

BIOMARIN PHARMACEUTICAL INC
Form 424B5
March 04, 2014
Table of Contents

Filed Pursuant to Rule 424(b)(5)
File Number 333-191604

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.

Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 424B5

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.

Table of Contents**TABLE OF CONTENTS****Prospectus Supplement**

| | Page |
|---|-------------|
| <u>About This Prospectus Supplement</u> | S-1 |
| <u>Cautionary Note Regarding Forward-Looking Statements</u> | S-2 |
| <u>Prospectus Supplement Summary</u> | S-3 |
| <u>The Offering</u> | S-12 |
| <u>Risk Factors</u> | S-14 |
| <u>Use of Proceeds</u> | S-34 |
| <u>Price Range of Common Stock</u> | S-35 |
| <u>Dividend Policy</u> | S-36 |
| <u>Certain Material U.S. Federal Income Tax Considerations For Non-U.S. Holders</u> | S-37 |
| <u>Underwriting</u> | S-41 |
| <u>Legal Matters</u> | S-47 |
| <u>Experts</u> | S-47 |
| <u>Where You Can Find More Information</u> | S-47 |
| <u>Information Incorporated by Reference</u> | S-47 |

Prospectus

| | |
|---|----|
| <u>About this Prospectus</u> | 1 |
| <u>BioMarin Pharmaceutical Inc.</u> | 3 |
| <u>Risk Factors</u> | 4 |
| <u>Cautionary Note Regarding Forward-Looking Statements</u> | 5 |
| <u>Where You Can Find More Information</u> | 6 |
| <u>Information Incorporated by Reference</u> | 7 |
| <u>Ratio of Earnings to Fixed Charges</u> | 9 |
| <u>Use of Proceeds</u> | 9 |
| <u>General Description of Securities</u> | 10 |
| <u>Description of Capital Stock</u> | 11 |
| <u>Description of Debt Securities</u> | 12 |
| <u>Plan of Distribution</u> | 21 |
| <u>Legal Matters</u> | 23 |
| <u>Experts</u> | 23 |

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.

Table of Contents

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](#) .

S-1

Table of Contents

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 intended to identify 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

Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 424B5

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.

S-2

Table of Contents

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-acetylglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo B syndrome or Mucopolysaccharidosis type IIIB (MPS IIIB).

Table of Contents**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.

S-4

Table of Contents

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-acetylglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo B syndrome or Mucopolysaccharidosis type IIIB (MPS IIIB). We 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. BMN 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.

Table of Contents

Commercial Products

Naglazyme

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

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

Kuvan

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

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

Table of Contents

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

Table of Contents

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 N-acetylgalactosamine-6-sulfatase (GALNS) causing excessive lysosomal storage of glycosaminoglycans such as keratan sulfate and chondroitin sulfate. This excessive storage causes a systemic skeletal dysplasia, short stature, and joint abnormalities, which limit mobility and endurance. Malformation of the chest impairs respiratory function, and looseness of joints in the neck cause spinal instability and potentially spinal cord compression. Other symptoms may include hearing loss, corneal clouding, and heart disease. Initial symptoms often become evident in the first five years of life. The disease substantially limits both the quality and length of life of those affected. We have identified approximately 1,500 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.

Table of Contents

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 pharmacokinetics 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

Table of Contents

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.

Table of Contents

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

Table of Contents

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;

S-12

Table of Contents

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.

S-13

Table of Contents

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.

Table of Contents

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

Table of Contents

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;

Table of Contents

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.

Table of Contents

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

S-18

Table of Contents

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.

Table of Contents

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.

Table of Contents

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

Table of Contents

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.

Table of Contents

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

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

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

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

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

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

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

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

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

Table of Contents

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.

Table of Contents

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

A significant portion of the sales of Aldurazyme and Naglazyme and all of the sales of Firdapse are generated from countries other than the 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

S-25

Table of Contents

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

Table of Contents

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.

Table of Contents

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.

Table of Contents

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,

Table of Contents

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.

Table of Contents

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.

Table of Contents

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;

Table of Contents

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.

Table of Contents

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.

S-34

Table of Contents**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 |
| Second Quarter | \$ 39.58 | \$ 32.13 |
| Third Quarter | \$ 43.30 | \$ 37.02 |
| Fourth Quarter | \$ 50.17 | \$ 36.78 |
| Year Ended December 31, 2013 | | |
| First Quarter | \$ 62.39 | \$ 51.56 |
| Second Quarter High | \$ 70.30 | \$ 54.72 |
| Third Quarter | \$ 78.39 | \$ 58.64 |
| Fourth Quarter | \$ 75.92 | \$ 59.30 |
| Year Ending December 31, 2014 | | |
| First Quarter (through March 3, 2014) | \$ 83.28 | \$ 64.99 |

The last reported sale price of our common stock on the NASDAQ Global Select Market on March 3, 2014 was \$79.72 per share. As of December 31, 2013, there were 54 holders of record of our common stock.

Table of Contents

DIVIDEND POLICY

We have never declared or paid any dividends on our capital stock. We currently intend to retain any future earnings to finance operations and the expansion of our business and do not intend to declare or pay cash dividends on our capital stock in the foreseeable future. Any future determination to pay dividends will be at the discretion of our Board of Directors and will depend upon our results of operations, financial condition, current and anticipated cash needs, contractual restrictions, restrictions imposed by applicable law and other factors that our Board of Directors deems relevant.

S-36

Table of Contents

CERTAIN MATERIAL U.S. FEDERAL INCOME TAX CONSIDERATIONS FOR NON-U.S. HOLDERS

The following is a summary of certain material U.S. federal income tax considerations relating to the purchase, ownership and disposition of our common stock applicable to non-U.S. holders as we define that term below. This discussion is not a complete analysis of all of the potential U.S. federal income tax consequences relating thereto, nor does it address any tax consequences arising under any state, local or foreign tax laws, or any other U.S. federal tax laws. This summary is based upon the provisions of the Internal Revenue Code of 1986, as amended (the Code), Treasury regulations promulgated thereunder, administrative rulings and judicial decisions, all as of the date hereof. These authorities may be changed, possibly retroactively, so as to result in U.S. federal income tax consequences different from those set forth below. We have not sought any ruling from the Internal Revenue Service (the IRS) with respect to the statements made and the conclusions reached in the following summary, and there can be no assurance that the IRS will agree with such statements and conclusions. The term non-U.S. holder means a beneficial owner of our common stock that, for U.S. federal income tax purposes, is not any entity taxable as a partnership, or any of the following:

an individual who is a citizen or resident of the U.S.;

a corporation or other entity taxable as a corporation for U.S. federal income tax purposes created or organized in the U.S. or under the laws of the U.S., any state thereof, or the District of Columbia or otherwise treated as such for U.S. federal income tax purposes;

an estate, the income of which is subject to U.S. federal income taxation regardless of its source; or

a trust that (1) is subject to the primary supervision of a U.S. court and the control of one or more U.S. persons or (2) has a valid election in effect under applicable Treasury regulations to be treated as a U.S. person.

This summary is limited to non-U.S. holders who purchase shares of our common stock issued pursuant to this offering and who hold our common stock as a capital asset within the meaning of Section 1221 of the Code (generally, property held for investment). In addition, this discussion does not address the impact of the Medicare contribution tax on net investment income or tax considerations applicable to an investor's particular circumstances or to investors that may be subject to special tax rules, including, without limitation:

banks, insurance companies, or other financial institutions;

persons subject to the alternative minimum tax or the net investment income tax;

tax-exempt organizations;

dealers in securities or currencies;

traders in securities that elect to use a mark-to-market method of accounting for their securities holdings;

controlled foreign corporations, passive foreign investment companies or corporations that accumulate earnings to avoid U.S. federal income tax;

Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 424B5

persons that are partnerships or other pass-through entities or partners or members of such entities;

certain former citizens or long-term residents of the U.S.; or

persons who hold our common stock as part of a hedge, straddle, constructive sale, or conversion transaction.

S-37

Table of Contents

YOU ARE URGED TO CONSULT YOUR TAX ADVISOR WITH RESPECT TO THE APPLICATION OF THE U.S. FEDERAL INCOME TAX LAWS TO YOUR PARTICULAR SITUATION, AS WELL AS ANY TAX CONSEQUENCES OF THE PURCHASE, OWNERSHIP AND DISPOSITION OF OUR COMMON STOCK ARISING UNDER THE FEDERAL ESTATE OR GIFT TAX RULES OR UNDER THE LAWS OF ANY STATE, LOCAL, FOREIGN OR OTHER TAXING JURISDICTION OR UNDER ANY APPLICABLE TAX TREATY.

Distributions on Common Stock

If we make cash or other property distributions on our common stock, such distributions will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. Distributions in excess of our earnings and profits will constitute a return of capital that will first be applied against and reduce the non-U.S. holder's adjusted tax basis in our common stock, but not below zero. Any remaining excess will be treated as gain realized on the sale or other disposition of the common stock and will be treated as described under "Gain on Disposition of Common Stock" below.

Dividends paid to a non-U.S. holder that are not effectively connected with the non-U.S. holder's conduct of a trade or business in the U.S. will generally be subject to withholding of U.S. federal income tax at the rate of 30%, or if a tax treaty applies, a lower rate specified by the treaty. Non-U.S. holders should consult their tax advisers regarding their entitlement to benefits under a relevant income tax treaty.

Dividends that are effectively connected with a non-U.S. holder's conduct of a trade or business in the U.S. and, if an income tax treaty applies, are attributable to a permanent establishment in the U.S., are generally exempt from withholding and will be taxed on a net income basis at the same graduated U.S. federal income tax rates applicable to a U.S. person, as defined under the Code. In such cases, we will not have to withhold U.S. federal income tax if the non-U.S. holder complies with applicable certification requirements. In addition, if the non-U.S. holder is a corporation, a branch profits tax equal to 30% (or lower applicable treaty rate) may be imposed on a portion of its effectively connected earnings and profits for the taxable year. Non-U.S. holders should consult their tax advisors regarding any applicable tax treaties that may provide for different rules.

