BIOMARIN PHARMACEUTICAL INC
Form 424B5
January 22, 2015
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Filed Pursuant to Rule 424(b)(5)
Registration No. 333-191604

CALCULATION OF REGISTRATION FEE

Title of securities to be registered	Amount to be registered(1)	Proposed maximum offering price per share	Proposed maximum aggregate offering price(1)	Amount of registration fee(2)
Common Stock, $0.001 par value	9,775,000	$93.25	$911,518,750	$105,918.48

(1) Includes up to 1,275,000 shares of common stock that may be purchased by the underwriters pursuant to their option to purchase additional shares.
(2) The filing fee is calculated in accordance with Rule 457(r) under the Securities Act of 1933, as amended, and relates to the Registration Statement on Form S-3 (File No. 333-191604) filed by the Registrant on October 7, 2013.

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PROSPECTUS SUPPLEMENT

(To prospectus dated October 7, 2013)

8,500,000 Shares

Common Stock

We are selling 8,500,000 shares of our common stock.

Our shares trade on the NASDAQ Global Select Market under the symbol BMRN. On January 21, 2015, the last sale price of our common stock as reported on the NASDAQ Global Select Market was $96.14 per share.

Investing in our common stock involves risks, including those described in the Risk Factors section beginning on page S-16 of this prospectus supplement and in our Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2014, which is incorporated herein by reference.

	Per Share	Total
Public offering price	$93.25	$792,625,000
Underwriting discount (1)	$2.3312	$19,815,200
Proceeds, before expenses, to us	$90.9188	$772,809,800

(1) We refer you to the Underwriting section of this prospectus supplement for additional information regarding underwriter compensation.

The underwriters may also exercise their option to purchase up to an additional 1,275,000 shares from us, at the public offering price, less the underwriting discount, for 30 days after the date of this prospectus supplement.

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 January 27, 2015.

BofA Merrill Lynch

J.P. Morgan **Morgan Stanley**

Barclays **Deutsche Bank Securities**

The date of this prospectus supplement is January 21, 2015.

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Prospectus Supplement

Prospectus

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

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ABOUT THIS PROSPECTUS SUPPLEMENT

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

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CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

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

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

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

The words anticipates, believes, estimates, expects, intends, may, plans, projects, will, would and similar expressions are inte forward-looking statements, although not all forward-looking statements contain these identifying words. We may not actually achieve the plans,

intentions or expectations disclosed in our forward-looking statements and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements that we make. We have identified some of the important factors that could cause future events to materially differ from our current expectations and they are described in this prospectus supplement under the caption Risk Factors as well as in our most recent Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2014. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make. We do not assume any obligation to update any forward-looking statement.

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PROSPECTUS SUPPLEMENT SUMMARY

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

BioMarin Pharmaceutical Inc.

Overview

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

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

We are conducting clinical trials on several investigational product candidates for the treatment of various diseases including: PEG PAL, an enzyme substitution therapy for the treatment of phenylketonuria (PKU); BMN 701, an enzyme replacement therapy for Pompe disease, a glycogen storage disorder; Talazoparib (BMN 673), an orally available poly-ADP ribose polymerase (PARP) inhibitor for the treatment of patients with certain cancers; BMN 111, a peptide therapeutic for the treatment of achondroplasia, the leading cause of dwarfism; and BMN 190 for the treatment of late infantile neuronal ceroid lipofuscinosis (CLN2), a lysomal storage disorder primarily affecting the brain. We are conducting or planning to conduct preclinical development of several other product candidates for genetic and other metabolic diseases and recently announced the selection of two new drug development candidates, BMN 270 and BMN 250. BMN 270 is a Factor VIII gene therapy drug development candidate, an AAV VIII vector, for the treatment of hemophilia A. We expect to initiate a Phase 1 study for BMN 270 in the first half of 2015. BMN 250 is a novel fusion of alpha-N-acetyglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo B syndrome, or Mucopolysaccharidosis type IIIB (MPS IIIB). We expect to initiate a Phase 1 study for BMN 250 in the second half of 2015.

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Summary of Commercial Products and Major Development Programs

A summary of our various commercial products and major development programs, including key metrics as of September 30, 2014, is provided below:

Commercial Products	Indication	Orphan Drug Exclusivity Expiration U.S.	Orphan Drug Exclusivity Expiration EU	Nine Months Ended September 30, 2014 Total Net Product Revenues (in millions)	Nine Months Ended September 30, 2014 Research & Development Expense (in millions)
Naglazyme	MPS VI (1)	Expired	September 2015	$ 246.0	$ 8.2
Kuvan	PKU (2)	June 2015	NA (11)	$ 145.6	$ 9.6
Aldurazyme (3)	MPS I (4)	Expired	Expired	$ 64.7	$ 1.2
Firdapse	LEMS (5)	NA (10)	2019	$ 14.0	$ 3.4
VIMIZIM	MPS IV A (6)	2021	2024	$ 40.4	$ 49.8

Products in Development	Target Indication	Stage	Nine Months Ended September 30, 2014 Research & Development Expense (in millions)
PEG PAL	PKU	Clinical Phase 3	$ 49.2
BMN 701	POMPE (7)	Clinical Phase 2/3	$ 35.2
BMN 673 (8)	BRCA BREAST CANCER	Clinical Phase 3	$ 42.7
BMN 111	ACHONDROPLASIA	Clinical Phase 2	$ 14.9
BMN 190	CLN2 (9)	Clinical Phase 1/2	$ 25.6

(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) BMN 673 is an orally available PARP inhibitor for the treatment of patients with certain cancers.
(9) CLN2, or late infantile neuronal ceroid lipofuscinosis, is a lysosomal storage disorder primarily affecting the brain.
(10) Firdapse has not received marketing approval in the U.S. and we have licensed the North American rights to develop and market Firdapse to a third party.
(11) Merck Serono markets Kuvan in the EU.

We expect our five marketed products to generate over $700 million in revenues in 2014.

Recent Developments

Paragraph IV Notice Letter from Dr. Reddy s Laboratories

As previously disclosed, we have received a paragraph IV notice letter, dated October 3, 2014, from Dr. Reddy s Laboratories, Inc. and Dr. Reddy s Laboratories, Ltd. (collectively, DRL), notifying us that DRL has filed an abbreviated new drug application (ANDA) seeking

approval of a proposed generic version of Kuvan

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(sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the U.S. Food and Drug Administration s (the FDA) Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book).

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

Acquisition of Prosensa Holding N.V.

On December 12, 2014, we commenced a tender offer to acquire all of the ordinary shares (Shares) of Prosensa Holding N.V., a public limited liability company (NASDAQ: RNA) organized under the laws of The Netherlands (Prosensa) in an all cash transaction for $17.75 per Share for an upfront purchase price of approximately $680.0 million. In addition, for each Prosensa Share purchased, we will issue one non-transferable contingent value right, which represents the contractual right to receive a cash payment of up to $4.14 per Share, or approximately $160.0 million, upon the achievement of certain product approval milestones.

On January 15, 2015, we closed the initial offering period relating to the offer and purchased approximately 93.4% of the Prosensa Shares. We immediately launched a subsequent offering period that is scheduled to expire on January 29, 2015. Following the expiration of the subsequent offering period, we intend to complete a corporate reorganization of Prosensa and its subsidiaries, which we expect will result in Prosensa or all of its assets becoming our indirect, wholly-owned subsidiary and all of the former Prosensa shareholders that did not tender receiving the same consideration that they would have if they had tendered into the original offer. On January 15, 2015, we paid approximately $637.5 million for the Prosensa Shares tendered at the closing of the initial offering period and options that vested pursuant to the definitive purchase agreement. We funded the acquisition with our available cash balances.

Prosensa is an innovative biotechnology company engaged in the discovery and development of ribonucleic acid (RNA)-modulating therapeutics for the treatment of genetic disorders. Prosensa s primary focus is on rare neuromuscular and neurodegenerative disorders with a large unmet medical need, including subsets of patients with Duchenne muscular dystrophy (DMD), myotonic dystrophy and Huntington s disease. Prosensa s clinical portfolio of RNA-based product candidates is focused on the treatment of DMD. Each of Prosensa s DMD compounds has been granted orphan drug status in the U.S. and the EU. Prosensa s lead product, drisapersen, is currently under a rolling review as part of a rolling new drug application with the FDA. Prosensa has announced that it expects to complete the filing of this application in the first quarter of 2015. Prosensa expects to file a marketing authorization application (MAA) for drisapersen with the EMA in the second quarter of 2015.

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

We expect to incur approximately $70.0-100.0 million in increased research and development expense and $60.0-70.0 million in additional accounting expense (related to the accretion of the CVR) in 2015 as a result of the acquisition.

