMN 673 in approximately 85 patients ages 18 and older with advanced or recurrent solid tumors. The study established a preliminary dose that is generally well-tolerated and reaches steady state with repeated daily doses. The study has focused on breast and ovarian cancers characterized by BRCA mutations, Ewing s sarcoma and small cell lung cancer, and has been expanded to include prostate and pancreatic cancers. In September 2013, we announced an update on the study at the 2013 San Antonio Breast Cancer Symposium. As presented, among 14 enrolled gBRCA breast cancer patients treated at the dose of 1mg/day, the confirmed RECIST response rate was 50% (seven confirmed objective responses: one complete and six partial). In addition, there were five patients with stable disease lasting

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at least 24 weeks for an overall clinical benefit response rate at this dose of 86% (12/14). In the complete cohort of 18 gBRCA breast cancer patients, which included six patients from the dose escalation cohort at doses ranging from 900 µg to 1100 µg and 12 patients from the dose expansion cohort at a dose of 1.0 mg, the RECIST response rate was 44% (8/18), with one complete and seven partial responses. The clinical benefit rate was 72% (13/18), with five patients having stable disease in excess of 24 weeks. At all doses (n=18) there has been a best response of partial response or better in 12 patients, and four patients progressed prior to confirmation. Of the 14 patients treated at 1 mg, there has been a best response of partial response or better in 8 patients, and one patient progressed prior to confirmation. Safety data continues to show that BMN 673 is generally well-tolerated. The dose-limiting toxicity has been thrombocytopenia. Myelosuppression is generally mild-to-moderate in severity. Greater than grade 1 anemia, thrombocytopenia and neutropenia has occurred in 23%, 18% and 11% of patients, respectively, with chronic dosing. Fatigue, nausea and alopecia were observed in 26-31% of patients. Enrollment continues for this study.

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

BMN 701

BMN 701 is a novel fusion of acid alpha glucosidase (GAA) with a peptide derived from insulin-like growth factor 2. We acquired the BMN 701 program in August 2010 in connection with the acquisition of ZyStor Therapeutics, Inc. (ZyStor). In January 2011, we announced the initiation of a Phase 1/2 clinical trial for BMN 701. This clinical trial was an open-label study to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamic and clinical activity of BMN 701 administered as an intravenous infusion every two weeks at doses of 20 milligrams per kilogram. We have completed enrollment of this study with 22 patients between the ages of 13 and 65 years old with late-onset Pompe disease for a treatment period of 24 weeks. The primary objectives of this study are to evaluate the safety and tolerability of BMN 701 as well as determine the antibody response to BMN 701. The secondary objectives of the study are to determine the single and multi-dose pharmacokinetics of BMN 701 and determine mobility and functional exercise capacity in patients receiving BMN 701. Pompe disease is a lysosomal storage disorder caused by a deficiency in GAA that prevents cells from adequately degrading glycogen. This results in the storage of glycogen in lysosomes, particularly those in muscle cells, thereby damaging those cells and causing progressive muscle weakness, which in turn can result in death due to pulmonary or cardiac insufficiency.

Results from the Phase 1/2 clinical trial, released in March 2013, exceeded our prespecified requirements. The results showed that in the 20 mg/kg every other week dose cohort, three out of 16 patients, or 19%, had a greater than 75 meter improvement in 6-minute walk distance, and that there was a 14.1% relative improvement in Maximal Expiratory Pressure (MEP) and a 27.0% relative improvement in Maximal Inspiratory Pressure (MIP) from pretreatment baseline to week 24, two important measures of overall respiratory muscle function and strength. Side effects for BMN 701 were generally consistent with those seen for other enzyme replacement therapies.

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

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BMN 111

BMN 111 is a peptide therapeutic in development for the treatment of achondroplasia. In September 2012, we announced the results of a Phase 1 clinical trial for BMN 111. The primary objective of the Phase 1 clinical trial was to assess the safety and tolerability of single and multiple doses of BMN 111 in normal healthy adult volunteers up to the maximum tolerated dose. BMN 111 was generally well-tolerated over the range of single and repeat doses studied. Pharmacokinetic data indicated that the dose levels studied resulted in exposure levels that are expected to stimulate growth based on non-clinical findings. In January 2014, we announced the initiation of a Phase 2 clinical trial for BMN 111 for the treatment of children with achondroplasia. This clinical trial is an open-label, sequential cohort, dose-escalation study of BMN 111 in children who are 5-14 years old. The primary objective of this study is to assess the safety and tolerability of daily subcutaneous doses of BMN 111 administered for 6 months. The secondary objectives will include an evaluation of change in annualized growth velocity, changes in absolute growth parameters, changes in body proportions and other medically relevant and functional aspects of achondroplasia, such as sleep apnea and joint range of motion. Prior to enrolling in the Phase 2 study, all patients will have participated in a six month natural history study to determine baseline growth velocity data. This is an international study that will enroll approximately 24 subjects for a treatment duration of six months.