To claim the benefit of a tax treaty or an exemption from withholding because the dividends are effectively connected with the conduct of a trade or business in the U.S., a non-U.S. holder must either (a) provide a properly executed IRS Form W-8BEN or Form W-8ECI (as applicable) before the payment of dividends or (b) if our common stock is held through certain foreign intermediaries, satisfy the relevant certification requirements of applicable U.S. Treasury regulations. These forms may need to be periodically updated. Non-U.S. holders may obtain a refund of any excess amounts withheld by timely filing an appropriate claim for refund with the IRS.

Gain on Disposition of Common Stock

A non-U.S. holder generally will not be subject to U.S. federal income tax or any withholding thereof with respect to gain recognized on a sale or other disposition of our common stock unless one of the following applies:

the gain is effectively connected with the non-U.S. holder's conduct of a trade or business in the U.S. and, if an income tax treaty applies, is attributable to a permanent establishment maintained by the non-U.S. holder in the U.S.; in these cases, the non-U.S. holder will generally be taxed on its net gain derived from the disposition at the same graduated U.S. federal income tax rates applicable to a U.S. person and, if the non-U.S. holder is a foreign corporation, the branch profits tax described above may also apply;

Table of Contents

the non-U.S. holder is a non-resident individual who is present in the U.S. for 183 days or more in the taxable year of the disposition and meets certain other requirements; in this case, the non-U.S. holder will be subject to U.S. federal income tax at a rate of 30% (or a reduced rate under an applicable treaty) on the amount by which capital gains (including gain recognized on a sale or other disposition of our common stock) allocable to U.S. sources exceed capital losses allocable to U.S. sources (provided that the non-U.S. holder has timely filed U.S. income tax returns with respect to such losses); or

our common stock constitutes a United States real property interest by reason of our status as a United States real property holding corporation, or USRPHC, for U.S. federal income tax purposes at any time during the shorter of the 5-year period ending on the date you dispose of our common stock or the period you held our common stock. The determination of whether we are a USRPHC depends on the fair market value of our U.S. real property interests relative to the fair market value of our other business assets. We believe that we currently are not and do not anticipate becoming a USRPHC.

Information Reporting and Backup Withholding

We must report annually to the IRS the amount of dividends or other distributions we pay to you on your shares of common stock and the amount of tax we withhold on these distributions regardless of whether withholding is required. The IRS may make copies of the information returns reporting those distributions and amounts withheld available to the tax authorities in the country in which you reside pursuant to the provisions of an applicable income tax treaty or exchange of information treaty. Backup withholding tax may also apply to payments made to a non-U.S. holder on or with respect to our common stock, unless the non-U.S. holder certifies as to its status as a non-U.S. holder under penalties of perjury or otherwise establishes an exemption, and certain other conditions are satisfied. Notwithstanding the foregoing, backup withholding may apply if either we or our paying agent has actual knowledge, or reason to know, that the holder is a U.S. person that is not an exempt recipient.

Information reporting and backup withholding generally are not required with respect to the amount of any proceeds from the sale of your shares of common stock outside the U.S. through a foreign office of a foreign broker that does not have certain specified connections to the U.S. However, if you sell your shares of common stock through a U.S. broker or the U.S. office of a foreign broker, the broker will be required to report to the IRS the amount of proceeds paid to you and also perform backup withholding on that amount unless you provide appropriate certification to the broker of your status as a non-U.S. holder or you otherwise establish an exemption. Information reporting will also apply if you sell your shares of common stock through a foreign broker deriving more than a specified percentage of its income from U.S. sources or having certain other connections to the U.S., unless such broker has documenting evidence in its records that you are a non-U.S. holder and certain other conditions are met or you otherwise establish an exemption.

Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder will be allowed as a refund or a credit against such non-U.S. holder's U.S. federal income tax liability, if any, provided that the required information is timely furnished to the IRS. Non-U.S. holders should consult their own tax advisors regarding the filing of a U.S. tax return for claiming a refund of such backup withholding.

Foreign Account Tax Compliance Act

Legislation enacted in 2010 and existing guidance issued thereunder will require, after June 30, 2014, withholding at a rate of 30% on dividends in respect of, and, after December 31, 2016, gross proceeds from the sale of, our common stock held by or through certain foreign financial institutions (as specially defined for purposes of these rules, including investment funds), unless such institution enters into an agreement with the

Table of Contents

Treasury to report, on an annual basis, information with respect to interests in, and accounts maintained by, the institution to the extent such interests or accounts are held by certain U.S. persons and by certain non-U.S. entities that are wholly or partially owned by U.S. persons and to withhold on certain payments or otherwise qualifies for an exemption from these rules. An intergovernmental agreement between the U.S. and an applicable foreign country, or future Treasury regulations or other guidance, may modify these requirements. Accordingly, the entity through which our common stock is held will affect the determination of whether such withholding is required. Similarly, dividends in respect of, and gross proceeds from the sale of, our common stock held by an investor that is a non-financial foreign entity (as specially defined for purposes of these rules) that does not qualify under certain exemptions will be subject to withholding at a rate of 30%, unless such entity either (i) certifies to us that such entity does not have any substantial United States owners or (ii) provides certain information regarding the entity's substantial United States owners, which we will in turn provide to the IRS. We will not pay any additional amounts to non-U.S. holders in respect of any amounts withheld. Non-U.S. holders are encouraged to consult their tax advisors regarding the possible implications of the legislation on their investment in our common stock.

THE SUMMARY OF MATERIAL U.S. FEDERAL INCOME TAX CONSEQUENCES ABOVE IS INCLUDED FOR GENERAL INFORMATION PURPOSES ONLY. POTENTIAL PURCHASERS OF OUR COMMON STOCK ARE URGED TO CONSULT THEIR TAX ADVISORS TO DETERMINE THE U.S. FEDERAL, STATE, LOCAL AND FOREIGN TAX CONSIDERATIONS OF PURCHASING, OWNING AND DISPOSING OF OUR COMMON STOCK.

Table of Contents

UNDERWRITING

Merrill Lynch, Pierce, Fenner & Smith Incorporated is acting as underwriter of this offering. Subject to the terms and conditions set forth in an underwriting agreement between us and the underwriter, we have agreed to sell to the underwriter, and the underwriter has agreed, to purchase from us, 1,500,000 shares of our common stock.

Subject to the terms and conditions set forth in the underwriting agreement, the underwriter has agreed to purchase all of the shares sold under the underwriting agreement if any of these shares are purchased.

We have agreed to indemnify the underwriter against certain liabilities, including liabilities under the Securities Act, or to contribute to payments the underwriter may be required to make in respect of those liabilities.

The underwriter is offering the shares, subject to prior sale, when, as and if issued to and accepted by it, subject to approval of legal matters by its counsel, including the validity of the shares, and other conditions contained in the underwriting agreement, such as the receipt by the underwriter of officer's certificates and legal opinions. The underwriter reserves the right to withdraw, cancel or modify offers to the public and to reject orders in whole or in part.

Commissions and Discounts

The underwriter is purchasing the shares of common stock from us at \$ per share (representing approximately \$ aggregate proceeds to us, before we deduct our out-of-pocket expenses of approximately \$250,000. 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. In connection with the sale of the shares of common stock offered hereby, the underwriter may be deemed to have received compensation in the form of underwriting discounts. The underwriter may effect such transactions by selling shares of common stock to or through dealers, and such dealers may receive compensation in the form of discounts, concessions or commissions from the underwriter and / or purchasers of shares of common stock for whom it may act as agents or to whom it may sell as principal.

No Sales of Similar Securities

We and our executive officers and directors have agreed, with certain limited exceptions, not to sell or transfer any of our common stock or any securities convertible into or exercisable or exchangeable for our common stock for 45 days after the date of this prospectus supplement without first obtaining the prior written consent of Merrill Lynch, Pierce, Fenner & Smith Incorporated. Specifically, we and these individuals have agreed not to directly or indirectly:

offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant for the sale of, or otherwise dispose of or transfer any shares of our common stock or any securities convertible into or exchangeable or exercisable for our common stock;

file, or cause to be filed, any registration statement under the Securities Act related to our common stock or any securities convertible into or exchangeable or exercisable for our common stock; or

enter into any swap or other agreement or transaction that transfers, in whole or in part, directly or indirectly, the economic consequence of ownership of any of our common stock or any securities convertible into or exchangeable or exercisable for our common stock, whether any such swap or transaction is to be settled by delivery of shares of our common stock or other securities, in cash or otherwise.

Table of Contents

These lock-up provisions apply to our common stock and to securities convertible into or exchangeable or exercisable for or repayable with our common stock. They also apply to common stock owned now or acquired later by the person executing the agreement or for which the person executing the agreement later acquires the power of disposition. In the event that either (x) during the last 17 days of the lock-up period referred to above, we issue an earnings release or material news or a material event relating to us occurs or (y) prior to the expiration of the lock-up period, we announce that we will release earnings results or become aware that material news or a material event will occur during the 16-day period beginning on the last day of the lock-up period, the restrictions described above shall continue to apply until the expiration of the 18-day period beginning on the issuance of the earnings release or the occurrence of the material news or material event.

NASDAQ Global Select Market Listing

The shares are listed on the NASDAQ Global Select Market under the symbol BMRN.

Short Positions

In connection with this offering, the underwriter may purchase and sell our common stock in the open market. These transactions may include short sales, purchases on the open market to cover positions created by short sales. Short sales involve the sale by the underwriter of a greater number of shares than it is required to purchase in this offering. The underwriter must close out any naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriter is concerned that there may be downward pressure on the price of our common stock in the open market after pricing that could adversely affect investors who purchase in this offering.

Similar to other purchase transactions, the underwriter's purchases to cover its short sales may have the effect of raising or maintaining the market price of our common stock or preventing or retarding a decline in the market price of our common stock. As a result, the price of our common stock may be higher than the price that might otherwise exist in the open market. The underwriter may conduct these transactions on the NASDAQ Global Select Market, in the over-the-counter market or otherwise.

Neither we nor the underwriter make any representation or prediction as to the direction or magnitude of any effect that the transactions described above may have on the price of our common stock. In addition, neither we nor the underwriter make any representation that the underwriter will engage in these transactions or that these transactions, once commenced, will not be discontinued without notice.