For additional information on the transaction, please see our Current Reports on Form 8-K filed on November 24, 2014, November 26, 2014, December 10, 2014 and January 16, 2015, each of which is incorporated by reference in this prospectus supplement.

Commercial Products

VIMIZIM

VIMIZIM is an enzyme replacement therapy for the treatment of MPS IV A, a lysosomal storage disorder. MPS IV A is a disease characterized by deficient activity of Nacetylgalactosamine- 6-sulfatase

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(GALNS) causing excessive lysosomal storage of glycosaminoglycans such as keratan sulfate and chondroitin sulfate. This excessive storage causes a systemic skeletal dysplasia, short stature, and joint abnormalities, which limit mobility and endurance. Malformation of the chest impairs respiratory function, and looseness of joints in the neck cause spinal instability and potentially spinal cord compression. Other symptoms may include hearing loss, corneal clouding, and heart disease. Initial symptoms often become evident in the first five years of life. The disease substantially limits both the quality and length of life of those affected. We have identified approximately 1,650 patients worldwide suffering from MPS IV A and estimate that the total number of patients suffering from MPS IV A worldwide could be as many as 3,000.

VIMIZIM was granted marketing approval in the U.S. and the EU in February 2014 and April 2014, respectively, and subsequently in several other countries. We immediately began marketing VIMIZIM in the U.S. using our own existing sales force and commercial organization and have completed our first commercial sales in the U.S. and the EU as well as several other countries. We plan to pursue registration and/or market VIMIZIM on a named patient basis in other regions. Many countries allow for named patient or other early access sales based on the FDA approval. We plan to institute sales in these countries where appropriate.

VIMIZIM net product revenues for the three and nine months ended September 30, 2014 totaled $25.2 million and $40.4 million, respectively. VIMIZIM sales for 2013 totaled $0.1 million and $0.0 million for all prior periods.

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 three and nine months ended September 30, 2014 totaled $67.5 million and $246.0 million, respectively, as compared to $63.2 million and $202.5 million for the three and nine months ended September 30, 2013, respectively. Naglazyme net product revenues for 2013 totaled $271.2 million, as compared to $257.0 million and $224.9 million for 2012 and 2011, 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

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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 market exclusivity in the U.S. for the treatment of PKU, expiring in June 2015. We expect that our patents will provide market exclusivity beyond the expiration of orphan status. Kuvan net product revenues for the three and nine months ended September 30, 2014 totaled $53.4 million and $145.6 million, respectively, as compared to $43.6 million and $122.1 million for the three and nine months ended September 30, 2013, respectively. Kuvan net product revenues for 2013 totaled $167.4 million, as compared to $143.1 million and $116.8 million for 2012 and 2011, respectively.

In May 2005, we entered into an agreement with Merck Serono S.A. (Merck Serono), for the further development and commercialization of Kuvan and any other product containing 6R-BH4, and PEG PAL for PKU. Through the agreement, as amended in 2007, Merck Serono acquired exclusive rights to market these 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 the three and nine months ended September 30, 2014 we earned $0.5 million and $1.7 million, respectively, in net royalties on net sales of $13.4 million and $42.0 million, respectively, of Kuvan by Merck Serono, as compared to the three and nine months ended September 30, 2013, when we earned $0.6 million and $1.6 million on their net sales of $12.3 million and $38.1 million, respectively. In 2013, we earned $2.0 million in net royalties on net sales of $51.0 million of Kuvan by Merck Serono, as compared to 2012 when we earned $1.9 million in net royalties on net sales of $46.8 million. In 2011, we earned $1.6 million in net royalties on net sales of $40.4 million. We recorded collaborative agreement revenue associated with shared Kuvan development costs in the amounts of $0.3 million and $0.7 million for the three and nine months ended September 30, 2014, respectively, and $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., the EU and in other countries for patients with mucopolysaccharidosis I (MPS I). MPS I is a progressive and debilitating life-threatening genetic disease, for which no other drug treatment currently exists, that is caused by the deficiency of alpha-L-iduronidase, a lysosomal enzyme normally required for the breakdown of GAGs. Patients with MPS I typically become progressively worse and experience multiple severe and debilitating symptoms resulting from the build-up of

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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 three and nine months ended September 30, 2014 totaled $22.6 million and $64.7 million, respectively, as compared to $23.4 million and $57.7 million for the three and nine months ended September 30, 2013, respectively. Aldurazyme net product revenues for 2013 totaled $83.6 million, as compared to $82.2 million and $82.8 million for 2012 and 2011, respectively. The net product revenues for the three and nine months ended September 30, 2014, and for each of the years ended December 31, 2013, 2012 and 2011 include $22.9 million, $69.1 million, $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 $54.3 million and $172.5 million for the three and nine months ended September 30, 2014, respectively, $212.4 million for 2013, $193.1 million for 2012 and $185.2 million for 2011. For the three and nine months ended September 30, 2014, Aldurazyme net product revenue included previously recognized Aldurazyme net product transfer revenue of $0.3 million and $4.4 million, 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 and incremental product transfer revenue of $1.8 million and $8.6 million, respectively. Product transfer revenue represents a fixed amount per unit of Aldurazyme that Genzyme is required to pay us if the product is unsold by Genzyme and is recognized when the product is released to Genzyme and our performance obligations are fulfilled. The amount of product transfer revenue will eventually be deducted from the calculated royalty rate when the product is sold by Genzyme. 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 three and nine months ended September 30, 2014 totaled $4.7 million and $14.0 million, respectively, compared to $4.1 million and $11.8 million for the three and nine months ended September 30, 2013, respectively. Firdapse net product revenues for 2013 totaled $16.1 million, as compared to

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$14.2 million and $13.1 million for 2012 and 2011, 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.

Products in Clinical Development

Drisapersen

We acquired drisapersen, Prosensa s lead candidate for a subset of DMD, on January 15, 2015. See Prospectus Supplement Summary Recent Developments Acquistion of Prosensa Holding N.V.

DMD is one of the most prevalent rare genetic diseases globally, affecting approximately 1 in 3,500 boys, and is invariably fatal. There is currently no approved disease-modifying therapy for DMD. The progressive muscle-wasting that characterizes DMD is caused by inadequate production of dystrophin, a protein necessary for muscle function, as a result of mutations in the dystrophin gene. The different mutations, which are mostly deletions of one or more exons found in the dystrophin gene, result in distinct sub-populations of DMD patients. Drisapersen aims to address a specific mutation in the dystrophin gene that represents approximately 13% of all DMD patients, or approximately 10,000 patients. In clinical trials, drisapersen has been shown to induce dystrophin expression and has shown a treatment effect on DMD patients. To date, over 300 patients have participated in clinical studies of drisapersen at more than 50 trial sites in 25 countries.

Each of the Phase 2 trials of drisapersen showed a statistically significant change in a 6 minute walk test as compared to a placebo. The first Phase 2 trial showed a mean 32-meter improvement for the drisapersen group compared to a 4 meter decline in the placebo group (p=0.014). The second Phase 2 trial showed a mean 16-meter improvement for the drisapersen group compared to a mean 11-meter decline in the placebo group (p=0.069). When the results of these trials were compared in a post hoc analysis, the trials showed a mean 20-meter improvement for the drisapersen group compared to a mean 11-meter decline in the placebo group (p=.003). In the Phase 3 trial, the drisapersen group experienced a mean 43-meter decrease compared to a mean 53-meter decrease in the placebo group, although the result did not reach statistical significance (p=0.415). However, in the open label extension study, including patients who lost ambulation, patients receiving 6 mg/kg of drisapersen experienced a mean 25-meter decline on the 6 minute walk test at 177 weeks as compared to an expected 115-meter decline at 156 weeks, based on the natural history database.

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Based on this data and the rest of the clinical trials, in June 2014, Prosensa announced that the FDA had outlined a regulatory path forward for drisapersen. Following the feedback from the FDA, Prosensa confirmed that it would pursue a new drug application (NDA) filing for drisapersen with the FDA under an accelerated approval pathway based on existing data and in October 2014 Prosensa submitted the first module for an NDA regulatory filing for drisapersen to the FDA. Drisapersen was granted Fast Track status and breakthrough therapy designation from the FDA, making it eligible for a rolling review of the NDA. We intend to complete the submission of the NDA for drisapersen in the first quarter of 2015 and submit an MAA with the EMA in the second quarter of 2015.

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. A Phase 3 clinical trial of PEG PAL was initiated in June 2013. This ongoing Phase 3 clinical trial includes an open-label study to evaluate safety and blood Phe levels in naïve patients and a randomized controlled study of the Phase 2 extension study patients and patients from the open-label trial to evaluate blood Phe levels and neurocognitive endpoints. The FDA has indicated that lowering Phe blood levels in adults could support accelerated approval and, additionally, that a favorable outcome on prospectively-specified analyses of inattention in patients with baseline problems with attention could be strongly supportive of full approval. We expect to report results from these trials in the first quarter of 2016.