BMN 190

BMN 190 is a recombinant human tripeptidyl peptidase 1 for the treatment of patients with CLN2, a form of Batten disease. In September 2013, we announced the initiation of a Phase 1/2 study for BMN 190. This clinical trial is an open-label, dose-escalation study in patients with CLN2. The primary objectives are to evaluate the safety and tolerability of BMN 190 and to evaluate effectiveness using a CLN2-specific rating scale score in comparison with natural history data after 48 weeks of treatment. Secondary objectives are to evaluate the impact of treatment on brain atrophy in comparison with CLN2 natural history after 48 weeks of treatment and to characterize pharmacokinetics and immunogenicity. The study is currently enrolling patients and plans to enroll approximately 22 subjects at up to ten clinical sites worldwide for a treatment duration of 48 weeks.

Company Information

We were incorporated in Delaware in October 1996 and began operations on March 21, 1997. Our principal executive offices are located at 770 Lindaro Street, San Rafael, California 94901 and our telephone number is (415) 506-6700. Our annual reports on Form 10-K, quarterly reports on Form 10-Q, proxy statements, current reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act are available free of charge at *www.bmrn.com* as soon as reasonably practicable after electronically filing such reports with the SEC. Such reports and other information may be obtained by visiting the SEC s Public Reference Room at 100 F Street, NE, Washington, D.C. 20549 or by calling the SEC at 1-800-SEC-0330. Additionally, these reports are available at the SEC s website at *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.

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THE OFFERING

The following is a brief summary of the terms of this offering.

Issuer	BioMarin Pharmaceutical Inc.
Common stock to be offered	1,500,000 shares
Common stock to be outstanding after the offering	144,963,668 shares
Use of proceeds	We intend to apply the net proceeds of this offering to the purchase of our corporate headquarters for $116.5 million, which we expect to close in the first quarter of 2014, and for general corporate purposes. See Prospectus Supplement Summary Recent Developments Contract to Purchase San Rafael Corporate Center. We reserve the right, at the sole discretion of our Board of Directors, to reallocate the proceeds of this offering in response to developments in our business. Accordingly, our management will have significant discretion in applying these proceeds. Until we use the net proceeds of this offering, we intend to invest the funds in short term, interest bearing instruments or other investment grade securities.
NASDAQ symbol for common stock	Our common stock is listed on the NASDAQ Global Select Market under the symbol BMRN.
Risk factors	See Risk Factors and other information included in this prospectus supplement, the accompanying prospectus and the documents incorporated by reference in this prospectus supplement and the accompanying prospectus for a discussion of factors you should carefully consider before deciding to invest in shares of our common stock.

The number of shares of common stock to be outstanding after this offering is based on 143,463,668 shares of common stock outstanding as of December 31, 2013 and does not take into account:

- 13,157,283 shares of our common stock issuable upon exercise of outstanding options issued under our equity incentive plans at a weighted average exercise price of $34.06 per share as of December 31, 2013;
- 1,133,835 shares of our common stock reserved for issuance in connection with service-based restricted stock units at a weighted average grant date fair value of $50.97 per share as of December 31, 2013;
- 860,000 shares of our common stock reserved for issuance in connection with performance and market-based restricted stock units at a weighted average grant date fair value of $34.66 per share as of December 31, 2013;
- 3,047,274 shares of our common stock issuable upon the conversion of our $62.0 million 1.875% convertible subordinated notes due 2017 as of December 31, 2013;

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3,982,988 shares of our common stock issuable upon the conversion of our $375.0 million 0.75% convertible subordinated notes due 2018 as of December 31, 2013;

3,982,988 shares of our common stock issuable upon the conversion of our $375.0 million 1.50% convertible subordinated notes due 2020 as of December 31, 2013; and

an aggregate of 23,202,906 shares of our common stock available for future equity awards under our equity incentive plans as of December 31, 2013.

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RISK FACTORS

An investment in our common stock involves a high degree of risk. We operate in a dynamic and rapidly changing industry that involves numerous risks and uncertainties. You should carefully consider the following risk factors, together with all of the other information contained in this prospectus supplement and the accompanying prospectus or incorporated by reference into this prospectus supplement and the accompanying prospectus. The risks and uncertainties described below are not the only ones we face. Other risks and uncertainties, including those that we do not currently consider material, may impair our business. If any of the risks discussed below actually occur, our business, financial condition, operating results or cash flows could be materially adversely affected. This could cause the value of our common stock to decline, and you may lose all or part of your investment.

Risk Related to Our Business

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

We must obtain and maintain regulatory approval to market and sell our drug products in the U.S. and in jurisdictions outside of the U.S. In the U.S., we must obtain FDA approval for each drug that we intend to commercialize. The FDA approval process is typically lengthy and expensive, and approval is never certain. Products distributed abroad are also subject to government regulation by international regulatory authorities. The approval process in the EU and other countries can also be lengthy and expensive and regulatory approval is also never certain. Naglazyme, Aldurazyme and Kuvan have received regulatory approval to be commercially marketed and sold in the U.S., EU and other countries. Firdapse has received regulatory approval to be commercially marketed only in the EU. VIMIZIM received regulatory approval in the U.S. on February 14, 2014 but has not been approved in the EU or any other jurisdiction and may never receive additional regulatory approvals for any jurisdiction outside of the U.S.