Passive Market Making

In connection with this offering, the underwriter may engage in passive market making transactions in the common stock on the NASDAQ Global Select Market in accordance with Rule 103 of Regulation M under the Exchange Act during a period before the commencement of offers or sales of common stock and extending through the completion of distribution. A passive market maker must display its bid at a price not in excess of the highest independent bid of that security. However, if all independent bids are lowered below the passive market maker's bid, that bid must then be lowered when specified purchase limits are exceeded. Passive market making may cause the price of our common stock to be higher than the price that otherwise would exist in the open market in the absence of those transactions. The underwriter is not required to engage in passive market making and may end passive market making activities at any time.

Electronic Distribution

In connection with this offering, the underwriter may distribute prospectuses by electronic means, such as e-mail.

Table of Contents

Other Relationships

The underwriter and its affiliates have engaged in, and may in the future engage in, investment banking and other commercial dealings in the ordinary course of business with us or our affiliates. The underwriter has received, or may in the future receive, customary fees and commissions for these transactions.

In addition, in the ordinary course of their business activities, the underwriter and its affiliates may make or hold a broad array of investments and actively trade debt and equity securities (or related derivative securities) and financial instruments (including bank loans) for their own account and for the accounts of their customers. Such investments and securities activities may involve securities and/or instruments of ours or our affiliates. The underwriter and its affiliates may also make investment recommendations and/or publish or express independent research views in respect of such securities or financial instruments and may hold, or recommend to clients that they acquire, long and/or short positions in such securities and instruments.

Notice to Prospective Investors in the European Economic Area

In relation to each Member State of the European Economic Area (each, a Relevant Member State), no offer of shares may be made to the public in that Relevant Member State other than:

- A. to any legal entity which is a qualified investor as defined in the Prospectus Directive;
- B. to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150, natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the underwriter; or
- C. in any other circumstances falling within Article 3(2) of the Prospectus Directive, provided that no such offer of shares shall require the Company or the underwriter to publish a prospectus pursuant to Article 3 of the Prospectus Directive or supplement a prospectus pursuant to Article 16 of the Prospectus Directive.

Each person in a Relevant Member State who initially acquires any shares or to whom any offer is made will be deemed to have represented, acknowledged and agreed that it is a qualified investor within the meaning of the law in that Relevant Member State implementing Article 2(1)(e) of the Prospectus Directive. In the case of any shares being offered to a financial intermediary as that term is used in Article 3(2) of the Prospectus Directive, each such financial intermediary will be deemed to have represented, acknowledged and agreed that the shares acquired by it in the offer have not been acquired on a non-discretionary basis on behalf of, nor have they been acquired with a view to their offer or resale to, persons in circumstances which may give rise to an offer of any shares to the public other than their offer or resale in a Relevant Member State to qualified investors as so defined or in circumstances in which the prior consent of the underwriter has been obtained to each such proposed offer or resale.

The Company, the underwriter and their affiliates will rely upon the truth and accuracy of the foregoing representations, acknowledgements and agreements.

This prospectus supplement has been prepared on the basis that any offer of shares in any Relevant Member State will be made pursuant to an exemption under the Prospectus Directive from the requirement to publish a prospectus for offers of shares. Accordingly any person making or intending to make an offer in that Relevant Member State of shares which are the subject of the offering contemplated in this prospectus supplement may only do so in circumstances in which no obligation arises for the Company or the underwriter to publish a prospectus pursuant to Article 3 of the Prospectus Directive in relation to such offer. Neither the Company nor the underwriter has authorized, nor do they authorize, the making of any offer of shares in circumstances in which an obligation arises for the Company or the underwriter to publish a prospectus for such offer.

Table of Contents

For the purpose of the above provisions, the expression "an offer to the public" in relation to any shares in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and the shares to be offered so as to enable an investor to decide to purchase or subscribe the shares, as the same may be varied in the Relevant Member State by any measure implementing the Prospectus Directive in the Relevant Member State and the expression "Prospectus Directive" means Directive 2003/71/EC (including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member States) and includes any relevant implementing measure in the Relevant Member State and the expression "2010 PD Amending Directive" means Directive 2010/73/EU.

Notice to Prospective Investors in the United Kingdom

In addition, in the United Kingdom, this document is being distributed only to, and is directed only at, and any offer subsequently made may only be directed at persons who are "qualified investors" (as defined in the Prospectus Directive) (i) who have professional experience in matters relating to investments falling within Article 19 (5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, as amended (the Order) and/or (ii) who are high net worth companies (or persons to whom it may otherwise be lawfully communicated) falling within Article 49(2)(a) to (d) of the Order (all such persons together being referred to as "relevant persons"). This document must not be acted on or relied on in the United Kingdom by persons who are not relevant persons. In the United Kingdom, any investment or investment activity to which this document relates is only available to, and will be engaged in with, relevant persons.

Notice to Prospective Investors in Australia

No placement document, prospectus, product disclosure statement or other disclosure document has been lodged with the Australian Securities and Investments Commission (ASIC), in relation to this offering. This prospectus supplement does not constitute a prospectus, product disclosure statement or other disclosure document under the Corporations Act 2001 (the Corporations Act), and does not purport to include the information required for a prospectus, product disclosure statement or other disclosure document under the Corporations Act.

Any offer in Australia of the shares may only be made to persons (the Exempt Investors) who are "sophisticated investors" (within the meaning of section 708(8) of the Corporations Act), "professional investors" (within the meaning of section 708(11) of the Corporations Act) or otherwise pursuant to one or more exemptions contained in section 708 of the Corporations Act so that it is lawful to offer the shares without disclosure to investors under Chapter 6D of the Corporations Act.

The shares applied for by Exempt Investors in Australia must not be offered for sale in Australia in the period of 12 months after the date of allotment under this offering, except in circumstances where disclosure to investors under Chapter 6D of the Corporations Act would not be required pursuant to an exemption under section 708 of the Corporations Act or otherwise or where the offer is pursuant to a disclosure document which complies with Chapter 6D of the Corporations Act. Any person acquiring shares must observe such Australian on-sale restrictions.

This prospectus supplement contains general information only and does not take account of the investment objectives, financial situation or particular needs of any particular person. It does not contain any securities recommendations or financial product advice. Before making an investment decision, investors need to consider whether the information in this prospectus supplement is appropriate to their needs, objectives and circumstances, and, if necessary, seek expert advice on those matters.

Notice to Prospective Investors in Hong Kong

The shares have not been offered or sold and will not be offered or sold in Hong Kong, by means of any document, other than (a) to "professional investors" as defined in the Securities and Futures Ordinance

Table of Contents

(Cap. 571) of Hong Kong and any rules made under that Ordinance; or (b) in other circumstances which do not result in the document being a prospectus as defined in the Companies Ordinance (Cap. 32) of Hong Kong or which do not constitute an offer to the public within the meaning of that Ordinance. No advertisement, invitation or document relating to the securities has been or may be issued or has been or may be in the possession of any person for the purposes of issue, whether in Hong Kong or elsewhere, which is directed at, or the contents of which are likely to be accessed or read by, the public of Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to securities which are or are intended to be disposed of only to persons outside Hong Kong or only to professional investors as defined in the Securities and Futures Ordinance and any rules made under that Ordinance.

Notice to Prospective Investors in Japan

The shares have not been and will not be registered under the Financial Instruments and Exchange Law of Japan (Law No. 25 of 1948, as amended) and, accordingly, will not be offered or sold, directly or indirectly, in Japan, or for the benefit of any Japanese Person or to others for re-offering or resale, directly or indirectly, in Japan or to any Japanese Person, except in compliance with all applicable laws, regulations and ministerial guidelines promulgated by relevant Japanese governmental or regulatory authorities in effect at the relevant time. For the purposes of this paragraph, Japanese Person shall mean any person resident in Japan, including any corporation or other entity organized under the laws of Japan.

Notice to Prospective Investors in Singapore

This prospectus supplement has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus supplement and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of Non-CIS Securities may not be circulated or distributed, nor may the Non-CIS Securities be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore (SFA), (ii) to a relevant person pursuant to Section 275(1), or any person pursuant to Section 275(1A), and in accordance with the conditions specified in Section 275, of the SFA, or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the Non-CIS Securities are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

- (a) a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or
- (b) a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary of the trust is an individual who is an accredited investor,

securities (as defined in Section 239(1) of the SFA) of that corporation or the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferred within six months after that corporation or that trust has acquired the Non-CIS Securities pursuant to an offer made under Section 275 of the SFA except:

- (a) to an institutional investor or to a relevant person defined in Section 275(2) of the SFA, or to any person arising from an offer referred to in Section 275(1A) or Section 276(4)(i)(B) of the SFA;
- (b) where no consideration is or will be given for the transfer;
- (c) where the transfer is by operation of law;

Table of Contents

(d) as specified in Section 276(7) of the SFA; or

(e) as specified in Regulation 32 of the Securities and Futures (Offers of Investments) (Shares and Debentures) Regulations 2005 of Singapore.

Notice to Prospective Investors in Switzerland

The shares may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange (SIX) or on any other stock exchange or regulated trading facility in Switzerland. This document has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this document nor any other offering or marketing material relating to the shares or this offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this document nor any other offering or marketing material relating to this offering, the Company, the shares have been or will be filed with or approved by any Swiss regulatory authority. In particular, this document will not be filed with, and the offer of shares will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA (FINMA), and the offer of shares has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes (CISA). The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of shares.

Notice to Prospective Investors in the Dubai International Financial Centre

This prospectus supplement relates to an Exempt Offer in accordance with the Offered Securities Rules of the Dubai Financial Services Authority (DFSA). This prospectus supplement is intended for distribution only to persons of a type specified in the Offered Securities Rules of the DFSA. It must not be delivered to, or relied on by, any other person. The DFSA has no responsibility for reviewing or verifying any documents in connection with Exempt Offers. The DFSA has not approved this prospectus supplement nor taken steps to verify the information set forth herein and has no responsibility for this prospectus supplement. The shares to which this prospectus supplement relates may be illiquid and/or subject to restrictions on their resale. Prospective purchasers of the shares offered should conduct their own due diligence on the shares. If you do not understand the contents of this prospectus supplement you should consult an authorized financial advisor.

Table of Contents

LEGAL MATTERS

Certain legal matters relating to the issuance of the shares offered by this prospectus supplement will be passed upon for us by Paul Hastings LLP, San Francisco, California. Latham & Watkins LLP, Costa Mesa, California, is counsel to the underwriter in connection with this offering.