Talazoparib (BMN 673)

Talazoparib (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 talazoparib for the treatment of patients with solid tumors. This clinical trial is an open-label study of once daily, orally administered talazoparib in approximately 105 patients ages 18 and older with advanced or recurrent solid tumors. The study established a preliminary dose that is generally well-tolerated and reaches steady state with repeated daily doses. The study has focused on patients with breast and ovarian cancers characterized by deleterious BRCA-1 and -2 mutations, Ewing s sarcoma and small cell lung cancer, and has been expanded to include patients with prostate and pancreatic cancers. In June 2014, we presented an update on the study at the 2014 annual meeting of the American Society for Clinical Oncology. As presented, among 14 enrolled germline BRCA (gBRCA) mutated breast cancer patients treated at the recommended Phase 3 dose of 1mg/day, the confirmed RECIST response rate was 50% (seven confirmed objective responses: one complete and six partial). In addition, there were five patients with stable disease lasting at least 24 weeks for an overall clinical benefit response (CBR) rate at this dose of 86% (12/14). In the complete cohort of 18 gBRCA mutated breast cancer patients, which included six patients from the dose-escalation cohort at doses ranging from 900 µg/day to 1100 µg/day and 12 patients from

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the dose expansion cohort at a dose of 1.0 mg/day, the RECIST response rate was 44% (8/18), with one complete and seven partial responses. The CBR rate was 72% (13/18), with five patients having stable disease in excess of 24 weeks. The median progression-free survival (PFS) was 32 weeks in this heavily pre-treated advanced breast cancer population. Safety data continue to show that talazoparib is generally well tolerated with the most common drug-related toxicities being myelosuppression (including thrombocytopenia, anemia and neutropenia), mild to moderate fatigue, nausea and alopecia.

Based on the results of this Phase 1/2 study, we initiated a Phase 3 trial in patients with gBRCA mutated breast cancer in October 2013. The Phase 3 trial is an open-label, 2:1 randomized, parallel, two-arm study of talazoparib as compared to the protocol-specified physicians choice of chemotherapy in gBRCA mutated locally advanced and/or metastatic breast cancer patients who have received no more than two prior chemotherapy regimens for metastatic disease. The study is enrolling approximately 429 patients and is being conducted at approximately 140 sites in sixteen countries. The primary objective of the study is to compare progression-free survival (PFS) of patients treated with talazoparib as a monotherapy relative to those treated with protocol-specified physicians choice. The secondary objectives are to evaluate objective response rate (ORR), overall survival (OS), safety and the pharmacokinetics of talazoparib. We expect to complete enrollment of this Phase 3 trial in the second half of 2015.

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

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

BMN 701

BMN 701 is a novel fusion protein 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 up to 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

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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.

Health authorities, including the FDA and the EMA have indicated that MIP is a potentially approvable primary endpoint for our Phase 2/3 switching trial with BMN 701, assuming the results of the trial are compelling and clinically meaningful. This switching trial is designed to enroll late onset Pompe patients who have previously been treated with alglucosidase alfa. The trial has been initiated with the first patient enrolled in May of 2014. We are targeting to enroll approximately 20 patients in the first quarter of 2015 to establish proof-of-concept. We are currently working on improving the manufacturing process for BMN 701, which we expect will be our commercial manufacturing process, and will target to enroll up to 50 additional patients in a trial to be administered the drug manufactured with this improved process.

The BMN 701 program now includes, in addition to the above studies, an Observational Study as well as another Phase 2 study which is designed to support MIP as a primary endpoint. Both of these studies are also currently active and enrolling.

BMN 111

BMN 111 is a peptide therapeutic in development for the treatment of achondroplasia. In September 2012, we announced the results of a Phase 1 clinical trial for BMN 111. The primary objective of the Phase 1 clinical trial was to assess the safety and tolerability of single and multiple doses of BMN 111 in normal healthy adult volunteers up to the maximum tolerated dose. BMN 111 was generally well-tolerated over the range of single and repeat doses studied. Pharmacokinetic data indicated that the dose levels studied resulted in exposure levels that are expected to stimulate growth based on non-clinical findings. In January 2014, we announced the initiation of a Phase 2 clinical trial for BMN 111 for the treatment of children with achondroplasia. This international clinical trial is an open-label, sequential cohort, dose-escalation study of BMN 111 in children who are 5-14 years old. The primary objective of this study is to assess the safety and tolerability of daily subcutaneous doses of BMN 111 administered for 6 months. The secondary objectives will include an evaluation of change in annualized growth velocity, changes in absolute growth parameters, changes in body proportions and other medically relevant and functional aspects of achondroplasia, such as sleep apnea and joint range of motion. Prior to enrolling in the Phase 2 study, all patients will have participated in a six month natural history study to determine baseline growth velocity data. We completed enrollment in the first three cohorts of this study in November 2014. A total of 26 subjects have been enrolled in this trial for a treatment duration of six months. The protocol was recently amended to allow subjects who completed six months of treatment to be enrolled in an 18-month extension study. We plan to report 6-month data for the first three cohorts in the second quarter of 2015. We may decide to enroll one or more additional cohorts in this study after we review the data for the first three cohorts and meet with regulatory authorities.

BMN 190

BMN 190 is a recombinant human tripeptidyl peptidase 1 in development for the treatment of patients with CLN2, a form of Batten disease. CLN2 is an incurable, rapidly progressive disease that ends in patient death by 10-12 years of age. Patients are initially healthy but begin to decline at approximately the age of three. It is estimated that 400-600 cases exist worldwide, but CLN2 is believed to be underdiagnosed. In September 2013, we announced the initiation of a Phase 1/2 study for 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

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atrophy in comparison with CLN2 natural history after 48 weeks of treatment and to characterize pharmacokinetics and immunogenicity. This trial was fully enrolled in December 2014 with 24 patients. In January 2015, we announced interim data from the trial, which indicates that in all nine of the patients in the trial who have been followed for at least six months and up to 15 months, the treatment appears to show stabilization of the disease compared to the natural history based on a standardized measure of motor and language function. All patients are tolerating the therapeutic dose. These preliminary results have the potential to support the feasibility of a single-study filing with regulatory authorities. We expect to announce complete results in the fourth quarter of 2015.

Company Information

We were incorporated in Delaware in October 1996 and began operations on March 21, 1997. Our principal executive offices are located at 770 Lindaro Street, San Rafael, California 94901 and our telephone number is (415) 506-6700. Our annual reports on Form 10-K, quarterly reports on Form 10-Q, proxy statements, current reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act are available free of charge at *www.bmrn.com* as soon as reasonably practicable after electronically filing such reports with the SEC. Such reports and other information may be obtained by visiting the SEC s Public Reference Room at 100 F Street, NE, Washington, D.C. 20549 or by calling the SEC at 1-800-SEC-0330. Additionally, these reports are available at the SEC s website at *www.sec.gov*. Information contained in our website is not part of this prospectus supplement or accompanying prospectus, or any report that we file with or furnish to the SEC.

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THE OFFERING

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

Issuer	BioMarin Pharmaceutical Inc.
Common stock offered	8,500,000 shares
Common stock outstanding after the offering	155,933,951 shares
Option to purchase additional shares	The underwriters have an option to purchase up to 1,275,000 additional shares of our common stock at the public offering price less the underwriting discount. The underwriters may exercise this option at any time within 30 days of the date of this prospectus supplement.
Use of proceeds	We intend to use the net proceeds from this offering for general corporate purposes and to fund the acquisition of Prosensa and pay related fees and expenses. See Prospectus Supplement Summary Recent Developments Acquistion of Prosensa Holding N.V. 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 147,433,951 shares of common stock outstanding as of September 30, 2014 and does not take into account:

- 12,780,019 shares of our common stock issuable upon exercise of outstanding options issued under our equity incentive plans at a weighted average exercise price of $37.28 per share as of September 30, 2014;
- 1,530,508 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 $58.83 per share as of September 30, 2014;

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 September 30, 2014;

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2,237,726 shares of our common stock issuable upon the conversion of our $45.6 million 1.875% convertible subordinated notes due 2017 as of September 30, 2014;

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 September 30, 2014;

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 September 30, 2014; and

an aggregate of 20,265,930 shares of our common stock available for future equity awards under our equity incentive plans as of September 30, 2014.

Unless otherwise stated, all information contained in this prospectus supplement assumes no exercise of the underwriters option to purchase additional shares.