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

In addition, the FDA and its international equivalents have substantial discretion over the approval process for pharmaceutical products. As such, these regulatory agencies may in the end not agree that we have demonstrated the requisite level of product safety and efficacy to grant approval and may require additional data. If we fail to obtain regulatory approval for our product candidates, we will be unable to market and sell those drug products. Because of the risks and uncertainties in pharmaceutical development, our product candidates could take a significantly longer time to gain regulatory approval than we expect or may never gain approval. We also rely on independent third-party contract research organizations (CROs), to file some of our ex-U.S. and ex-EU marketing applications and important aspects of the services performed for us by the CROs are out of our direct control. If we fail to adequately manage our CROs, if the CRO elects to prioritize work on our projects below other projects or if there is any dispute or disruption in our relationship with our CROs, the filing of our applications may be delayed.

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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 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

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same drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process.

We may face competition from biological products approved through an abbreviated regulatory pathway.

Our Naglazyme, Aldurazyme and VIMIZIM products are regulated by the FDA as biologics under the Federal Food, Drug and Cosmetic Act (FDC Act), and the Public Health Service Act (the PHS Act). Biologics require the submission of a Biologics License Application (BLA), and approval by the FDA prior to being marketed in the U.S. Historically, a biologic product approved under a BLA was not subject to the generic drug review and approval provisions of the FDC Act. However, the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the PPACA), created a regulatory pathway under the PHS Act for the abbreviated approval for biological products that are demonstrated to be biosimilar or interchangeable with an FDA-approved biological product. In order to meet the standard of interchangeability, a sponsor must demonstrate that the biosimilar product can be expected to produce the same clinical result as the reference product, and for a product that is administered more than once, that the risk of switching between the reference product and biosimilar product is not greater than the risk of maintaining the patient on the reference product. Such biosimilars would reference biological products approved in the U.S. The law establishes a period of 12 years of data exclusivity for reference products, which protects the data in the original BLA by prohibiting sponsors of biosimilars from gaining FDA approval based in part on reference to data in the original BLA. Our products approved under BLAs, as well as products in development that may be approved under BLAs, could be reference products for such abbreviated BLAs.

To obtain regulatory approval to market our products, preclinical studies and costly and lengthy preclinical and clinical trials are required and the results of the studies and trials are highly uncertain.

As part of the regulatory approval process we must conduct, at our own expense, preclinical studies in the laboratory and clinical trials on humans for each product candidate. We expect the number of preclinical studies and clinical trials that the regulatory authorities will require will vary depending on the product candidate, the disease or condition the drug is being developed to address and regulations applicable to the particular drug. Generally, the number and size of clinical trials required for approval increase based on the expected patient population that may be treated with a drug. We may need to perform multiple preclinical studies using various doses and formulations before we can begin clinical trials, which could result in delays in our ability to market any of our product candidates. Furthermore, even if we obtain favorable results in preclinical studies, the results in humans may be significantly different. After we have conducted preclinical studies, we must demonstrate that our drug products are safe and efficacious for use in the targeted human patients in order to receive regulatory approval for commercial sale.

Adverse or inconclusive clinical results would stop us from filing for regulatory approval of our product candidates. Additional factors that can cause delay or termination of our clinical trials include:

- slow or insufficient patient enrollment;
- slow recruitment of, and completion of necessary institutional approvals at, clinical sites;
- longer treatment time required to demonstrate efficacy;
- lack of sufficient supplies of the product candidate;
- adverse medical events or side effects in treated patients;

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lack of effectiveness of the product candidate being tested; and

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

Typically, if a drug product is intended to treat a chronic disease, as is the case with some of our product candidates, safety and efficacy data must be gathered over an extended period of time, which can range from nine months to three years or more. We also rely on independent third-party CROs to perform most of our clinical studies and many important aspects of the services performed for us by the CROs are out of our direct control. If we fail to adequately manage our CROs, or if there is any dispute or disruption in our relationship with our CROs, our clinical trials may be delayed. Moreover, in our regulatory submissions, we rely on the quality and validity of the clinical work performed by third-party CROs. If any of our CROs processes, methodologies or results were determined to be invalid or inadequate, our own clinical data and results and related regulatory approvals could adversely be impacted.

If we continue to incur operating losses for a period longer than anticipated, we may be unable to continue our operations at planned levels and be forced to reduce our operations.

Since we began operations in March 1997, we have been engaged in very substantial research and development and operated at a net loss until 2008. Although we were profitable in 2008 and 2010, we operated at a net loss in 2009, 2011 and 2012. Based upon our current plan for investments in research and development for existing and new programs, we expect to operate at a net loss for at least the next 12 months. Our future profitability depends on our marketing and selling of Naglazyme, Kuvan, Firdapse and VIMIZIM, the successful continued commercialization of Aldurazyme by Genzyme, the receipt of regulatory approval of our product candidates, our ability to successfully manufacture and market any approved drugs, either by ourselves or jointly with others, our spending on our development programs and the impact of any possible future business development transactions. The extent of our future losses and the timing of profitability are highly uncertain. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

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

Before we can begin commercial manufacture of our products, we or our contract manufacturers, must obtain regulatory approval of our manufacturing facilities, processes and quality systems. In addition, our pharmaceutical manufacturing facilities are continuously subject to inspection by the FDA and international regulatory authorities, before and after product approval. Our manufacturing facilities in the U.S. have been approved by the FDA, the European Commission (EC), and health agencies in other countries for the manufacture of Aldurazyme and Naglazyme. In addition, our third-party manufacturers facilities involved with the manufacture of Naglazyme, Kuvan, Firdapse, Aldurazyme and VIMIZIM have also been inspected and approved by various regulatory authorities. The manufacturing facility located in Shanbally, Cork, Ireland that we purchased in 2011 has not yet been approved by the FDA or the EMA. We intend to make a substantial investment in the build-out of the Shanbally facility in order to manufacture VIMIZIM and other products. If the facility is not ultimately approved by the FDA or the EMA, we will not be able to manufacture VIMIZIM or other products at this facility and we may not be able to meet the anticipated commercial demand for VIMIZIM which would have an adverse effect on our financial results.