EXPERTS

The consolidated financial statements of BioMarin Pharmaceutical Inc. and subsidiaries as of December 31, 2013 and 2012, and for each of the years in the three-year period ended December 31, 2013, and management's assessment of the effectiveness of internal control over financial reporting as of December 31, 2013 have been incorporated by reference herein and in the registration statement in reliance upon the reports of KPMG LLP, independent registered public accounting firm, incorporated by reference herein, and upon the authority of said firm as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We are subject to the information reporting requirements of the Exchange Act, and we file annual, quarterly and special reports, proxy statements and other information with the SEC relating to our business, financial results and other matters. The reports, proxy statements and other information we file may be inspected and copied at prescribed rates at the SEC's Public Reference Room and via the SEC's website (see below for more information).

This prospectus supplement and the accompanying prospectus are part of a registration statement on Form S-3 that we filed under the Securities Act with the SEC. This prospectus supplement and the accompanying prospectus do not contain all of the information included in that registration statement and its accompanying exhibits and schedules. For further information with respect to our securities and us, you should refer to that registration statement and its accompanying exhibits and schedules. Statements in this prospectus supplement and the accompanying prospectus concerning any document that we filed as an exhibit to the registration statement or that we otherwise filed with the SEC are not intended to be comprehensive and are qualified by reference to these filings. You should review the complete document to evaluate these statements.

You may inspect a copy of the registration statement of which this prospectus supplement is a part and its accompanying exhibits and schedules, as well as the reports, proxy statements and other information we file with the SEC, without charge at the SEC's Public Reference Room, 100 F Street, N.E., Room 1580, Washington, D.C. 20549, and you may obtain copies of all or any part of the registration statement from those offices for a fee. You may obtain information on the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330. The SEC maintains a web site that contains reports, proxy and information statements and other information regarding registrants that file electronically, including us. The address of the site is www.sec.gov.

INFORMATION INCORPORATED BY REFERENCE

The SEC allows us to incorporate by reference into this prospectus supplement the information contained in the documents we file with the SEC, which means that we can disclose important information to you by referring you to those documents. The information incorporated by reference is considered to be part of this prospectus supplement, and later information that we file with the SEC will update and supersede this information. We are incorporating by reference the following documents into this prospectus supplement:

our annual report on Form 10-K for the year ended December 31, 2013, filed with the SEC on February 26, 2014;

our Definitive Proxy Statement on Schedule 14A filed with the SEC on April 5, 2013; and

Table of Contents

the description of our common stock contained in our registration statement on Form 8-A, as filed with the SEC on July 15, 1999, including any amendment or report filed for the purpose of updating such description.

We also are incorporating by reference into this prospectus supplement any future filings we make with the SEC under Sections 13(a), 13(c), 14 or 15(d) of the Securities Exchange Act of 1934 after the date of the registration statement of which this prospectus supplement is a part and prior to the termination of the offering of the securities to which this prospectus supplement relates. You can request a copy of these filings, at no cost, by writing or telephoning us at the following address or telephone number:

BioMarin Pharmaceutical Inc.

770 Lindero Street

San Rafael, California

(415) 455-7558

Attn: Investor Relations

In no event, however, will any of the information that we furnish to the SEC in any current report on Form 8-K or any other report or filing be incorporated by reference into, or otherwise included in, this prospectus supplement and the accompanying prospectus. Any statement contained in a document incorporated or deemed to be incorporated by reference herein shall be deemed to be modified or superseded for purposes of this prospectus supplement to the extent that a statement contained herein, or in any other subsequently filed document that also is or is deemed to be incorporated by reference herein, modifies or supersedes such statement. Any such statement so modified or superseded shall not be deemed, except as so modified or superseded, to constitute a part of this prospectus supplement.

S-48

Table of Contents

PROSPECTUS

Common Stock

Debt Securities

We may offer and sell the following securities, from time to time in one or more offerings:

shares of our common stock;

our unsecured debt securities, in one or more series, which may be either senior, senior subordinated or subordinated debt securities; or

any combination of the foregoing.

This prospectus provides you with a general description of the securities we may offer. Each time we sell securities, we will provide a supplement to this prospectus that contains specific information about the offering and the amounts, prices and terms of the securities. A prospectus supplement may also add, update or change information contained in this prospectus. You should read this prospectus and the accompanying prospectus supplement, together with the documents we incorporate by reference, carefully before you invest in any of our securities. This prospectus may not be used to offer or sell securities unless it accompanies a prospectus supplement.

Our principal executive offices are located at 770 Lindero Street, San Rafael, California 94901, and our telephone number is (415) 506-6700.

Our common stock is listed on the Nasdaq Global Select Market under the symbol BMRN . On October 4, 2013, the last reported sale price of our common stock was \$73.82 per share.

Investing in our securities involves various risks. See the sections entitled RISK FACTORS on page 4 and CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS on page 5. Additional risks associated with an investment in us as well as with the particular types of securities will be described in the related prospectus supplements.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed upon the accuracy or adequacy of this prospectus. Any representation to the contrary is a criminal offense.

The securities may be offered directly by us from time to time, through agents designated by us or to or through underwriters or dealers. If any agents, dealers or underwriters are involved in the sale of any of these securities, the applicable prospectus supplement will provide the names of the agents, dealers or underwriters and any applicable fees, commissions or discounts.

The date of this prospectus is October 7, 2013.

Table of Contents

TABLE OF CONTENTS

| | Page |
|---|-------------|
| <u>About this Prospectus</u> | 1 |
| <u>BioMarin Pharmaceutical Inc.</u> | 3 |
| <u>Risk Factors</u> | 4 |
| <u>Cautionary Note Regarding Forward-looking Statements</u> | 5 |
| <u>Where You Can Find More Information</u> | 6 |
| <u>Information Incorporated by Reference</u> | 7 |
| <u>Ratio of Earnings to Fixed Charges</u> | 9 |
| <u>Use of Proceeds</u> | 9 |
| <u>General Description of Securities</u> | 10 |
| <u>Description of Capital Stock</u> | 11 |
| <u>Description of Debt Securities</u> | 12 |
| <u>Plan of Distribution</u> | 21 |
| <u>Legal Matters</u> | 23 |
| <u>Experts</u> | 23 |

Table of Contents

ABOUT THIS PROSPECTUS

Whenever we refer to we, our or us in this prospectus, we mean BioMarin Pharmaceutical Inc. and its consolidated subsidiaries, unless the context suggests otherwise. When we refer to you or yours, we mean the holders of the applicable series of securities.

This prospectus is part of an automatic shelf registration statement that we filed with the Securities and Exchange Commission, or the SEC, as a well-known seasoned issuer as defined in Rule 405 under the Securities Act of 1933, as amended, or the Securities Act, under a shelf registration process. Under this shelf registration process, we may sell from time to time in one or more offerings the following securities:

shares of our common stock;

our unsecured debt securities, in one or more series, which may be either senior, senior subordinated or subordinated debt securities; or

any combination of the foregoing.

This prospectus provides you with a general description of the securities we may offer. Each time we offer securities, we will provide you with a prospectus supplement that describes the specific amounts, prices and terms of the securities we offer. Any prospectus supplement and any pricing supplement may also add to, update or change the information contained in this prospectus. To the extent that any statement that we make in a prospectus supplement is inconsistent with statements made in this prospectus, the statements made in this prospectus will be deemed modified or superseded by those made in a prospectus supplement. Please carefully read this prospectus, any prospectus supplement and any pricing supplement or free writing prospectus prepared by or on behalf of us, in addition to the information described below under the headings **Where You Can Find More Information** and **Information Incorporated by Reference**.

This prospectus does not contain all of the information provided in the registration statement we filed with the SEC. For further information about us or the securities offered hereby, you should refer to that registration statement, which you can obtain from the SEC as described below under **Where You Can Find More Information** and **Information Incorporated by Reference**.

You should rely only on the information contained in this prospectus, in any accompanying prospectus supplement or incorporated by reference herein or therein. We have not authorized any other person to provide you with different information or make any representation that is different. If anyone provides you with different or inconsistent information, you should not rely on it. This prospectus and any accompanying prospectus supplement do not constitute an offer to sell or a solicitation of an offer to buy any securities other than the registered securities to which they relate, and this prospectus and any accompanying prospectus supplement do not constitute an offer to sell or the solicitation of an offer to buy securities in any jurisdiction where, or to any person to whom, it is unlawful to make such an offer or solicitation. You should not assume that the information contained in this prospectus and any accompanying prospectus supplement is correct on any date after the respective dates of the prospectus and such prospectus supplement or supplements, as applicable, even though this prospectus and such prospectus supplement or supplements are delivered or securities are sold at a later date pursuant to the prospectus and such prospectus supplement or supplements. Since the respective dates of the prospectus contained in this registration statement and any accompanying prospectus supplement, our business, financial condition, results of operations and prospects may have changed. We may only sell securities pursuant to this prospectus if this prospectus is accompanied by a prospectus supplement.

Table of Contents

We may sell the securities to or through underwriters, dealers or agents or directly to purchasers. We and our agents reserve the sole right to accept or reject in whole or in part any proposed purchase of securities. The prospectus supplement, which we will provide to you each time we offer securities, will set forth the names of any underwriters, dealers or agents involved in the sale of the securities, if any, and any applicable fee, commission or discount arrangements with them. See Plan of Distribution.

Table of Contents

BIOMARIN PHARMACEUTICAL INC.

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 four approved products and multiple investigational product candidates. Approved products include Naglazyme[®] (galsulfase), Kuvan[®] (sapropterin dihydrochloride), Aldurazyme[®] (aronidase) and Firdapse[®] (amifampridine phosphate).

Naglazyme received marketing approval in the United States (U.S.) in May 2005, in the European Union (EU) in January 2006 and subsequently in other countries. Kuvan was granted marketing approval in the U.S. and EU in December 2007 and December 2008, respectively. In December 2009, the European Medicines Agency (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 other countries.