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RISK FACTORS

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

Risks Related to Our Business

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

We must obtain and maintain regulatory approval to market and sell our drug products in the U.S. and in jurisdictions outside of the U.S. In the U.S., we must obtain FDA approval for each drug that we intend to commercialize. The FDA approval process is typically lengthy and expensive, and approval is never certain. Products distributed abroad are also subject to government regulation by international regulatory authorities. The approval process in the EU and other countries can also be lengthy and expensive and regulatory approval is also never certain. Naglazyme, Aldurazyme and Kuvan have received regulatory approval to be commercially marketed and sold in the U.S., EU and other countries. Firdapse has received regulatory approval to be commercially marketed only in the EU. VIMIZIM received regulatory approval in the U.S. on February 14, 2014, in the EU on April 28, 2014, in Canada on July 7, 2014 and in Australia on December 2, 2014 but has not been approved in any other jurisdiction and may never receive additional regulatory approvals for any other jurisdiction.

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

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

From time to time during the regulatory approval process for our products and our product candidates, we engage in discussions with the FDA and comparable international regulatory authorities regarding the

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regulatory requirements for our development programs. To the extent appropriate, we accommodate the requests of the regulatory authorities and, to date, we have generally been able to reach reasonable accommodations and resolutions regarding the underlying issues. However, we are often unable to determine the outcome of such deliberations until they are final. If we are unable to effectively and efficiently resolve and comply with the inquiries and requests of the FDA and other non-U.S. regulatory authorities, the approval of our product candidates may be delayed and their value may be reduced.

After any of our products receive regulatory approval, they remain subject to ongoing regulation, which can impact, among other things product labeling, manufacturing practices, adverse event reporting, storage, expiration, distribution, advertising and promotion, record keeping and import and export. If we do not comply with the applicable regulations, the range of possible sanctions includes issuance of warning or untitled letters or adverse publicity, product recalls or seizures, fines, total or partial suspensions of production and/or distribution, suspension of marketing applications, and other enforcement actions, including injunctions and civil or criminal prosecution. The FDA and comparable international regulatory agencies can withdraw a product s approval under some circumstances, such as the failure to comply with regulatory requirements or unexpected safety issues. Further, the FDA often requires post-marketing testing and surveillance to monitor the effects of approved products. The FDA and comparable international regulatory agencies may condition approval of our product candidates on the completion of such post-marketing clinical studies. These post-marketing studies may suggest that a product causes undesirable side effects or may present a risk to the patient. If data we collect from post-marketing studies suggest that one of our approved products may present a risk to safety, the government authorities could withdraw our product approval, suspend production or place other marketing restrictions on our products. If regulatory sanctions are applied or if regulatory approval is delayed or withdrawn, the value of our company and our operating results will be adversely affected. Additionally, we will be unable to generate revenue from the sale of these products, our potential for generating positive cash flow will be diminished and the capital necessary to fund our operations will be increased.

If we fail to obtain or maintain orphan drug exclusivity for some of our products, our competitors may sell products to treat the same conditions and our revenues will be reduced.

As part of our business strategy, we intend to develop some drugs that may be eligible for FDA and EU orphan drug designation. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is intended to treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the U.S. The company that first obtains FDA approval for a designated orphan drug for a given rare disease receives marketing exclusivity for use of that drug for the stated condition for a period of seven years. Orphan drug exclusive marketing rights may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug. Similar regulations are available in the EU with a ten-year period of market exclusivity.

Because the extent and scope of patent protection for some of our drug products is limited, orphan drug designation is especially important for our products that are eligible for orphan drug designation. For eligible drugs, we plan to rely on the exclusivity period under the Orphan Drug Act to maintain a competitive position. If we do not obtain orphan drug exclusivity for our drug products that do not have broad patent protection, our competitors may then sell the same drug to treat the same condition and our revenues will be reduced.

Even though we have obtained orphan drug designation for certain of our products and product candidates and even if we obtain orphan drug designation for our future product candidates, due to the uncertainties associated with developing pharmaceutical products, we may not be the first to obtain marketing approval for any particular orphan indication, which means that we may not obtain orphan drug exclusivity and could also potentially be blocked from approval until the first product s orphan drug exclusivity period expires. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved and granted orphan drug exclusivity, the FDA can subsequently approve the same drug for the

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

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

Our Naglazyme, Aldurazyme and VIMIZIM products are regulated by the FDA as biologics under the Federal Food, Drug, and Cosmetic Act (the FDC Act), and the Public Health Service Act (the PHS Act). Biologics require the submission of a 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 biosimilar marketing applications.

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

As part of the regulatory approval process we must conduct, at our own expense, preclinical studies in the laboratory and clinical trials on humans for each product candidate. We expect the number of preclinical studies and clinical trials that the regulatory authorities will require will vary depending on the product candidate, the disease or condition the drug is being developed to address and regulations applicable to the particular drug. Generally, the number and size of clinical trials required for approval increase based on the expected patient population that may be treated with a drug. We may need to perform multiple preclinical studies using various doses and formulations before we can begin clinical trials, which could result in delays in our ability to market any of our product candidates. Furthermore, even if we obtain favorable results in preclinical studies, the results in humans may be significantly different. After we have conducted preclinical studies, we must demonstrate that our drug products are safe and efficacious for use in the targeted human patients in order to receive regulatory approval for commercial sale. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials, and favorable data from interim analyses do not ensure the final results of a trial will be favorable. Product candidates may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials, or despite having favorable data in connection with an interim analysis. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Our future clinical trial results may not be successful.

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

slow or insufficient patient enrollment;

slow recruitment of, and completion of necessary institutional approvals at, clinical sites;

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longer treatment time required to demonstrate efficacy;

lack of sufficient supplies of the product candidate;

adverse medical events or side effects in treated patients;

lack of effectiveness of the product candidate being tested; and

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

Typically, if a drug product is intended to treat a chronic disease, as is the case with some of our product candidates, safety and efficacy data must be gathered over an extended period of time, which can range from nine months to three years or more. We also rely on independent third-party CROs to perform most of our clinical studies and many important aspects of the services performed for us by the CROs are out of our direct control. If we fail to adequately manage our CROs, or if there is any dispute or disruption in our relationship with our CROs, our clinical trials may be delayed. Moreover, in our regulatory submissions, we rely on the quality and validity of the clinical work performed by third-party CROs. If any of our CROs processes, methodologies or results were determined to be not conducted in accordance with current good clinical practices, invalid or inadequate, our own clinical data and results and related regulatory approvals could adversely be impacted.

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

Since we began operations in March 1997, we have been engaged in substantial research and development and operated at a net loss until 2008. Although we were profitable in 2008, 2010 and the third quarter of 2014, we operated at a net loss in 2009, 2011, 2012 and 2013. Based upon our current plan for investments in research and development for existing and new programs, we expect to operate at a net loss for at least the next 12 months. Our future profitability depends on our marketing and selling of VIMIZIM, Naglazyme, Kuvan and Firdapse, the successful continued commercialization of Aldurazyme by Genzyme, the receipt of regulatory approval of our product candidates, our ability to successfully manufacture and market any approved drugs, either by ourselves or jointly with others, our spending on our development programs and the impact of any possible future business development transactions. The extent of our future losses and the timing of profitability are highly uncertain. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

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

Before we can begin commercial manufacture of our products, regulatory authorities must approve marketing applications that identify manufacturing facilities operated by us or our contract manufacturers that have passed regulatory inspection and manufacturing processes that are acceptable to the regulatory authorities. In addition, our pharmaceutical manufacturing facilities are continuously subject to inspection by the FDA and international regulatory authorities, before and after product approval. Our manufacturing facilities in the U.S. have been approved by the FDA, the EC, and health agencies in other countries for the manufacture of Aldurazyme and Naglazyme. In addition, our third-party manufacturers facilities involved with the manufacture of VIMIZIM, Naglazyme, Kuvan, Aldurazyme and Firdapse have also been inspected and approved by various regulatory authorities. Although we are not involved in the day-to-day operations of our contract manufacturers, we are ultimately responsible for ensuring that our products are manufactured in accordance with current Good Manufacturing Practices (cGMP) regulations. The manufacturing facility located in Shanbally, Cork, Ireland that we purchased in 2011 has not yet been approved by the FDA to manufacture any of our products, or the EMA. We intend to make a substantial investment in the build-out of the Shanbally facility in order to manufacture

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VIMIZIM and other products. If the facility is not ultimately approved by the FDA to manufacture any of our products 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 VIMIZIM, Naglazyme, Kuvan, Aldurazyme and Firdapse or our product candidates may be unable to comply with cGMP regulations in a cost-effective manner and may be unable to initially or continue to pass a federal or international regulatory inspection.