Due to the complexity of the processes used to manufacture our products and product candidates, we may be unable to continue to pass or initially pass federal or international regulatory inspections in a cost effective manner. For the same reason, any potential third-party manufacturer of Naglazyme, Kuvan, Aldurazyme, Firdapse and VIMIZIM or our product candidates may be unable to comply with GMP regulations in a cost effective manner and may be unable to initially or continue to pass a federal or international regulatory inspection.

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If we, or third-party manufacturers with whom we contract, are unable to comply with manufacturing regulations, we may be subject to delay of approval of our product candidates, warning or untitled letters, fines, unanticipated compliance expenses, recall or seizure of our products, total or partial suspension of production and/or enforcement actions, including injunctions, and criminal or civil prosecution. These possible sanctions would adversely affect our financial results and financial condition.

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

As of December 31, 2013, we had cash, cash equivalents and short and long-term investments totaling $1,052.4 million and long-term debt obligations of $655.6 million. In October 2013, we completed an offering of senior subordinated convertible notes and received net proceeds of approximately $696.4 million, after deducting commissions, estimated offering expenses payable by us and the purchase of the related capped calls. We will need cash to not only repay the principal amount of the Notes but also the ongoing interest due on the Notes during their term. In addition, we may require additional financing to fund our future operations, including the commercialization of our approved drugs and drug product candidates currently under development, preclinical studies and clinical trials, and potential licenses and acquisitions. We may be unable to raise additional financing, if needed, due to a variety of factors, including our financial condition, the status of our product programs, and the general condition of the financial markets. If we fail to raise any necessary additional financing we may have to delay or terminate some or all of our product development programs and our financial condition and operating results will be adversely affected.

We expect to continue to spend substantial amounts of capital for our operations for the foreseeable future. The amount of capital we will need depends on many factors, including:

- our ability to successfully market and sell Naglazyme, Kuvan, Firdapse and VIMIZIM;
- Genzyme s ability to continue to successfully commercialize Aldurazyme;
- the progress and success of our preclinical studies and clinical trials (including studies and the manufacture of materials);
- the timing, number, size and scope of our preclinical studies and clinical trials;
- the time and cost necessary to obtain regulatory approvals and the costs of post-marketing studies which may be required by regulatory authorities;
- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities;
- the progress of research programs carried out by us;
- our possible achievement of milestones identified in our purchase agreements with the former stockholders of LEAD Therapeutics, Inc., ZyStor, Huxley Pharmaceuticals, Inc., and Zacharon Pharmaceuticals Inc. that trigger related milestone payments;
- any changes made to, or new developments in, our existing collaborative, licensing and other commercial relationships or any new collaborative, licensing and other commercial relationships that we may establish; and

whether our convertible debt is converted to common stock in the future.

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

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

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

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

Due to the complexity of manufacturing our products, we may not be able to manufacture drug products successfully with a commercially viable process or at a scale large enough to support their respective commercial markets or at acceptable margins.

The development of commercially viable manufacturing processes typically is very difficult to achieve and is often very expensive and may require extended periods of time. Changes in manufacturing processes (including manufacturing cell lines), equipment or facilities may require us to complete clinical trials to receive regulatory approval of any manufacturing improvements. Also, we may be required to demonstrate product comparability between a biological product made after a manufacturing change and the product made before implementation of the change through additional types of analytical and functional testing or may have to complete additional clinical studies. If we contract for manufacturing services with an unproven process, our contractor is subject to the same uncertainties, high standards and regulatory controls, and may therefore experience difficulty if further process development is necessary.

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

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

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

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

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

Our manufacturing facility for Naglazyme, Aldurazyme and VIMIZIM is located near known earthquake fault zones, and the occurrence of an earthquake or other catastrophic disaster could cause damage to our facility and equipment, or that of our third-party manufacturers or single-source suppliers, which could materially impair our ability to manufacture Naglazyme, Aldurazyme and VIMIZIM or our third-party manufacturer s ability to manufacture Kuvan or Firdapse.

Our Galli Drive facility located in Novato, California is currently our only manufacturing facility for Naglazyme, Aldurazyme and VIMIZIM. It is located in the San Francisco Bay Area near known earthquake fault zones and is vulnerable to significant damage from earthquakes. We, the third-party manufacturers with whom we contract and our single-source suppliers of raw materials, which include many of our critical raw materials, are also vulnerable to damage from other types of disasters, including fires, floods, power loss and similar events. If any disaster were to occur, or any terrorist or criminal activity caused significant damage to our facilities or the facilities of our third-party manufacturers and suppliers, our ability to manufacture Naglazyme, Aldurazyme and VIMIZIM, or to have Kuvan or Firdapse manufactured, could be seriously, or potentially completely impaired, and our commercialization efforts and revenue could be seriously impaired. The insurance that we carry, the inventory that we maintain and our risk mitigation plans may not be adequate to cover our losses resulting from disasters or other business interruptions.