We are conducting clinical trials on several investigational product candidates for the treatment of various diseases including: Vimizim (formerly referred to as GALNS), an enzyme replacement therapy for the treatment of Mucopolysaccharidosis Type IV or Morquio Syndrome Type A, or MPS IV A, PEG-PAL, an enzyme substitution therapy for the treatment of phenylketonuria or PKU, BMN-701, an enzyme replacement therapy for Pompe disease, a glycogen storage disorder, BMN-673, an orally available poly (ADP-ribose) polymerase, or PARP inhibitor for the treatment of patients with certain cancers, BMN-111, a peptide therapeutic for the treatment of achondroplasia and BMN-190 for the treatment of late infantile neuronal ceroid lipofuscinosis, or CLN2, a form of Batten disease. We are conducting or planning to conduct preclinical development of several other enzyme product candidates for genetic and other metabolic diseases and have recently licensed a Factor VIII gene therapy program for Hemophilia A from University College London and St. Jude's Children's Research Hospital.

Vimizim is our trademark. BioMarin[®], Naglazyme[®], Kuvan[®] and Firdapse[®] are our registered trademarks. Aldurazyme is a registered trademark of BioMarin/Genzyme LLC.

We were incorporated in Delaware in October 1996 and began operations on March 21, 1997. Our principal executive offices are located at 770 Lindero Street, San Rafael, California 94901 and our telephone number is (415) 506-6700.

Table of Contents

RISK FACTORS

Investment in any securities offered pursuant to this prospectus involves risks. Before making an investment decision, you should carefully consider the risk factors incorporated by reference in this prospectus from our most recent Annual Report on Form 10-K and our subsequent Quarterly Reports on Form 10-Q and the other information contained in this prospectus, as updated by our subsequent filings under the Securities Exchange Act of 1934, as amended, or the Exchange Act, and the risk factors and other information contained in the applicable prospectus supplement before acquiring any of such securities. The risks so described are not the only risks we face. Additional risks not presently known to us or that we currently deem immaterial may also impair our business operations. Our business, financial condition, results of operations or prospects could be materially adversely affected by any of these risks. The occurrence of any of these risks might cause you to lose all or part of your investment in the offered securities. Please also refer to the section below entitled "Cautionary Note Regarding Forward-Looking Statements."

Table of Contents

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus, any prospectus supplement and the documents incorporated by reference in this prospectus or any prospectus supplement contain forward-looking statements within the meaning of Section 27A of the Securities Act and Section 21E of the Exchange Act that involve substantial risks and uncertainties. All statements, other than statements of historical facts, included in this prospectus, any prospectus supplement or any document incorporated by reference in this prospectus or any prospectus supplement 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 or 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 intended to identify 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

Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 424B5

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 and any 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.

Table of Contents

WHERE YOU CAN FIND MORE INFORMATION

We are subject to the informational requirements of the Exchange Act and file annual, quarterly and current reports, proxy statements and other information with the SEC. You may read and copy these reports, proxy statements and other information at the SEC's Public Reference Room at 100 F Street, N.E., Washington, D.C. 20549. You can request copies of these documents by writing to the SEC and paying a fee for the copying cost. Please call the SEC at 1-800-SEC-0330 for more information about the operation of the Public Reference Room. Our SEC filings are also available at the SEC's website at <http://www.sec.gov>. The address of our internet site is <http://www.BMRN.com>. We make available free of charge on or through our internet site our Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act, as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. Other than the electronic prospectus, the information on our website is not part of this prospectus.

Table of Contents

INFORMATION INCORPORATED BY REFERENCE

We have filed with the SEC a registration statement on Form S-3 under the Securities Act relating to the securities covered by this prospectus. This prospectus is a part of the registration statement and does not contain all the information in the registration statement. Whenever a reference is made in this prospectus to a contract, agreement or other document, the reference is only a summary and you should refer to the exhibits that are a part of the registration statement for a copy of the contract, agreement or other document. Each statement regarding a contract, agreement or other document is qualified in its entirety by reference to the actual document. You may review a copy of the registration statement at the SEC's Public Reference Room in Washington, D.C., as well as through the SEC's Internet website.

The SEC allows us to incorporate by reference into this prospectus the information we file with it. This means that we can disclose important information to you by referring you to those documents. The information we incorporate by reference is considered a part of this prospectus and any accompanying prospectus supplement, and later information we file with the SEC will automatically update and supersede this information. We incorporate by reference the documents listed below:

Our Annual Report on Form 10-K for the year ended December 31, 2012, as filed with the SEC on February 26, 2013;

Our Quarterly Report on Form 10-Q for the quarter ended March 31, 2013, as filed with the SEC on April 29, 2013;

Our Quarterly Report on Form 10-Q for the quarter ended June 30, 2013, as filed with the SEC on July 29, 2013;

Our Definitive Proxy Statement on Schedule 14A filed with the SEC on April 5, 2013;

Our Current Reports on Form 8-K, as filed with the SEC on March 18, 2013, March 27, 2013, May 16, 2013, July 30, 2013 and August 26, 2013;

The description of our common stock contained in our registration statement on Form 8-A, as filed with the SEC on July 15, 1999, including any amendment or report filed for the purpose of updating such description; and

All other reports and other documents filed by us with the SEC under Section 13(a), 13(c), 14 or 15(d) of the Exchange Act after the date of this prospectus and before the filing of a post-effective amendment which indicates that all securities offered hereby have been sold or which deregisters all securities then remaining unsold, except as to any portion of any future annual or quarterly report to stockholders or document or current report furnished under current Items 2.02 or 7.01 of Form 8-K that is not deemed filed under such provisions.

We will provide to you at no cost a copy of any and all of the information incorporated by reference into the registration statement of which this prospectus is a part. You may make a request for copies of this information in writing or by telephone. Requests should be directed to:

BioMarin Pharmaceutical Inc.

Attention: Corporate Secretary

770 Lindero Street

San Rafael, California 94901

(415) 506-6700

Table of Contents

Any statement contained in a document incorporated or deemed to be incorporated by reference in this prospectus shall be deemed modified, superceded or replaced for purposes of this prospectus to the extent that a statement contained in this prospectus, or in any subsequently filed document that also is deemed to be incorporated by reference in this prospectus, modifies, supercedes or replaces such statement. Any statement so modified, superceded or replaced shall not be deemed, except as so modified, superceded or replaced, to constitute a part of this prospectus.

You should rely only on the information provided or incorporated by reference in this prospectus or any related prospectus. We have not authorized anyone to provide you with different information. You should not assume that the information in this prospectus or any related prospectus is accurate as of any date other than the date on the front of the document.

Table of Contents**RATIO OF EARNINGS TO FIXED CHARGES**

Our ratios of earnings to fixed charges are as follows for the periods indicated:

| | Six Months Ended | | Fiscal Year Ended | | | |
|------------------------------------|------------------|----------------------|----------------------|----------------------|----------------------|----------------------|
| | June 30, 2013 | December 31, 2012 | December 31, 2011 | December 31, 2010 | December 31, 2009 | December 31, 2008 |
| Ratio of earnings to fixed charges | * | * | * | * | 1.2 | 2.6 |

* For the six months ended June 30, 2013 and the fiscal years ended December 31, 2012, 2011 and 2010, our earnings were insufficient to cover fixed charges by \$64.2 million, \$117.0 million, \$41.1 million and \$19.1 million, respectively.

For purposes of calculating the ratio of earnings to fixed charges, earnings represent our income before provision for income taxes, pretax income (loss) attributable to noncontrolling interest and fixed charges. Fixed charges consist of: (i) interest expense, (ii) an estimate of the interest within rental expense and (iii) amortized premiums, discounts or capitalized expenses related to indebtedness. Rental expense amounts relate to the interest factor inherent in our operating leases.

USE OF PROCEEDS

We will retain broad discretion over the use of the net proceeds from the sale of our securities offered hereby. Except as described in any prospectus supplement, we currently intend to use the net proceeds from the sale of securities offered by this prospectus to repay or refinance debt, and for working capital, capital expenditures and other general corporate purposes. We may also use the proceeds to fund acquisitions of businesses, technologies or product lines that complement our current business. However, we currently have no commitments or agreements for any specific acquisitions. Accordingly, our management will have significant flexibility in applying these proceeds. When a particular series of securities is offered, the prospectus supplement relating thereto will set forth our intended use for the net proceeds we receive from the sale of the securities. Pending the application of the net proceeds, we expect to invest the proceeds in short term, interest bearing instruments or other investment grade securities.

Table of Contents

GENERAL DESCRIPTION OF SECURITIES

We, directly or through agents, dealers or underwriters designated from time to time, may offer, issue and sell, together or separately, in one or more offerings:

shares of our common stock;

our unsecured debt securities, in one or more series, which may be either senior, senior subordinated or subordinated debt securities; or

any combination of the foregoing.

We may issue the debt securities as exchangeable for and/or convertible into shares of our common stock. The common stock and the debt securities are collectively referred to herein as the securities. This prospectus provides you with a general description of the securities we may offer. Each time we offer securities, we will provide you with a prospectus supplement that describes the specific amounts, prices and terms of the securities we offer. The securities involve various risks that we will describe in a section entitled "Risk Factors" that will be included in each prospectus supplement.

Table of Contents

DESCRIPTION OF CAPITAL STOCK

Our authorized common stock consists of 250,000,000 shares, \$0.001 par value per share. At October 4, 2013, there were 142,206,962 shares of our common stock issued and outstanding. The approximate number of stockholders of record of our common stock as of October 4, 2013 was 51.

The holders of our common stock are entitled to one vote for each share held of record on all matters submitted to a vote of the stockholders. Subject to preferences that may be applicable to any outstanding preferred stock, holders of common stock are entitled to receive ratably such dividends as may be declared by our board of directors out of funds legally available. In the event of liquidation, dissolution or winding up of us, holders of common stock are entitled to share ratably in all assets remaining after payment of liabilities and the liquidation preference of any outstanding preferred stock. Holders of common stock have no preemptive rights and no right to cumulate votes in the election of directors. There are no redemption or sinking fund provisions applicable to the common stock. All outstanding shares of common stock are fully paid and nonassessable.