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

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

As of September 30, 2014, we had cash, cash equivalents and short and long-term investments totaling $1,114.7 million and long-term debt obligations of $795.6 million (undiscounted). On January 15, 2015, we closed the initial offering period relating to the tender offer to acquire Prosensa and paid $637.5 million to acquire approximately 93.4% of Prosensa s outstanding common shares. We expect to pay an additional $42.5 million to acquire the remaining shares and up to $160.0 million if certain development milestones are attained. In October 2013, we completed an offering of senior subordinated convertible notes and received net proceeds of approximately $696.4 million, after deducting commissions, estimated offering expenses payable by us and the purchase of the related capped calls. We will need cash to not only repay the principal amount of our 0.75% senior subordinated convertible notes due 2018 and 1.50% senior subordinated convertible notes due in 2020 (collectively, the Notes) but also the ongoing interest due on the Notes during their term. In March 2014, we completed an offering of 1.5 million shares of our common stock at a price of $78.45 per share. We received net proceeds of $117.5 million from that public offering; however, we may require additional financing to fund our future operations, including the commercialization of our approved drugs and drug product candidates currently under development, preclinical studies and clinical trials, and potential licenses and acquisitions. We may be unable to raise additional financing, if needed, due to a variety of factors, including our financial condition, the status of our product programs, and the general condition of the financial markets. If we fail to raise any necessary additional financing we may have to delay or terminate some or all of our product development programs and our financial condition and operating results will be adversely affected.

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

- our ability to successfully market and sell VIMIZIM, Naglazyme, Kuvan and Firdapse;
- Genzyme s ability to continue to successfully commercialize Aldurazyme;
- the progress and success of our preclinical studies and clinical trials (including studies and the manufacture of materials);
- the timing, number, size and scope of our preclinical studies and clinical trials;

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- the time and cost necessary to obtain regulatory approvals and the costs of post-marketing studies which may be required by regulatory authorities;

- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities;

- the progress of research programs carried out by us;

- our possible achievement of milestones identified in our purchase agreements with the former stockholders of LEAD Therapeutics, Inc., ZyStor, Huxley Pharmaceuticals, Inc., and Zacharon Pharmaceuticals Inc. that trigger related milestone payments;

- any changes made to, or new developments in, our existing collaborative, licensing and other commercial relationships or any new collaborative, licensing and other commercial relationships that we may establish; and

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

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

- additional licenses and collaborative agreements;

- additional contracts for product manufacturing; and

- additional financing facilities.

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

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

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

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

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

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

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

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

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

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

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

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Supply interruptions may disrupt our inventory levels and the availability of our products and product candidates and cause delays in obtaining regulatory approval for our product candidates, or harm our business by reducing our revenues.

Numerous factors could cause interruptions in the supply of our products and product candidates, including:

- timing, scheduling and prioritization of production by our contract manufacturers or a breach of our agreements by our contract manufacturers;
- labor interruptions;
- changes in our sources for manufacturing;
- the timing and delivery of shipments;
- our failure to locate and obtain replacement manufacturers as needed on a timely basis; and
- conditions affecting the cost and availability of raw materials.

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

With respect to our product candidates, production of product is necessary to perform clinical trials and successful registration batches are necessary to file for approval to commercially market and sell product candidates. Delays in obtaining clinical material or registration batches could adversely impact our clinical trials and delay regulatory approval for our product candidates.

Because the target patient populations for our products are small, we must achieve significant market share and maintain high per-patient prices for our products to achieve profitability.

All of our products target diseases with small patient populations. As a result, our per-patient prices must be relatively high in order to recover our development and manufacturing costs and achieve profitability. For Naglazyme and VIMIZIM, 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.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. These changes include aggregate reductions in Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and will remain in effect through 2024 unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and, accordingly, our financial operations.

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

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

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

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

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

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

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

Substantial new provisions affecting compliance have also been adopted, which may require us to modify our business practices with health care practitioners. The PPACA, among other things, requires drug manufacturers to collect and report information on payments or transfers of value to physicians and teaching hospitals, as well as investment and ownership interests held by physicians and their immediate family members

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during the preceding calendar year. Failure to submit required information may result in civil monetary penalties. Manufacturers were required to begin collecting required information on August 1, 2013 and the Centers for Medicare & Medicaid Services (CMS) made public the reported data in a searchable form on September 30, 2014. Manufacturers are required to submit reports to CMS by the 90th day of each subsequent calendar year.

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

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

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

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

- changes in international regulatory and compliance requirements that could restrict our ability to manufacture, market and sell our products;
- political and economic instability;
- diminished protection of intellectual property in some countries outside of the U.S.;
- trade protection measures and import or export licensing requirements;
- difficulty in staffing and managing international operations;
- differing labor regulations and business practices;
- potentially negative consequences from changes in or interpretations of tax laws;

changes in international medical reimbursement policies and programs;

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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.

Our international operations pose currency risks, which may adversely affect our operating results and net income.

A significant and growing portion of our revenues and earnings, as well as our substantial international net assets, are exposed to changes in foreign exchange rates. As we operate in multiple foreign currencies, including the euro, the Brazilian real, the U.K. pound, the Canadian dollar, the Swiss Franc, the Japanese yen and several other currencies, changes in those currencies relative to the U.S. dollar will impact our revenues and expenses. If the U.S. dollar were to weaken against another currency, assuming all other variables remained constant, our revenues would increase, having a positive impact on earnings, and our overall expenses would increase, having a negative impact on earnings. Conversely, if the U.S. dollar were to strengthen against another currency, assuming all other variables remained constant, our revenues would decrease, having a negative impact on earnings, and our overall expenses would decrease, having a positive impact on earnings. In addition, because our financial statements are reported in U.S. dollars, changes in currency exchange rates between the U.S. dollar and other currencies have had, and will continue to have, an impact on our results of operations. Therefore, significant changes in foreign exchange rates can impact our results and our financial guidance.

From time to time, we may implement currency hedges intended to reduce our exposure to changes in foreign currency exchange rates. However, our hedging strategies may not be successful, and any of our unhedged foreign exchange exposures will continue to be subject to market fluctuations. Moreover, when we do implement currency hedges, we only hedge our net exposure, or the difference between our revenues in a currency and the offsetting expenses in that currency. Since we do not generally hedge the portion of our revenues that has offsetting expenses in that currency, our revenues can be particularly affected by changes in exchange rates. These risks could cause a material adverse effect on our business, financial position and results of operations and could cause the market value of our common stock to decline.

If we are unable to protect our proprietary technology, we may not be able to compete as effectively.

Where appropriate, we seek patent protection for certain aspects of our technology. Patent protection may not be available for some of the products we are developing. If we must spend significant time and money protecting or enforcing our patents, designing around patents held by others or licensing, potentially for large fees, patents or other proprietary rights held by others, our business and financial prospects may be harmed.

The patent positions of biopharmaceutical products are complex and uncertain. The scope and extent of patent protection for some of our products and product candidates are particularly uncertain because key information on some of our product candidates has existed in the public domain for many years. The composition and genetic sequences of animal and/or human versions of Naglazyme, Aldurazyme and many of our product

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candidates have been published and are believed to be in the public domain. The chemical structure of BH4 (the active ingredient in Kuvan) and 3,4-DAP (the active ingredient in Firdapse) have also been published. Publication of this information may prevent us from obtaining or enforcing patents relating to our products and product candidates, including without limitation composition-of-matter patents, which are generally believed to offer the strongest patent protection.

We own or have licensed patents and patent applications related to VIMIZIM, Naglazyme, Kuvan, Aldurazyme and Firdapse. However, these patents and patent applications do not ensure the protection of our intellectual property for a number of reasons, including without limitation the following:

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

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

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

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

The Leahy-Smith America Invents Act of 2011, which reformed certain patent laws in the U.S., may create additional uncertainty. Among the significant changes are switching from a first-to-invent system to a first-to-file system, and the implementation of new procedures that permit competitors to challenge our patents in the 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.

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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 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

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

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

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

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

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

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

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

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

Our competitors compete with us to attract organizations for acquisitions, joint ventures, licensing arrangements or other collaborations. To date, several of our product programs have been acquired through acquisitions, such as BMN 701 and BMN 673 and several of our product programs have been developed through licensing or collaborative arrangements, such as Naglazyme, Aldurazyme, Kuvan and Firdapse. These collaborations include licensing proprietary technology from, and other relationships with, academic research institutions. Our future success will depend, in part, on our ability to identify additional opportunities and to successfully enter into partnering or acquisition agreements for those opportunities. If our competitors successfully enter into partnering arrangements or license agreements with academic research institutions, we will then be precluded from pursuing those specific opportunities. Since each of these opportunities is unique, we may not be able to find a substitute. Several pharmaceutical and biotechnology companies have already established themselves in the field of genetic diseases. These companies have already begun many drug development programs, some of which may target diseases that we are also targeting, and have already entered into partnering and licensing arrangements with academic research institutions, reducing the pool of available opportunities.