Supply interruptions may disrupt our inventory levels and the availability of our products and 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.

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

With respect to our product candidates, production of product is necessary to perform clinical trials and successful registration batches are necessary to file for approval to commercially market and sell product candidates. Delays in obtaining clinical material or registration batches could 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 outside of the U.S., we must market worldwide to achieve significant market penetration of the product. In addition, because the number of potential patients in the disease populations are small, it is not only important to find patients who begin therapy to achieve significant market penetration of the product, but we also need to be able to maintain these patients on therapy for an extended period of time. Due to the expected costs of treatment for our products for genetic diseases, we may be unable to maintain or obtain sufficient market share at a price high enough to justify our product development efforts and manufacturing expenses.

If we fail to obtain an adequate level of coverage and reimbursement for our drug products by third-party payers, the sales of our drugs would be adversely affected or there may be no commercially viable markets for our products.

The course of treatment for patients using our products is expensive. We expect patients to need treatment for extended periods, and for some products throughout the lifetimes of the patients. We expect that most families of patients will not be capable of paying for this treatment themselves. There will be no commercially viable market for our products without coverage and reimbursement from third-party payers. Additionally, even if there is a commercially viable market, if the level of reimbursement is below our expectations, our revenue and gross margins will be adversely affected.

Third-party payers, such as government or private health care insurers, carefully review and increasingly challenge the prices charged for drugs. Reimbursement rates from private companies vary depending on the third-party payer, the insurance plan and other factors. Reimbursement systems in international markets vary significantly by country and by region, and reimbursement approvals must be obtained on a country-by-country basis.

Reimbursement in the EU and many other territories must be negotiated on a country-by-country basis and in many countries the product cannot be commercially launched until reimbursement is approved. The timing to complete the negotiation process in each country is highly uncertain, and in some countries we expect that it may exceed 12 months. Even after a price is negotiated, countries frequently request or require adjustments to the price and other concessions over time.

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

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

We make a significant portion of our international sales of Naglazyme through special access or named patient programs, which do not require full product approval. We expect to also utilize these programs for

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VIMIZIM. The specifics of the programs vary from country to country. Generally, special approval must be obtained for each patient. The approval normally requires an application or a lawsuit accompanied by evidence of medical need. Generally, the approvals for each patient must be renewed from time to time.

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

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

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

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

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

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

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

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

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

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

Several provisions of the new law, which have varying effective dates, may affect us and will likely increase certain of our costs. For example, the Medicaid rebate rate was increased and the volume of rebated drugs has been expanded to include beneficiaries in Medicaid managed care organizations. Among other things, the PPACA also expanded the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance; included a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or donut hole, and imposed a new fee on certain manufacturers and importers of branded prescription drugs (excluding orphan drugs under certain conditions). The law also revised the definition of average manufacturer price for reporting purposes, which could increase the amount of the Medicaid drug rebates paid to states.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. These changes include aggregate reductions in Medicare payments to providers of up to 2% per fiscal year, which went into effect on April 1, 2013. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and, accordingly, our financial operations.

We anticipate that PPACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and an additional downward pressure on the reimbursement our customers may receive for our products. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our products.

We face credit risks from customers outside of the U.S. that may adversely affect our results of operations.

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

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

We are subject to various federal and state health care fraud and abuse laws, including anti-kickback laws, false claims laws and laws related to ensuring compliance. The federal health care program anti-kickback statute makes it illegal for any person, including a pharmaceutical company, to knowingly and willfully offer, solicit, pay or receive any remuneration, directly or indirectly, in exchange for or to induce the referral of business, including the purchase, order or prescription of a particular drug, for which payment may be made under federal health care programs, such as Medicare and Medicaid. Under federal government regulations,

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certain arrangements, or safe harbors, are deemed not to violate the federal anti-kickback statute. However, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration not intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor. Our practices may not in all cases meet all of the criteria for safe harbor protection from anti-kickback liability, although we seek to comply with these safe harbors. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs.

Federal and state false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. Under the Health Insurance Portability and Accountability Act of 1996, we also are prohibited from knowingly and willfully executing a scheme to defraud any health care benefit program, including private payers, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for health care benefits, items or services. Sanctions under these federal and state laws may include civil monetary penalties, exclusion of a manufacturer s products from reimbursement under government programs, criminal fines and imprisonment.

Many states have adopted laws similar to the federal anti-kickback statute, some of which apply to referral of patients for health care services reimbursed by any source, not just governmental payers.

Substantial new provisions affecting compliance also have been adopted, which may require us to modify our business practices with health care practitioners. The PPACA, among other things, requires drug manufacturers to collect and report information on payments or transfers of value to physicians and teaching hospitals, as well as investment interests held by physicians and their immediate family members during the preceding calendar year. Failure to submit required information may result in civil monetary penalties. The CMS has issued a final rule that requires manufacturers to begin collecting required information on August 1, 2013 with the first reports due March 31, 2014 (and by the 90th day of each calendar year thereafter) and publication of the reported data in a searchable form on a public website beginning September 30, 2014.