Table of Contents

DESCRIPTION OF DEBT SECURITIES

This prospectus describes certain general terms and provisions of our debt securities. When we offer to sell a particular series of debt securities, we will describe the specific terms of the series in a prospectus supplement or a pricing supplement. We will also indicate in the supplement whether the general terms and provisions described in this prospectus apply to a particular series of debt securities. Unless otherwise specified in a supplement to this prospectus, our debt securities will be our direct obligations and they may be unsecured, senior, senior-subordinated or subordinated indebtedness. We may issue our debt securities under one or more indentures and each indenture will be dated on or before the issuance of the debt securities to which it relates. Additionally, each indenture must be in the form filed as an exhibit to the Registration Statement containing this prospectus or in a form incorporated by reference to this prospectus in a post-effective amendment to the Registration Statement or a Form 8-K. The form of indenture is subject to any amendments or supplements that may be adopted from time to time. We will enter into each indenture with a trustee and the trustee for each indenture may be the same. The indenture will be subject to, and governed by, the Trust Indenture Act of 1939, as amended. Because this description of debt securities is a summary, it does not contain all the information that may be important to you. You should read all provisions of our indenture and our debt securities to assure that you have all the important information you need to make any required decisions. All capitalized terms used, but not defined, in this section shall have the meanings set forth in the form of indenture.

TERMS

The particular terms of any series of our debt securities will be described in a prospectus supplement. Additionally, any applicable modifications of or additions to the general terms of our debt securities described in this prospectus and in the applicable indenture will also be described in a prospectus supplement. Accordingly, for a description of the terms of any series of our debt securities, you must refer to both the prospectus supplement relating to those debt securities and the description of the debt securities set forth in this prospectus. If any particular terms of our debt securities described in a prospectus supplement differ from any of the terms described in this prospectus, then those terms as set forth in the relevant prospectus supplement will control.

The terms of each series of debt securities will be established by or pursuant to a resolution of our Board of Directors and detailed or determined in the manner provided in a Board of Directors resolution, an officers' certificate pursuant to the authority granted under a Board resolution or by a supplemental indenture. The particular terms of each series of debt securities will be described in a prospectus supplement relating to the series, including any pricing supplement.

We can issue an unlimited amount of debt securities under the indenture that may be in one or more series with the same or various maturities, at par, at a premium or at a discount. We will set forth in a prospectus supplement (including any pricing supplement) relating to any series of debt securities being offered, the initial offering price, the aggregate principal amount and the following terms of the debt securities:

the title of the debt securities;

the price or prices (expressed as a percentage of the aggregate principal amount) at which we will sell the debt securities;

any limit on the aggregate principal amount of the debt securities;

the date or dates on which we will pay the principal on the debt securities;

the rate or rates (which may be fixed or variable) per annum or the method used to determine the rate or rates (including any commodity, commodity index, stock exchange index or financial index) at which the debt securities will bear interest, the date or dates from which interest will accrue, the

Table of Contents

date or dates on which interest will commence and be payable and any regular record date for the interest payable on any interest payment date;

the place or places where the principal of, premium, if any, and interest on the debt securities will be payable;

the terms and conditions upon which the debt securities are convertible into or exchangeable for other securities;

the terms and conditions upon which we may redeem the debt securities;

whether and under what circumstances, if any, we will pay additional amounts on any debt securities held by a person who is not a United States person for tax purposes, and whether we can redeem the debt securities if we have to pay such additional amounts;

any obligation we have to redeem or purchase the debt securities pursuant to any sinking fund or analogous provisions or at the option of a holder of debt securities;

the dates on which and the price or prices at which we will repurchase the debt securities at the option of the holders of debt securities and other detailed terms and provisions of these repurchase obligations;

the denominations in which the debt securities will be issued, if other than denominations of \$1,000 and any integral multiple thereof;

whether the debt securities will be issued in the form of certificated debt securities or global debt securities;

the principal amount due at maturity, and whether the debt securities will be issued with original issue discount;

the portion of principal amount of the debt securities payable upon declaration of acceleration of the maturity date, if other than the principal amount;

the currency of denomination of the debt securities;

the designation of the currency or currency units in which payment of principal of, premium, if any, and interest on the debt securities will be made;

the manner in which the amounts of payment of principal of, premium, if any, or interest on the debt securities will be determined, if these amounts may be determined by reference to an index based on a currency or currencies other than that in which the debt securities are denominated or designated to be payable or by reference to a commodity, commodity index, stock exchange index or financial index;

the terms of the subordination of any series of the debt securities;

restrictions on transfer, sale or other assignment of the debt securities, if any;

any addition to or change in the events of default described in this prospectus or in the indenture with respect to the debt securities and any change in the acceleration provisions described in this prospectus or in the indenture with respect to the debt securities;

any addition to or change in the covenants described in this prospectus or in the indenture with respect to the debt securities;

Table of Contents

any other terms of the debt securities, which may supplement, modify or delete any provision of the indenture as it applies to that series; and

any depositaries, interest rate calculation agents, exchange rate calculation agents or other agents with respect to the debt securities.

We may issue debt securities that provide for an amount less than their stated principal amount to be due and payable upon declaration of acceleration of their maturity pursuant to the terms of the indenture. We will provide you with information on the federal income tax considerations and other special considerations applicable to any of these debt securities in the applicable prospectus supplement.

EXCHANGE OF SECURITIES

Each debt security will be represented by either one or more global securities registered in the name of The Depository Trust Company, as Depository, or a nominee of the Depository (we refer herein to any debt security represented by a global debt security as a book-entry debt security), or a certificate issued in definitive registered form (we refer herein to any debt security represented by a certificated security as a certificated debt security), as described in the applicable prospectus supplement. Except as described under Global Debt Securities and Book-Entry System below, book-entry debt securities will not be issuable in certificated form.

Certificated Debt Securities. You may transfer or exchange certificated debt securities with the Registrar's office or paying agencies in accordance with the terms of the indenture. No service charge will be made for any transfer or exchange of certificated debt securities, but we may require payment of a sum sufficient to cover any tax or other governmental charge payable in connection with a transfer or exchange.

You may transfer certificated debt securities and the right to receive the principal of, premium, if any, and interest on certificated debt securities only by surrendering the old certificate representing those certificated debt securities and either we or the trustee will reissue the old certificate to the new holder or we or the trustee will issue a new certificate to the new holder.

Global Debt Securities And Book-Entry System. Each global debt security representing book-entry debt securities will be deposited with, or on behalf of, the Depository, and registered in the name of the Depository or a nominee of the Depository.

The Depository has indicated it intends to follow the following procedures with respect to book-entry debt securities.

Ownership of beneficial interests in book-entry debt securities will be limited to persons that have accounts with the Depository for the related global debt security (we refer herein to these persons as participants) or persons that may hold interests through participants. Upon the issuance of a global debt security, the Depository will credit, on its book-entry registration and transfer system, each participant's account with the respective principal amounts of the book-entry debt securities represented by the global debt security beneficially owned by each such participant. The accounts to be credited will be designated by any dealers, underwriters or agents participating in the distribution of the book-entry debt securities. Ownership of book-entry debt securities will be shown on, and the transfer of the ownership interests will be effected only through, records maintained by the Depository for the related global debt security (with respect to interests of participants) and on the records of participants (with respect to interests of persons holding through participants). The laws of some states may require that certain purchasers of securities take physical delivery of such securities in definitive form. These laws may impair the ability to own, transfer or pledge beneficial interests in book-entry debt securities.

So long as the Depository for a global debt security, or its nominee, is the registered owner of that global debt security, the Depository or its nominee, as the case may be, will be considered the sole owner or holder of the

Table of Contents

book-entry debt securities represented by such global debt security for all purposes under the indenture. Except as described herein, beneficial owners of book-entry debt securities will not be entitled to have securities registered in their names, will not receive or be entitled to receive physical delivery of a certificate in definitive form representing securities and will not be considered the owners or holders of those securities under the indenture. Accordingly, to exercise any rights of a holder under the indenture, each person beneficially owning book-entry debt securities must rely on the procedures of the Depository for the related global debt security and, if that person is not a participant, on the procedures of the participant through which that person owns its interest.

We understand, however, that under existing industry practice, the Depository will authorize the persons on whose behalf it holds a global debt security to exercise certain rights of holders of debt securities, and the indenture provides that we, the trustee and our respective agents will treat as the holder of a debt security the persons specified in a written statement of the Depository with respect to that global debt security for purposes of obtaining any consents or directions required to be given by holders of the debt securities pursuant to the indenture.

We will make payments of principal of, premium, if any, and interest on book-entry debt securities to the Depository or its nominee, as the case may be, as the registered holder of the related global debt security. We, the trustee and any other agent of ours or agent of the trustee will not have any responsibility or liability for any aspect of the records relating to or payments made on account of beneficial ownership interests in a global debt security or for maintaining, supervising or reviewing any records relating to such beneficial ownership interests.

We expect that the Depository, upon receipt of any payment of principal of, premium, if any, or interest on a global debt security, will credit participants' accounts with payments in amounts proportionate to the respective amounts of book-entry debt securities held by each participant as shown on the records of the Depository. We also expect that payments by participants to owners of beneficial interests in book-entry debt securities held through those participants will be governed by standing customer instructions and customary practices, as is now the case with the securities held for the accounts of customers in bearer form or registered in street name, and will be the responsibility of those participants.

We will issue certificated debt securities in exchange for each global debt security if the Depository is at any time unwilling or unable to continue as Depository or ceases to be a clearing agency registered under the Exchange Act, and a successor Depository registered as a clearing agency under the Exchange Act is not appointed by us within 90 days. In addition, we may at any time and in our sole discretion determine not to have any of the book-entry debt securities of any series represented by one or more global debt securities and, in that event, we will issue certificated debt securities in exchange for the global debt securities of that series. Global debt securities will also be exchangeable by the holders for certificated debt securities if an event of default with respect to the book-entry debt securities represented by those global debt securities has occurred and is continuing and the Depository requests that we issue certificated debt securities. Any certificated debt securities issued in exchange for a global debt security will be registered in such name or names as the Depository shall instruct the trustee. We expect that such instructions will be based upon directions received by the Depository from participants with respect to ownership of book-entry debt securities relating to such global debt security.

We have obtained the foregoing information in this section concerning the Depository and the Depository's book-entry system from sources we believe to be reliable, but we take no responsibility for the accuracy of this information.