Universities and public and private research institutions also compete with us. While these organizations primarily have educational or basic research objectives, they may develop proprietary technology and acquire patents that we may need for the development of our product candidates. We will attempt to license this proprietary technology, if available. These licenses may not be available to us on acceptable terms, if at all. If we are unable to compete successfully with respect to acquisitions, joint venture and other collaboration opportunities, we may be limited in our ability to develop new products and to continue to expand our product pipeline.

If generic manufacturers use litigation and regulatory means to obtain approval for generic versions of Kuvan, our revenue and results of operations would be adversely affected.

The Drug Price Competition and Patent Term Restoration Act of 1984, known as the Hatch-Waxman Act, permits the FDA to approve ANDAs for generic versions of branded drugs. We refer to this process as the ANDA process. The ANDA process permits competitor companies to obtain marketing approval for a drug with the same active ingredient as a branded drug, but does not generally require the conduct and submission of clinical efficacy studies for the generic product. In place of such clinical studies, an ANDA applicant usually needs only to submit data demonstrating that its product is bioequivalent to the branded product. Pursuant to the Hatch-Waxman Act, companies were permitted to file ANDA applications for proposed generic versions of Kuvan® (sapropterin hydrochloride) at any time after December 2011.

BioMarin owns several patents that cover Kuvan (sapropterin dihydrochloride), and we have listed those patents in conjunction with that product in the Orange Book. The Hatch-Waxman Act requires an ANDA applicant that seeks FDA approval of its proposed generic product prior to the expiration of our Orange Book-listed patents to certify that the applicant believes that our patents are invalid or will not be infringed by the manufacture, use or sale of the drug for which the application has been submitted (a paragraph IV certification) and notify us of such certification. Upon receipt of such a notice, the Hatch-Waxman Act allows us, with proper basis, to bring an action for patent infringement against the ANDA filer, asking that the proposed generic product not be approved until after our patents expire. If we commence a lawsuit within 45 days from receipt of the notice of a paragraph IV certification, the Hatch-Waxman Act provides a 30-month stay, during which time the FDA cannot finally approve the generic s application. If the litigation is resolved in favor of the ANDA applicant during the 30-month stay period, the stay is lifted and the FDA s review of the application may be completed. The discovery, trial and appeals process in such a lawsuit is costly, time consuming, and may result in generic competition if the ANDA applicant prevails. Regardless of any litigation results, generic versions of Kuvan (sapropterin dihydrochloride) would be prohibited until the expiration of orphan drug exclusivity in June 2015,

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including pediatric exclusivity, at the earliest. We have also received three-year Hatch-Waxman exclusivity for a New Patient Population for Kuvan (sapropterin dihydrochloride) that expires in October 2017, including pediatric exclusivity. Thus, depending on the proposed labeling of a generic product, generic versions of Kuvan (sapropterin dihydrochloride) may be prohibited until October 2017, though it is possible that an ANDA applicant could propose to carve out information in the Kuvan labeling protected by the New Patient Population exclusivity and obtain approval earlier.

We have received a paragraph IV notice letter, dated October 3, 2014, from DRL, notifying us that DRL has filed an ANDA seeking approval of a proposed generic version Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our Orange Book-listed patents. On November 17, 2014, we, together with Merck & Cie, filed a lawsuit against DRL in the United States District Court for the District of New Jersey alleging patent infringement for our patents relating to Kuvan.

The filing of DRL s ANDA in respect to Kuvan (sapropterin dihydrochloride) could have an adverse impact on our stock price, and litigation to enforce our patents is likely to cost a substantial amount and require significant management attention. If the patents covering Kuvan (sapropterin dihydrochloride) and its use are not upheld in litigation, or if DRL is found to not infringe our asserted patents, the resulting generic competition following the expiration of regulatory exclusivity would have a material adverse effect on our revenue and results of operations.

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

For planning purposes, we estimate the timing of the accomplishment of various scientific, clinical, regulatory and other product development goals, which we sometimes refer to as milestones. These milestones may include the commencement or completion of scientific studies and clinical trials and the submission of regulatory filings. From time to time, we publicly announce the expected timing of some of these milestones. All of these milestones are based on a variety of assumptions. The actual timing of these milestones can vary dramatically compared to our estimates, in many cases for reasons beyond our control. If we do not meet these milestones as publicly announced, the commercialization of our products may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

We depend upon our key personnel and our ability to attract and retain employees.

Our future growth and success will depend in large part on our continued ability to attract, retain, manage and motivate our employees. The loss of the services of any member of our senior management or the inability to hire or retain experienced management personnel could adversely affect our ability to execute our business plan and harm our operating results.

Because of the specialized scientific and managerial nature of our business, we rely heavily on our ability to attract and retain qualified scientific, technical and managerial personnel. In particular, the loss of one or more of our senior executive officers could be detrimental to us if we do not have an adequate succession plan or if we cannot recruit suitable replacements in a timely manner. While our senior executive officers are parties to employment agreements with us, these agreements do not guarantee that they will remain employed with us in the future. In addition, in many cases, these agreements do not restrict our senior executive officers ability to compete with us after their employment is terminated. The competition for qualified personnel in the pharmaceutical field is intense, and there is a limited pool of qualified potential employees to recruit. Due to this intense competition, we may be unable to continue to attract and retain qualified personnel necessary for the development of our business or to recruit suitable replacement personnel. If we are unsuccessful in our recruitment and retention efforts, our business may be harmed.

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Our success depends on our ability to manage our growth.

Product candidates that we are currently developing or may acquire in the future may be intended for patient populations that are significantly larger than any of MPS I, MPS VI, PKU or LEMS. In order to continue development and marketing of these products, if approved, we will need to significantly expand our operations. To manage expansion effectively, we need to continue to develop and improve our research and development capabilities, manufacturing and quality capacities, sales and marketing capabilities, financial and administrative systems and standard processes for global operations. Our staff, financial resources, systems, procedures or controls may be inadequate to support our operations and may increase our exposure to regulatory and corruption risks and our management may be unable to manage successfully future market opportunities or our relationships with customers and other third-parties.

Changes in methods of treatment of disease could reduce demand for our products and adversely affect revenues.

Even if our drug products are approved, if doctors elect a course of treatment which does not include our drug products, this decision would reduce demand for our drug products and adversely affect revenues. For example, if gene therapy becomes widely used as a treatment of genetic diseases, the use of enzyme replacement therapy, such as Naglazyme, VIMIZIM, and Aldurazyme in MPS diseases, could be greatly reduced. Changes in treatment method can be caused by the introduction of other companies products or the development of new technologies or surgical procedures which may not directly compete with ours, but which have the effect of changing how doctors decide to treat a disease.

If product liability lawsuits are successfully brought against us, we may incur substantial liabilities.

We are exposed to the potential product liability risks inherent in the testing, manufacturing and marketing of human pharmaceuticals. We currently maintain insurance against product liability lawsuits for the commercial sale of our products and for the clinical trials of our product candidates. Pharmaceutical companies must balance the cost of insurance with the level of coverage based on estimates of potential liability. Historically, the potential liability associated with product liability lawsuits for pharmaceutical products has been unpredictable. Although we believe that our current insurance is a reasonable estimate of our potential liability and represents a commercially reasonable balancing of the level of coverage as compared to the cost of the insurance, we may be subject to claims in connection with our clinical trials and commercial use of VIMIZIM, Naglazyme, Kuvan, Aldurazyme and Firdapse, or our clinical trials for 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

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disruptions in systems, misappropriation of our confidential or otherwise protected information and corruption of data. Cybersecurity incidents resulting in the failure of our enterprise resource planning system, production management or other systems to operate effectively or to integrate with other systems, or a breach in security or other unauthorized access of these systems, may affect our ability to manage and maintain our inventory and internal reports, and result in delays in product fulfillment and reduced efficiency of our operations. A breach in security, unauthorized access resulting in misappropriation, theft, or sabotage with respect to our proprietary and confidential information, including research or clinical data, could require significant capital investments to remediate and could adversely affect our business, financial condition and results of operations.

Our business is affected by macroeconomic conditions.

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

For the three and nine months ended September 30, 2014 approximately 6% and 4%, respectively, of our net product revenues were from the countries of Italy, Spain, Portugal, Greece and Russia. Approximately 9% of our total accounts receivable as of September 30, 2014 related to such countries and we have included an allowance for doubtful accounts for certain accounts receivable from Greece. If the financial conditions of these countries continues to decline, a substantial portion of the receivables may be uncollectable, which would mean we would have to provide for additional allowances for doubtful accounts or cease selling products in these countries, either of which could adversely affect our results of operations. Additionally, if one or more of these countries were unable to purchase our products, our revenue would be adversely affected. We also sell our products in other countries that face economic crises and local currency devaluation. Although we have historically collected receivables from customers in those countries, sustained weakness or further deterioration of the local economies and currencies may cause our customers in those countries to be unable to pay for our products with the same negative effect on our operations.