In addition, there has been a recent trend of increased state regulation of payments made to physicians. Certain states mandate implementation of compliance programs, compliance with the Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers and the PhRMA Code on Interactions with Healthcare Professionals, and/or the tracking and reporting of gifts, compensation and other remuneration to physicians. The shifting compliance environment and the need to implement systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that a pharmaceutical manufacturer may violate one or more of the requirements.

While we believe we have structured our business arrangements to comply with these laws, because of the breadth of these laws, the narrowness of available statutory and regulatory exceptions and the increased focus by law enforcement agencies in enforcing such laws, it is possible that some of our business activities could be subject to challenge under one or more of such laws. In addition, recent health care reform legislation has strengthened, these laws. For example, the PPACA, among other things, amends the intent requirement of the federal anti-kickback and criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. Moreover, the PPACA provides that the government may assert that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the False Claims Act. If we are found in violation of one of these laws, we may be subject to criminal, civil or administrative sanctions, including debarment, suspension or exclusion from participation in federal or state health care programs any of which could adversely affect our business, financial condition and results of operation.

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We conduct a significant amount of our sales and operations outside of the U.S., which subjects us to additional business risks that could adversely affect our revenue and results of operations.

A significant portion of the sales of Aldurazyme and Naglazyme and all of the sales of Firdapse are generated from countries other than the U.S. Additionally, we have operations in several European countries, Brazil, other Latin American countries, Turkey and other Asian countries. We expect that we will continue to expand our international operations in the future. International operations inherently subject us to a number of risks and uncertainties, including:

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

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

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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, Firdapse and VIMIZIM. However, these patents and patent applications do not ensure the protection of our intellectual property for a number of reasons, including without limitation the following:

- With respect to pending patent applications, unless and until actually issued, the protective value of these applications is impossible to determine. We do not know whether our patent applications will result in issued patents.

- Competitors may interfere with our patent process in a variety of ways. Competitors may claim that they invented the claimed invention prior to us or that they filed their application for a patent on a claimed invention before we did. Competitors may also claim that we are infringing on their patents and therefore we cannot practice our technology. Competitors may also contest our patents by showing the patent examiner or a court that the invention was not original, was not novel or was obvious, for example. In litigation, a competitor could claim that our issued patents are not valid or are unenforceable for a number of reasons. If a court agrees, we would not be able to enforce that patent. We have no meaningful experience with competitors interfering with or challenging the validity or enforceability of our patents or patent applications.

- Enforcing patents is expensive and may absorb significant time of our management. Management would spend less time and resources on developing products, which could increase our operating expenses and delay product programs. We may not have the financial ability to sustain a patent infringement action, or it may not be financially reasonable to do so.

- Receipt of a patent may not provide much, if any, practical protection. For example, if we receive a patent with a narrow scope, then it will be easier for competitors to design products that do not infringe on our patent

- The recently enacted America Invents Act, which reformed certain patent laws in the U.S., may create additional uncertainty. Among the significant changes are switching from a first-to-invent system to a first-to-file system, and the implementation of new procedures that permit competitors to challenge our patents in the U.S. Patent and Trademark Office after grant.

It is also unclear whether our trade secrets are adequately protected. Our employees, consultants or contractors may unintentionally or willfully disclose trade secrets to competitors. Enforcing a claim that someone else illegally obtained and is using our trade secrets, as with patent litigation, is expensive and time consuming, requires significant resources and the outcome is unpredictable. In addition, courts outside of the U.S. are sometimes less willing to protect trade secrets. Furthermore, our competitors may independently develop equivalent knowledge, methods and know-how, in which case we would not be able to enforce our trade secret rights against such competitors.

If we are unable to protect our intellectual property, third parties could develop competing products, which could adversely affect our revenue and financial results generally.

Competitors and other third parties may have developed intellectual property that could limit our ability to market and commercialize our products and product candidates, if approved.

Similar to us, competitors continually seek intellectual property protection for their technology. Several of our development programs, such as BMN 673, BMN 701, BMN 111 and BMN 270, focus on therapeutic areas

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that have been the subject of extensive research and development by third parties for many years. Due to the amount of intellectual property in our field of technology, we cannot be certain that we do not infringe intellectual property rights of competitors or that we will not infringe intellectual property rights of competitors granted or created in the future. For example, if a patent holder believes our product infringes its patent, the patent holder may sue us even if we have received patent protection for our technology. If someone else claims we infringe its intellectual property, we would face a number of issues, including the following:

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

We may also support and collaborate in research conducted by government organizations, hospitals, universities or other educational institutions. These research partners may be unwilling to grant us any exclusive rights to technology or products derived from these collaborations.

If we do not obtain required licenses or rights, we could encounter delays in our product development efforts while we attempt to design around other patents or may be prohibited from making, using, importing, offering to sell or selling products requiring these licenses or rights. There is also a risk that disputes may arise as to the rights to technology or products developed in collaboration with other parties. If we are not able to resolve such disputes and obtain the licenses or rights we need, we may not be able to develop or market our products.