NO PROTECTION IN THE EVENT OF A CHANGE OF CONTROL

Unless we provide otherwise in the applicable prospectus supplement, the debt securities will not contain any provisions which may afford holders of the debt securities protection in the event we have a change in control or in the event of a highly leveraged transaction (whether or not such transaction results in a change in control).

Table of Contents

COVENANTS

Unless we provide otherwise in the applicable prospectus supplement, the debt securities will not contain any restrictive covenants, including without limitation covenants restricting us or any of our subsidiaries from incurring, issuing, assuming or guarantying any indebtedness whether or not secured by a lien on any of our or our subsidiaries' property or capital stock, or restricting us or any of our subsidiaries from entering into any sale and leaseback transactions.

CONSOLIDATION, MERGER AND SALE OF ASSETS

Unless we provide otherwise in the applicable prospectus supplement, we may not consolidate with or merge into, or convey, transfer or lease all or substantially all of our properties and assets to, any person (a successor person), and we may not permit any person to merge into, or convey, transfer or lease its properties and assets substantially as an entirety, to us, unless:

the successor person is a corporation, partnership, trust or other entity organized and validly existing under the laws of any U.S. domestic jurisdiction and expressly assumes our obligations on the debt securities and under the indenture;

after giving effect to the transaction, no event of default, and no event which, after notice or lapse of time, or both, would become an event of default, shall have occurred and be continuing under the indenture; and

certain other conditions are met.

EVENTS OF DEFAULT

Unless we provide otherwise in the applicable prospectus supplement, event of default means with respect to any series of debt securities, any of the following:

default in the payment of any interest upon any debt security of that series when it becomes due and payable, and continuance of that default for a period of 30 days (unless the entire amount of such payment is deposited by us with the trustee or with a paying agent prior to the expiration of the 30-day period);

default in the payment of principal on any debt security of that series when due and payable;

default in the deposit of any sinking fund payment, when and as due in respect of any debt security of that series;

default in the performance or breach of any other covenant or warranty by us in the indenture or any debt security (other than a covenant or warranty that has been included in the indenture solely for the benefit of a series of debt securities other than that series), which default continues uncured for a period of 60 days after we receive written notice of such default from the trustee or we and the trustee receive written notice of such default from the holders of at least 25% in principal amount of the outstanding debt securities of that series as provided in the indenture;

certain events of our bankruptcy, insolvency or reorganization;

Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 424B5

default under any of our debt (including a default with respect to any debt security of a different series) or of our subsidiaries, if (1) such default results from the failure to pay any such debt when it becomes due, (2) the principal amount of such debt, together with the principal amount of any other

Table of Contents

such debt in default for failure to pay principal at stated final maturity or the maturity of which has been so accelerated, aggregates \$15.0 million or more at any one time outstanding, and (3) such debt is not discharged or such acceleration is not rescinded or annulled within 30 days after written notice to us and the trustee by the holder or holders of such debt in the manner provided for in the applicable debt instrument; or

any other event of default provided with respect to debt securities of that series that is described in the applicable prospectus supplement accompanying this prospectus.

An event of default may also be an event of default if so specified in the applicable supplemental indenture and prospectus supplement under our bank credit agreements or other debt securities in existence from time to time, including, without limitation, our 1.875% senior subordinated convertible notes due 2017, and under certain guaranties by us of any subsidiary indebtedness. In addition, certain events of default or an acceleration under the indenture may also be an event of default under some of our other indebtedness outstanding from time to time.

Unless we provide otherwise in the applicable prospectus supplement, if an event of default with respect to debt securities of any series at the time outstanding under the indenture occurs and is continuing (other than certain events of our bankruptcy, insolvency or reorganization), then the trustee or the holders of not less than 25% in principal amount of the outstanding debt securities of that series may, by written notice to us (and to the trustee if given by the holders), declare to be due and payable immediately the principal (or, if the debt securities of that series are discount securities, that portion of the principal amount as may be specified in the terms of that series) and premium, if any, of all debt securities of that series. In the case of an event of default resulting from certain events of bankruptcy, insolvency or reorganization, the principal (or such specified amount) and premium, if any, of all outstanding debt securities under the indenture will become and be immediately due and payable without any declaration or other act by the trustee or any holder of outstanding debt securities. At any time after a declaration of acceleration with respect to debt securities of any series under the indenture has been made, but before the trustee has obtained a judgment or decree for payment of the money due, the holders of a majority in principal amount of the outstanding debt securities of that series may, subject to our having paid or deposited with the trustee a sum sufficient to pay overdue interest and principal which has become due other than by acceleration and certain other conditions, rescind and annul such acceleration if all events of default, other than the non-payment of accelerated principal and premium, if any, with respect to debt securities of that series, have been cured or waived as provided in the indenture. For information as to waiver of defaults see the discussion under **Modification and Waiver** below. We refer you to the prospectus supplement relating to any series of debt securities that are discount securities for the particular provisions relating to acceleration of a portion of the principal amount of the discount securities upon the occurrence of an event of default and the continuation of an event of default.

The indenture will provide that the trustee will be under no obligation to exercise any of its rights or powers under the indenture at the request of any holder of outstanding debt securities, unless the trustee receives indemnity satisfactory to it against any loss, liability or expense. Subject to certain rights of the trustee, the holders of a majority in principal amount of the outstanding debt securities of any series shall have the right to direct the time, method and place of conducting any proceeding for any remedy available to the trustee or exercising any trust or power conferred on the trustee with respect to the debt securities of that series.

No holder of any debt security of any series will have any right to institute any proceeding, judicial or otherwise, with respect to the indenture or for the appointment of a receiver or trustee, or for any remedy under the indenture, unless:

that holder has previously given to the trustee written notice of a continuing event of default with respect to debt securities of that series; and

the holders of at least 25% in principal amount of the outstanding debt securities of that series have made written request, and offered satisfactory indemnity, to the trustee to institute such proceeding

Table of Contents

as trustee, and the trustee shall not have received from the holders of a majority in principal amount of the outstanding debt securities of that series a direction inconsistent with that request and has failed to institute the proceeding within 60 days. Notwithstanding the foregoing, the holder of any debt security will have an absolute and unconditional right to receive payment of the principal of and any interest on that debt security on or after the due dates expressed in that debt security and to institute suit for the enforcement of payment.

The indenture requires us, within 90 days after the end of our fiscal year, to furnish to the trustee a certificate as to compliance with the indenture. The indenture provides that the trustee may withhold notice to the holders of debt securities of any series of any default or event of default (except in payment on any debt securities of that series) with respect to debt securities of that series if it in good faith determines that withholding notice is in the interests of the holders of those debt securities.

MODIFICATION AND WAIVER

Unless we provide otherwise in the applicable prospectus supplement, we and the trustee may modify and amend the indenture with the consent of the holders of at least a majority in principal amount of the outstanding debt securities of each series affected by the modifications or amendments. We and the trustee may not make any modification or amendment without the consent of the holder of each affected debt security then outstanding if that amendment will:

change the amount of debt securities whose holders must consent to an amendment or waiver;

reduce the rate of or extend the time for payment of interest (including default interest) on any debt security;

reduce the principal of or change the stated maturity of any debt security or reduce the amount of, or postpone the date fixed for, the payment of any sinking fund or analogous obligation with respect to any series of debt securities;

reduce the principal amount of discount securities payable upon acceleration of maturity;

waive a default in the payment of the principal of or interest on any debt security (except a rescission of acceleration of the debt securities of any series by the holders of at least a majority in aggregate principal amount of the then outstanding debt securities of that series and a waiver of the payment default that resulted from that acceleration);

make the principal of or interest on any debt security payable in currency other than that stated in the debt security;

make any change to certain provisions of the indenture relating to, among other things, the right of holders of debt securities to receive payment of the principal of and interest on those debt securities and the right of holders to institute suit for the enforcement of such payment; the right of holders to waive past defaults; the right of holders of a specified principal amount of debt securities which are denominated in a foreign currency to be deemed for the purposes of taking action under the indenture, that amount of U.S. dollars at the Market Exchange Rate (as defined in the indenture); certain terms regarding judgments in foreign currencies; or to amend the limitations described in this bullet point; or

waive a redemption payment with respect to any debt security or change any of the provisions with respect to the redemption of any debt securities.

Table of Contents

Except for certain specified provisions, the holders of at least a majority in principal amount of the outstanding debt securities of any series may, on behalf of the holders of all debt securities of that series, waive our compliance with provisions of the indenture. The holders of a majority in principal amount of the outstanding debt securities of any series may, on behalf of the holders of all the debt securities of that series, waive any past or existing default under the indenture with respect to that series and its consequences, except a default in the payment of the principal of, premium, if any, or any interest on any debt security of that series; provided, however, that the holders of a majority in principal amount of the outstanding debt securities of any series may rescind an acceleration and its consequences, including any related payment default that resulted from the acceleration.

DEFEASANCE OF DEBT SECURITIES AND CERTAIN COVENANTS IN CERTAIN CIRCUMSTANCES

Legal Defeasance. The indenture provides that, unless the terms of the applicable series of debt securities provide otherwise, we may be discharged from any and all obligations in respect of the debt securities of any series (except for certain obligations to register the transfer or exchange of debt securities of the series, to replace stolen, lost or mutilated debt securities of the series, to maintain paying agencies and certain provisions relating to the treatment of funds held by paying agents, the rights, powers, trusts and immunities of the Trustee under the indenture and our obligations in connection therewith and the rights of the holders of the securities to receive payment of principal and interest from the funds deposited with the trustee as described herein). We will be so discharged 90 days after the deposit with the trustee, in trust, of money, U.S. government obligations or a combination of money and U.S. government obligations that (in the case of U.S. government obligations, through the payment of interest and principal in accordance with their terms (and without reinvestment)) will provide money in an amount sufficient in the opinion of a nationally recognized firm of independent public accountants to pay and discharge each installment of principal, premium, if any, and interest on and any mandatory sinking fund payments in respect of the debt securities of that series on the dates such payments are due in accordance with the terms of the indenture and those debt securities.

This discharge may occur only if, among other things, we have delivered to the trustee an officers' certificate and an opinion of counsel stating that we have received from, or there has been published by, the United States Internal Revenue Service a ruling or, since the date of execution of the indenture, there has been a change in the applicable United States federal income tax law, in either case to the effect that holders of the debt securities of such series will not recognize income, gain or loss for United States federal income tax purposes as a result of the deposit, defeasance and discharge and will be subject to United States federal income tax on the same amount and in the same manner and at the same times as would have been the case if the deposit, defeasance and discharge had not occurred except for changes in applicable tax rates.