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

Recent and future regulatory actions and other events may adversely affect the trading price and liquidity of our senior subordinated convertible notes.

We expect that many investors in, and potential purchasers of, the Notes will employ, or seek to employ, a convertible arbitrage strategy with respect to the Notes. Investors would typically implement such a strategy by selling short the common stock underlying the Notes and dynamically adjusting their short position while continuing to hold the Notes. Investors may also implement this type of strategy by entering into swaps on our common stock in lieu of or in addition to short selling the common stock.

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The SEC and other regulatory and self-regulatory authorities have implemented various rules and taken certain actions, and may in the future adopt additional rules and take other actions, that may impact those engaging in short selling activity involving equity securities (including our common stock). Such rules and actions include Rule 201 of SEC Regulation SHO, the adoption by the Financial Industry Regulatory Authority, Inc. of a Limit Up-Limit Down program, the imposition of market-wide circuit breakers that halt trading of securities for certain periods following specific market declines, and the implementation of certain regulatory reforms required by the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010. Any governmental or regulatory action that restricts the ability of investors in, or potential purchasers of, the Notes to effect short sales of our common stock or enter into swaps on our common stock could adversely affect the trading price and the liquidity of the Notes.

In addition, if investors and potential purchasers seeking to employ a convertible arbitrage strategy are unable to borrow or enter into swaps on our common stock, in each case on commercially reasonable terms, the trading price and liquidity of the Notes may be adversely affected.

Risks Related to our Acquisition of Prosensa Holding N.V.

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

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

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

Many of these risks may be accentuated because the majority of Prosensa s operations, employees and customers are located outside of the United States. Any one or all of these factors may increase operating costs or lower anticipated financial performance. Many of these factors are also outside of our control. Achieving anticipated synergies and the potential benefits underlying our reasons for the acquisition will depend on successful integration of the businesses. The failure to integrate the business operations of Prosensa successfully would have a material adverse effect on our business, financial condition and results of operations.

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

Even if the integration is successful, we have made certain assumptions relating to the impact on our capital structure and financial results in respect of the acquisition. These assumptions relate to numerous matters, including:

- our expected capital structure after the acquisition;

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- the amount of goodwill and intangibles that will result from the acquisition;
- certain other purchase accounting adjustments that we expect will be recorded in our financial statements in connection with the acquisition;
- acquisition costs, including restructuring charges and transaction costs; and
- other financial and strategic risks of the acquisition.

Irrespective of our assumptions, we may incur higher than expected operating, transaction and integration costs, and we may encounter general economic and business conditions that adversely affect the combined company following the acquisition. If one or more of these assumptions are incorrect, it could have an adverse effect on our business and operating results, and the perceived benefits from the acquisition may not be realized.

We may have exposure to additional tax liabilities as a result of the acquisition.

As a multinational corporation, we are subject to income taxes as well as non-income based taxes, in both the United States and various foreign jurisdictions. Significant judgment is required in determining our worldwide provision for income taxes and other tax liabilities. Changes in tax laws or tax rulings may have a significantly adverse impact on our effective tax rate. Proposals by the current U.S. administration for fundamental U.S. international tax reform, including without limitation provisions that would limit the ability of U.S. multinationals to defer U.S. taxes on foreign income, if enacted, could have a significant adverse impact on our effective tax rate following the acquisition.

We are subject to a variety of additional risks as a result of the acquisition that may negatively impact our operations.

As a result of the acquisition, we are subject to new and additional risks associated with the business and operations of Prosensa and its global operations. The additional risks we may be exposed to include but are not limited to the following:

- tariffs and trade barriers;
- regulations related to customs and import/export matters (including sanctions);
- longer payment cycles;
- tax issues, such as tax law changes and variations in tax laws as compared to the jurisdictions in which we already operate;
- operating under regulations in new jurisdictions related to obtaining eligibility for government or private payor reimbursement for our products at the wholesale/retail level;
- cultural and language differences in the new jurisdictions in which we will operate;
- complying with additional employment regulations in the new jurisdictions in which we will operate; and

risks related to crimes, strikes, riots, civil disturbances, terrorist attacks and wars in new geographical locations.

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We cannot assure you that we will be able to adequately address these additional risks. If we are unable to do so, our operations might suffer.

Additionally, although prior to the acquisition we had international operations, as a result of the acquisition, we operate on an expanded global basis with additional offices or activities in Europe. We will face increased exposure to risks inherent in conducting business internationally, including compliance with international laws and regulations and laws and regulations of the United States and various other countries that apply to our international operations. Compliance with these laws and regulations may increase our cost of doing business in foreign jurisdictions. These laws and regulations include laws relating to the pharmaceutical industry, data privacy requirements, labor relations laws, tax laws, anti-competition regulations, import and trade restrictions, export requirements, U.S. laws such as the Foreign Corrupt Practices Act, other U.S. federal statutes and regulations, including those established by the Office of Foreign Assets Control, and local laws which prohibit payments to governmental officials. Given the high level of complexity of these laws, however, there is a risk that some provisions may be inadvertently breached by BioMarin, for example through fraudulent or negligent behavior of individual employees, our failure to comply with certain formal documentation requirements, or otherwise. Violations of these laws and regulations could result in fines, criminal sanctions against us, our officers or our employees, requirements to obtain export licenses, cessation of business activities in sanctioned countries, implementation of compliance programs, and prohibitions on the conduct of our business. Any such violations could include prohibitions on our ability to offer our products in one or more countries and could materially damage our reputation, our brand, our international expansion efforts, our ability to attract and retain employees, our business and our operating results. Our success depends, in part, on our ability to anticipate these risks and manage these challenges. These factors or any combination of these factors may adversely affect our revenue or our overall financial performance.

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

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

Prosensa depends heavily on the success of drisapersen. Drisapersen is still in clinical development. If we are unable to commercialize drisapersen or experience significant delays in doing so, our business, financial condition and results of operations will be materially adversely affected.

Our ability to generate product revenues from Prosensa will depend heavily on the successful development and eventual commercialization of drisapersen.

In September 2013, Prosensa announced that the Phase 3 clinical trial of drisapersen did not meet its primary endpoint. Although we believe that the collective data from Prosensa s various Phase 2 and Phase 3 clinical trials of drisapersen, including retrospective and subgroup analyses, provide strong support for concluding that drisapersen showed clinically meaningful improvements over placebo in these trials, we cannot be sure that Prosensa s data will be sufficient to satisfy the EMA or the FDA. We may need to conduct additional clinical trials at significant delay and cost or abandon development of drisapersen altogether.

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

- we may not achieve market acceptance of drisapersen by physicians, patients and third-party payors;

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drisapersen may not have an acceptable safety profile following approval;

we may not be able to manufacture drisapersen in compliance with requirements of the EMA, the FDA and similar regulatory agencies in commercial quantities sufficient to meet market demand;

we may not achieve sufficient pricing for drisapersen to compensate for future development and commercialization costs and to recoup our cost to acquire Prosensa;

we may not compete successfully with any alternative therapies for DMD; and

we may not successfully enforce and defend our intellectual property rights and claims.

The occurrence of any of these events could materially adversely affect our business, financial condition and results of operations.

Our conclusions regarding the efficacy of drisapersen are based on retrospective analyses of the results of Prosensa s clinical trials, and these analyses may be considered less reliable indicators of efficacy than pre-specified analyses.

After determining that it did not achieve the primary efficacy endpoint in the completed Phase 3 clinical trial of drisapersen, Prosensa performed retrospective and subgroup analyses of the Phase 3 clinical trial and prior Phase 2 clinical trials of drisapersen that we believe provide strong support for concluding that drisapersen showed clinically meaningful improvements over placebo in these trials. Although Prosensa believed that these additional analyses were warranted, a retrospective analysis performed after unblinding trial results can result in the introduction of bias if the analysis is inappropriately tailored or influenced by knowledge of the data and actual results. Because of these limitations, regulatory authorities typically give greatest weight to results from pre-specified analyses and less weight to results from post-hoc, retrospective analyses. Thus, this increases the likelihood that we will have to conduct an additional clinical trial or trials of drisapersen before we can apply for marketing approval.

Because Prosensa is developing product candidates for the treatment of diseases in which there is little clinical experience and, in some cases, using new endpoints or methodologies, there is more risk that the outcome of clinical trials for Prosensa s product candidates will not be favorable.

There is currently no approved disease-modifying therapy for DMD. In addition, there has been limited historical clinical trial experience generally for the development of drugs to treat the underlying cause of DMD. As a result, the design and conduct of clinical trials for this disease, particularly for drugs to address the underlying cause of this disease, are subject to increased risks. In particular, regulatory authorities in the United States and the EU have not issued definitive guidance as to how to measure and achieve efficacy.