If our Manufacturing, Marketing and Sales Agreement with Genzyme were terminated, we could be prevented from continuing to commercialize Aldurazyme or our ability to successfully commercialize Aldurazyme would be delayed or diminished.

Either party may terminate the Manufacturing, Marketing and Sales Agreement (the MMS Agreement), between Genzyme and us related to Aldurazyme for specified reasons, including if the other party is in material breach of the MMS Agreement, has experienced a change of control, as such term is defined in the MMS Agreement, or has declared bankruptcy and also is in breach of the MMS Agreement. Although we are not currently in breach of the MMS Agreement, there is a risk that either party could breach the MMS Agreement in the future. Either party may also terminate the MMS Agreement upon one year prior written notice for any reason.

If the MMS Agreement is terminated for breach, the breaching party will transfer its interest in BioMarin/Genzyme LLC (the LLC), to the non-breaching party, and the non-breaching party will pay a specified buyout amount for the breaching party s interest in Aldurazyme and in the LLC. If we are the breaching party, we would lose our rights to Aldurazyme and the related intellectual property and regulatory approvals. If the MMS Agreement is terminated without cause, the non-terminating party would have the option, exercisable for one year, to buy out the terminating party s interest in Aldurazyme and in the LLC at a specified buyout amount.

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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 of the PEG PAL development program, we do not have any right to commercialize PEG PAL outside of the U.S. and Japan or to grant anyone else such rights.

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

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

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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 (ANDAs) for generic versions of branded drugs. We refer to this process as the ANDA process . The ANDA process permits competitor companies to obtain marketing approval for a drug with the same active ingredient for the same uses but does not generally require the conduct and submission of clinical efficacy studies for that product. In place of such clinical studies, an ANDA applicant usually needs only to submit data demonstrating that its product is bioequivalent to the branded product based on pharmacokinetic studies. Pursuant to the Hatch-Waxman Act, companies were able to file an ANDA application for the active ingredient in Kuvan at any time after December 2011. At present, we have not received information that any other party has filed or has conducted the bioequivalency study necessary to file an ANDA for Kuvan.

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

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generic versions of Kuvan would be prohibited until the expiration of orphan drug exclusivity in December 2014 or June 2015 if we receive pediatric exclusivity.

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

If we do not achieve our projected development goals in the timeframes we announce and expect, the commercialization of our products may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

For planning purposes, we estimate the timing of the accomplishment of various scientific, clinical, regulatory and other product development goals, which we sometimes refer to as milestones. These milestones 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 currently maintain insurance against product liability lawsuits for the commercial sale of our products and for the clinical trials of our product candidates. Pharmaceutical companies must balance the cost of insurance with the level of coverage based on estimates of potential liability. Historically, the potential liability associated with product liability lawsuits for pharmaceutical products has been unpredictable. Although we believe that our current insurance is a reasonable estimate of our potential liability and represents a commercially reasonable balancing of the level of coverage as compared to the cost of the insurance, we may be subject to claims in connection with our clinical trials and commercial use of Naglazyme, Kuvan, Aldurazyme, Firdapse and VIMIZIM, or our clinical trials for PEG PAL, BMN 701, BMN 673, BMN 111, BMN 190 or BMN 270 for which our insurance coverage may not be adequate and we may be unable to avoid significant liability if any product liability lawsuit is brought against us. If we are the subject of a successful product liability claim that exceeds the limits of any insurance coverage we obtain, we may incur substantial charges that would adversely affect our earnings and require the commitment of capital resources that might otherwise be available for the development and commercialization of our product programs.

We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cybersecurity incidents, could harm our ability to operate our business effectively.

We rely significantly on our information technology and manufacturing infrastructure to effectively manage and maintain our inventory and internal reports, to manufacture and ship products to customers and to timely invoice them. Any failure, inadequacy or interruption of that infrastructure or security lapse of that technology, including cybersecurity incidents could harm our ability to operate our business effectively. Our ability to manage and maintain our inventory and internal reports, to manufacture and ship our products to customers and timely invoice them depends significantly on our enterprise resource planning, production management and other information systems. Cybersecurity attacks in particular are evolving and include, but are not limited to, malicious software, attempts to gain unauthorized access to data and other electronic security breaches that could lead to disruptions in systems, misappropriation of our confidential or otherwise protected information and corruption of data. Cybersecurity incidents resulting in the failure of our enterprise resource planning system, production management or other systems to operate effectively or to integrate with other systems, or a breach in security or other unauthorized access of these systems, may affect our ability to manage and maintain our inventory and internal reports, and result in delays in product fulfillment and reduced efficiency of our operations. A breach in security, unauthorized access resulting in misappropriation, theft, or sabotage with respect to our proprietary and confidential information, including research or clinical data, could require significant capital investments to remediate and 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, 2013 approximately 4% of our net product revenues were from the Southern European countries of Italy, Spain, Portugal and Greece. Approximately 16% of our total accounts receivable as of December 31, 2013 related to such countries and we have included an allowance for doubtful accounts for certain accounts receivable from Greece. If the financial conditions of these countries continues to decline, a substantial portion of the receivables may be uncollectable, which would mean we would have to provide for additional allowances for doubtful accounts or cease selling products in these countries, either of which could adversely affect our results of operations. Additionally, if one or more of these countries were unable to purchase our products, our revenue would be adversely affected. We also sell our products in other countries that face economic crises and local currency devaluation. Although we have historically collected receivables from customers in those countries, sustained weakness or further deterioration of the local economies and currencies may cause our customers in those countries to be unable to pay for our products with the same negative effect on our operations.