Defeasance of Certain Covenants. The indenture provides that, unless the terms of the applicable series of debt securities provide otherwise, upon compliance with certain conditions, we may omit to comply with the restrictive covenants of the indenture entitled SEC Reports, Compliance Certificate, Stay, Extension and Usury Laws, Corporate Existence, Taxes and When We May Merge, Etc., as well as any additional covenants contained in a supplement to the indenture, a board resolution or an officers' certificate delivered pursuant to the indenture. The conditions include, without limitation:

depositing with the trustee, in trust, money, U.S. government obligations or a combination of money and U.S. government obligations that (in the case of U.S. government obligations, through the payment of interest and principal in accordance with their terms (and without reinvestment)) will provide money in an amount sufficient in the opinion of a nationally recognized firm of independent public accountants to pay principal, premium, if any, and interest on and any mandatory sinking fund payments in respect of the debt securities of that series on the dates those payments are due in accordance with the terms of the indenture and those debt securities;

such deposit does not result in a breach or constitute a default under the indenture or any other agreement to which we are a party;

Table of Contents

no default or event of default with respect to the debt securities shall have occurred and be continuing on the date of deposit or during the period ending 90 days after such date; and

delivering to the trustee an opinion of counsel to the effect that the holders of the debt securities of that series will not recognize income, gain or loss for United States federal income tax purposes as a result of the deposit and related covenant defeasance and will be subject to United States federal income tax in the same amount and in the same manner and at the same times as would have been the case if the deposit and related covenant defeasance had not occurred except for changes in applicable tax rates.

Covenant Defeasance and Events of Default. In the event we exercise our option, as described above, not to comply with certain covenants of the indenture with respect to any series of debt securities and the debt securities of that series are declared due and payable because of the occurrence of any event of default, the amount of money, U.S. government obligations or a combination of money and U.S. government obligations on deposit with the trustee will be sufficient to pay amounts due on the debt securities of that series at the time such payments are due but may not be sufficient to pay amounts due on the debt securities of that series at the time of the acceleration resulting from the event of default. However, we will remain liable for those payments.

CONVERSION AND EXCHANGE RIGHTS

The debt securities may be exchanged for and/or converted into shares of common stock, shares of preferred stock or other securities. The terms, if any, on which the debt securities may be exchanged for and/or converted will be set forth in the applicable prospectus supplement. Such terms may include provisions for conversion, either mandatory, at the option of the holder, or at our option, in which case the number of shares of common stock, preferred stock or other securities to be received by the holders of the debt securities would be calculated as of a time and in the manner stated in the prospectus supplement.

GOVERNING LAW

The indenture and the debt securities as well as the relationship of the holders of debt securities and us will be governed by the laws of the State of New York applicable to agreements made and to be performed in such state, without regard to conflict of law principles thereof to the extent that the application of the laws of another jurisdiction would be required thereby.

Table of Contents

PLAN OF DISTRIBUTION

We may sell the securities from time to time in one or more transactions through underwriters or dealers, through agents, or directly to one or more purchasers or through a combination of these methods. We may also sell securities to one or more underwriters or to one or more dealers or agents and then register the resale of the securities by any such underwriters, dealers or agents. The distribution of the securities may be effected from time to time in one or more transactions at a fixed price or prices, which may be changed, or at market prices prevailing at the time of sale, at prices related to such prevailing market prices, or at negotiated prices. Sales of securities offered pursuant to this registration statement may be effected from time to time in one or more transactions on the Nasdaq Global Select Market or in negotiated transactions or a combination of these methods.

The applicable prospectus supplement will describe the terms of the offering of the securities, including:

the name or names of any underwriters, if any, and if required, any dealers or agents;

the purchase price of the securities and the proceeds we will receive from the sale;

any underwriting discounts and other items constituting underwriters' compensation;

any initial public offering price;

any over-allotment options under which underwriters may purchase additional securities from us;

any discounts or concessions allowed or reallocated or paid to dealers; and

any securities exchange or market on which the securities may be listed.

We may distribute the securities from time to time in one or more transactions at:

at a fixed price or prices, which may be changed;

market prices prevailing at the time of sale;

prices related to such prevailing market prices; or

negotiated prices.

Only underwriters named in a prospectus supplement are underwriters of the securities offered by such prospectus supplement.

If we use underwriters in the sale, they will acquire the securities for their own account and may resell them from time to time in one or more transactions at a fixed public offering price or at varying prices determined at the time of sale. We may offer the securities to the public through underwriting syndicates represented by managing underwriters or by underwriters without a syndicate. Subject to specific limited conditions, the

Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 424B5

underwriters will be obligated to purchase all the securities of the series offered by the applicable prospectus supplement. Any public offering price and any discounts or concessions allowed or reallocated or paid to dealers may change from time to time.

If we use a dealer in the sale of the securities being offered pursuant to this prospectus, we will sell the securities to the dealer, as principal. The dealer may then resell the securities to the public at varying prices to be determined by the dealer at the time of resale.

Table of Contents

We may sell securities directly or through agents we designate from time to time. We will name any agent involved in the offering and sale of securities and we will describe any commissions we will pay the agent in the applicable prospectus supplement. Unless the applicable prospectus supplement states otherwise, our agent will act on a best-efforts basis for the period of its appointment.

We may authorize agents or underwriters to solicit offers by institutional investors to purchase securities from us at the public offering price set forth in the applicable prospectus supplement pursuant to delayed delivery contracts providing for payment and delivery on a specified date in the future. We will describe the conditions to these contracts and the commissions we must pay for solicitation of these contracts in the applicable prospectus supplement.

In connection with the sale of the securities, underwriters, dealers or agents may receive compensation from us or from purchasers of the securities for whom they act as agents in the form of discounts, concessions or commissions. Underwriters may sell the securities to or through dealers, and those dealers may receive compensation in the form of discounts, concessions or commissions from the underwriters or commissions from the purchasers for whom they may act as agents. Underwriters, dealers and agents that participate in the distribution of the securities, and any institutional investors or others that purchase securities directly and then resell the securities, may be deemed to be underwriters, and any discounts or commissions received by them from us and any profit on the resale of the securities by them may be deemed to be underwriting discounts and commissions under the Securities Act.

We may provide agents and underwriters with indemnification against particular civil liabilities, including liabilities under the Securities Act, or contribution with respect to payments that the agents or underwriters may make with respect to such liabilities. Agents and underwriters may engage in transactions with, or perform services for, us in the ordinary course of business.

In addition, we may enter into derivative transactions with third parties (including the writing of options), or sell securities not covered by this prospectus to third parties in privately negotiated transactions. If the applicable prospectus supplement indicates, in connection with such a transaction the third parties may, pursuant to this prospectus and the applicable prospectus supplement, sell securities covered by this prospectus and the applicable prospectus supplement. If so, the third party may use securities borrowed from us or others to settle such sales and may use securities received from us to close out any related short positions. We may also loan or pledge securities covered by this prospectus and the applicable prospectus supplement to third parties, who may sell the loaned securities or, in an event of default in the case of a pledge, sell the pledged securities pursuant to this prospectus and the applicable prospectus supplement. The third party in such sale transactions will be an underwriter and will be identified in the applicable prospectus supplement or in a post-effective amendment.

All securities we offer other than common stock will be new issues of securities with no established trading market. Any underwriters may make a market in these securities, but will not be obligated to do so and may discontinue any market making at any time without notice. We cannot guarantee the liquidity of the trading markets for any securities.

Until the distribution of securities is completed, SEC rules may limit the underwriters from bidding for and purchasing our common stock. However, the underwriters may engage in transactions that stabilize the price of the common stock, such as bids or purchases to peg, fix or maintain that price.

These transactions may include short sales, stabilizing transactions and purchases to cover positions created by short sales. Short sales involve the sale by the underwriters of more shares than are listed on the cover of this prospectus supplement. Covered short sales are sales made in an amount not greater than the underwriters' option to purchase additional shares from us in the related offering. The underwriters may reduce the short position by purchasing shares in the open market, or by exercising all or part of any over-allotment.

Table of Contents

option which may be granted to them. In determining the source of shares to close out the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase shares through the over-allotment option. Naked short sales are any sales in excess of such option. The underwriters must close out any naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of our common stock in the open market after pricing that could adversely affect investors who purchase in the related offering. Stabilizing transactions consist of various bids for or purchases of shares of common stock made by the underwriters in the open market prior to the completion of the related offering.

Similar to the other purchase transactions, the underwriters' purchases of the securities to stabilize their price or to reduce a short position may cause the price of the common stock to be higher than it might be in the absence of such purchases.

Neither the underwriters nor we make any representation or prediction as to the direction or magnitude of any effect that the transactions described above may have on the price of the common stock or any securities sold in any offering pursuant to this prospectus. In addition, neither the underwriters nor we make any representation that the underwriters will engage in these transactions or that these transactions, once commenced, will not be discontinued without notice.

LEGAL MATTERS

Paul Hastings LLP, San Francisco, California, will pass upon certain legal matters relating to the issuance of the securities we are offering in this prospectus.

EXPERTS

The consolidated financial statements of BioMarin Pharmaceutical Inc. and subsidiaries as of December 31, 2012 and 2011, and for each of the years in the three-year period ended December 31, 2012, and management's assessment of the effectiveness of internal control over financial reporting as of December 31, 2012 have been incorporated by reference herein and in the registration statement in reliance upon the reports of KPMG LLP, independent registered public accounting firm, incorporated by reference herein, and upon the authority of said firm as experts in accounting and auditing.

The financial statements of BioMarin/Genzyme LLC for the year ended December 31, 2010 incorporated in this prospectus by reference to the Annual Report on Form 10-K of BioMarin Pharmaceutical Inc. for the year ended December 31, 2012 have been so incorporated in reliance on the report of PricewaterhouseCoopers LLP, independent accountants, given on the authority of said firm as experts in auditing and accounting.

Table of Contents

1,500,000 Shares

Common Stock

PROSPECTUS SUPPLEMENT

BofA Merrill Lynch

March , 2014