In the last several years, the six minute walk test (the 6MWT) has been used in several trials of product candidates for patients with DMD, and is accepted by U.S. and European regulators to be an appropriate primary outcome measure for DMD trials. Because of the limited clinical experience in this indication however, regulators have not yet established what difference in the distance walked in the 6MWT (6MWD) is required to be demonstrated in a clinical trial of a DMD therapy in order to signify a clinically meaningful result and/or obtain regulatory approvals. As a result, it is not clear what is required in terms of 6MWD or other end points to obtain regulatory approval for drisapersen and Prosensa s other product candidates. If we are required to conduct additional clinical trials of drisapersen, the design of such trials could be subject to such uncertainties.

We could also face similar challenges in designing clinical trials and obtaining regulatory approval for future product candidates, including any that we may develop for myotonic dystrophy or Huntington s disease because there is also limited historical clinical trial experience for the development of drugs to treat these diseases.

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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 VIMIZIM, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- manufacture, supply or distribution of VIMIZIM, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- announcements related to the acquisition of Prosensa
- progress of our product candidates through the regulatory process and our ability to successfully commercialize any such products that receive regulatory approval;
- results of clinical trials, announcements of technological innovations or new products by us or our competitors;
- results relating to our lawsuit against DRL to protect our patents relating to Kuvan;
- government regulatory action affecting our product candidates or our competitors drug products in both the U.S. and non-U.S. countries;
- developments or disputes concerning patent or proprietary rights;
- general market conditions and fluctuations for the emerging growth and pharmaceutical market sectors;
- economic conditions in the U.S. or abroad;
- broad market fluctuations in the U.S., the EU or in other parts of the world;
- actual or anticipated fluctuations in our operating results; and
- changes in company assessments or financial estimates by securities analysts.

In the past, following periods of large price declines in the public market price of a company s securities, securities class action litigation has often been initiated against that company. Litigation of this type could result in substantial costs and diversion of management s attention and resources, which would hurt our business. Any adverse determination in litigation could also subject us to significant liabilities. In addition, our

stock price can be materially adversely affected by factors beyond our control, such as disruptions in global financial markets or negative trends in the biotechnology sector of the economy, even if our business is operating well.

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

We are incorporated in Delaware. Certain anti-takeover provisions of Delaware law and our charter documents as currently in effect may make a change in control of our company more difficult, even if a change in control would be beneficial to the stockholders. Our anti-takeover provisions include provisions in our

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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 for general corporate purposes and to fund the acquisition of Prosensa. We reserve the right, at the sole discretion of our Board of Directors, to reallocate our use of proceeds in response to developments in our business. Accordingly, our management will have significant discretion in applying these proceeds and could spend the proceeds in ways that do not necessarily improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business or financial condition, cause the price of our common stock to decline and delay product development.

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USE OF PROCEEDS

We estimate that the net proceeds from the sale of 8,500,000 shares of our common stock in this offering will be approximately $772.3 million, or approximately $888.2 million if the underwriters exercise their option to purchase 1,275,000 additional shares in full, after deducting the underwriting discounts and commissions and the other estimated offering expenses that we are to pay.

We intend to use the net proceeds from this offering for general corporate purposes and to fund the acquisition of Prosensa and pay related fees and expenses. See Prospectus Supplement Summary Recent Developments Acquisition of Prosensa Holding N.V. 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.

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PRICE RANGE OF COMMON STOCK

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

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

	High	Low
Year Ending December 31, 2015		
First Quarter (through January 21, 2015)	$ 100.50	$ 88.51
Year Ending December 31, 2014		
Fourth Quarter	$ 96.36	$ 65.91
Third Quarter	$ 73.35	$ 55.36
Second Quarter	$ 70.42	$ 55.04
First Quarter	$ 84.25	$ 64.61
Year Ended December 31, 2013		
Fourth Quarter	$ 76.02	$ 58.65
Third Quarter	$ 80.67	$ 56.31
Second Quarter	$ 71.56	$ 53.53
First Quarter	$ 62.96	$ 49.71
Year Ended December 31, 2012		
Fourth Quarter	$ 52.96	$ 36.28
Third Quarter	$ 44.18	$ 36.50
Second Quarter	$ 39.64	$ 31.91
First Quarter	$ 38.95	$ 33.61

The last reported sale price of our common stock on the NASDAQ Global Select Market on January 21, 2015 was $96.14 per share. As of September 30, 2014, there were 53 holders of record of our common stock.

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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.

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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 defined 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;

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.

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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 advisors 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, IRS Form W-8BEN-E, or IRS 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;

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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

Pursuant to Sections 1471 to 1474 of the Code and the Treasury regulations promulgated thereunder (FATCA), dividends paid in respect of our common stock, and, after December 31, 2016, gross proceeds from the sale or other disposition of our common stock held by or through certain foreign financial institutions (as specially defined for purposes of these rules, including investment funds) will be subject to withholding at a rate

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of 30%, unless (1) such institution enters into an agreement with the 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 (2) such institution 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. A foreign financial institution or non-financial foreign entity can generally meet the certification requirements by providing a properly executed IRS Form W-8BEN, IRS Form W-8BEN-E, or IRS Form W-8ECI, as applicable. 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.

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UNDERWRITING

Merrill Lynch, Pierce, Fenner & Smith Incorporated is acting as representative of each of the underwriters named below. Subject to the terms and conditions set forth in an underwriting agreement among us and the underwriters, we have agreed to sell to the underwriters, and each of the underwriters has agreed, severally and not jointly, to purchase from us, the number of shares of common stock set forth opposite its name below.

Underwriter	Number of Shares
Merrill Lynch, Pierce, Fenner & Smith Incorporated	3,400,000
J.P. Morgan Securities LLC	1,487,500
Morgan Stanley & Co. LLC	1,487,500
Barclays Capital Inc.	1,062,500
Deutsche Bank Securities Inc.	1,062,500
Total	8,500,000

Subject to the terms and conditions set forth in the underwriting agreement, the underwriters have agreed, severally and not jointly, to purchase all of the shares sold under the underwriting agreement if any of these shares are purchased. If an underwriter defaults, the underwriting agreement provides that the purchase commitments of the non-defaulting underwriters may be increased or the underwriting agreement may be terminated.

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

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

Commissions and Discounts

The representative has advised us that the underwriters propose initially to offer the shares to the public at the public offering price set forth on the cover page of this prospectus supplement and to dealers at that price less a concession not in excess of $1.25 per share. After the initial offering, the public offering price, concession or any other term of the offering may be changed. Sales of shares made outside of the United States may be made by affiliates of the underwriters.

The following table shows the public offering price, underwriting discount and proceeds before expenses to us. The information assumes either no exercise or full exercise by the underwriters of their option to purchase additional shares.

	Per Share	Without Option	With Option
Public offering price	$93.25	$792,625,000	$911,518,750
Underwriting discount	$2.3312	$19,815,200	$22,787,480
Proceeds, before expenses, to us	$90.9188	$772,809,800	$888,731,270

The expenses of the offering payable by us, not including the underwriting discount, are estimated at $520,000.

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Option to Purchase Additional Shares

We have granted an option to the underwriters, exercisable for 30 days after the date of this prospectus supplement, to purchase up to 1,275,000 additional shares of our common stock at the public offering price, less the underwriting discount, set forth on the cover page of this prospectus. If the underwriters exercise this option, each will be obligated, subject to conditions contained in the underwriting agreement, to purchase a number of additional shares proportionate to that underwriter s initial amount reflected in the above table.

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 an securities convertible into or exercisable or exchangeable for our common stock until 90 days with respect to us and the earlier of March 8, 2015 or 45 days with respect to our executive officers and directors 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.

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.

Price Stabilization; Short Positions

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

In connection with this offering, the underwriters 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

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short sales and stabilizing transactions. Short sales involve the sale by the underwriters of a greater number of shares than they are required to purchase in this offering. Covered short sales are sales made in an amount not greater than the underwriters option to purchase additional shares described above. The underwriters may close out any covered short position by either exercising their option to purchase additional shares or purchasing shares in the open market. 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 option granted to them. Naked short sales are 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 this 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 offering.

Similar to other purchase transactions, the underwriters purchases to cover the syndicate 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 underwriters may conduct these transactions on the NASDAQ Global Select Market, in the over-the-counter market or otherwise.

Neither we nor any of the underwriters 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 any of the underwriters make any representation that the representative 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 underwriters 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 underwriters are 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, certain of the underwriters or securities dealers may distribute prospectuses by electronic means, such as e-mail.

Other Relationships

Some of the underwriters and their 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. They have 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 underwriters and their 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 underwriters and their affiliates may also make investment recommendations and/or publish or

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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 sh