Interest rates and the ability to access credit markets could also adversely affect the ability of our customers/distributors to purchase, pay for and effectively distribute our products. Similarly, these macroeconomic factors could affect the ability of our contract manufacturers, sole-source or single-source suppliers to remain in business or otherwise manufacture or supply product. Failure by any of them to remain a going concern could affect our ability to manufacture products.

Risks Related to this Offering and Ownership of Our Common Stock

Our stock price may be volatile, and an investment in our stock could suffer a decline in value.

Our valuation and stock price since the beginning of trading after our initial public offering have had no meaningful relationship to current or historical earnings, asset values, book value or many other criteria based on conventional measures of stock value. The market price of our common stock will fluctuate due to factors including:

- product sales and profitability of Naglazyme, Aldurazyme, Kuvan, Firdapse and VIMIZIM;
- manufacture, supply or distribution of Naglazyme, Aldurazyme, Kuvan, Firdapse and VIMIZIM;
- progress of our product candidates through the regulatory process and our ability to successfully commercialize any such products that receive regulatory approval;
- results of clinical trials, announcements of technological innovations or new products by us or our competitors;

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government regulatory action affecting our product candidates or our competitors drug products in both the U.S. and non-U.S. countries;

developments or disputes concerning patent or proprietary rights;

general market conditions and fluctuations for the emerging growth and pharmaceutical market sectors;

economic conditions in the U.S. or abroad;

broad market fluctuations in the U.S., the EU or in other parts of the world;

actual or anticipated fluctuations in our operating results; and

changes in company assessments or financial estimates by securities analysts.

In the past, following periods of large price declines in the public market price of a company s securities, securities class action litigation has often been initiated against that company. Litigation of this type could result in substantial costs and diversion of management s attention and resources, which would hurt our business. Any adverse determination in litigation could also subject us to significant liabilities. In addition, our stock price can be materially adversely affected by factors beyond our control, such as disruptions in global financial markets or negative trends in the biotechnology sector of the economy, even if our business is operating well.

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

We are incorporated in Delaware. Certain anti-takeover provisions of Delaware law and our charter documents as currently in effect may make a change in control of our company more difficult, even if a change in control would be beneficial to the stockholders. Our anti-takeover provisions include provisions in our certificate of incorporation providing that stockholders meetings may only be called by our Board of Directors and provisions in our bylaws providing that the stockholders may not take action by written consent and requiring that stockholders that desire to nominate any person for election to our Board of Directors or to make any proposal with respect to business to be conducted at a meeting of our stockholders be submitted in appropriate form to our Secretary within a specified period of time in advance of any such meeting. Additionally, our Board of Directors has the authority to issue shares of preferred stock and to determine the terms of those shares of stock without any further action by our stockholders. The rights of holders of our common stock are subject to the rights of the holders of any preferred stock that may be issued. The issuance of preferred stock could make it more difficult for a third-party to acquire a majority of our outstanding voting stock. Delaware law also prohibits corporations from engaging in a business combination with any holders of 15% or more of their capital stock until the holder has held the stock for three years unless, among other possibilities, our Board of Directors approves the transaction. Our Board of Directors may use these provisions to prevent changes in the management and control of our company. Also, under applicable Delaware law, our Board of Directors may adopt additional anti-takeover measures in the future.

We have broad discretion in the use of the net proceeds from this offering, and we may not use these proceeds effectively.

We intend to apply the net proceeds of this offering to the purchase of our corporate headquarters for $116.5 million, which we expect to close in the first quarter of 2014, and for general corporate purposes. We reserve the right, at the sole discretion of our Board of Directors, to reallocate our use of proceeds in response to developments in our business. Accordingly, our management will have significant discretion in applying these proceeds and could spend the proceeds in ways that do not necessarily improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business or financial condition, cause the price of our common stock to decline and delay product development.

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USE OF PROCEEDS

We expect to receive approximately $ million from the sale of 1,500,000 shares of our common stock in this offering, based on the price set forth on the cover page of this prospectus supplement, after deducting the estimated offering expenses that we are to pay.

We intend to apply the net proceeds of this offering to the purchase of our corporate headquarters for $116.5 million, which we expect to close in the first quarter of 2014, and for general corporate purposes. See Prospectus Supplement Summary Recent Developments Contract to Purchase San Rafael Corporate Center. We reserve the right, at the sole discretion of our Board of Directors, to reallocate our use of proceeds in response to developments in our business. Accordingly, our management will have significant discretion in applying these proceeds. Until we use the net proceeds of this offering, we intend to invest the funds in short term, interest bearing instruments or other investment grade securities.

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PRICE RANGE OF COMMON STOCK

Our common stock is listed on the NASDAQ Global Select Market under the symbol BMRN.

The following table shows the high and low closing sale prices for our common stock as reported by the NASDAQ Global Select Market during the periods indicated:

	High	Low
Year Ended December 31, 2012		
First Quarter	$ 38.34	$ 33.68