

BIOMARIN PHARMACEUTICAL INC

Form 424B5

May 30, 2012

**Table of Contents**

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

**Filed Pursuant to Rule 424(b)(5)  
File Number 333-181766**

**Subject to Completion**

**Preliminary Prospectus Supplement dated May 30, 2012**

**PROSPECTUS SUPPLEMENT**

**(To prospectus dated May 30, 2012)**

**6,500,000 Shares**

**Common Stock**

We are selling 6,500,000 shares of our common stock.

Our shares trade on the Nasdaq Global Select Market under the symbol BMRN. On May 29, 2012, the last sale price of the shares as reported on the Nasdaq Global Select Market was \$39.06 per share.

**Investing in the common stock involves risks, including those described in the Risk Factors section beginning on page S-12 of this prospectus supplement.**

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

## Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 424B5

The underwriters may exercise their option to purchase up to an additional 650,000 shares from us, at the price per share set forth above 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 June , 2012.

**BofA Merrill Lynch**

**Barclays**

The date of this prospectus supplement is , 2012.

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

	<b>Page</b>
<u>About this Prospectus Supplement</u>	S-1
<u>Cautionary Note Regarding Forward-Looking Statements</u>	S-2
<u>Prospectus Supplement Summary</u>	S-3
<u>The Offering</u>	S-10
<u>Risk Factors</u>	S-12
<u>Use of Proceeds</u>	S-32
<u>Price Range of Common Stock</u>	S-33
<u>Dividend Policy</u>	S-34
<u>Material U.S. Federal Income Tax Considerations for Non-U.S. Holders</u>	S-35
<u>Underwriting</u>	S-39
<u>Legal Matters</u>	S-45
<u>Experts</u>	S-45

**Prospectus**

<u>About this Prospectus</u>	1
<u>BioMarin Pharmaceutical Inc.</u>	2
<u>Risk Factors</u>	3
<u>Cautionary Note Regarding Forward-Looking Statements</u>	4
<u>Where You Can Find More Information</u>	5
<u>Information Incorporated by Reference</u>	6
<u>Use of Proceeds</u>	7
<u>Description of Capital Stock</u>	8
<u>Plan of Distribution</u>	9
<u>Legal Matters</u>	11
<u>Experts</u>	11

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 the prospectus entitled Where You Can Find More Information and Information Incorporated by Reference.

General information about us can be found on our website at <http://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, or the SEC.

**Table of Contents**

**ABOUT THIS PROSPECTUS SUPPLEMENT**

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

S-1

**Table of Contents**

**CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS**

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

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

our expectations with respect to regulatory submissions and approvals and our clinical trials;

any projection or expectation of earnings, revenue or other financial items;

the plans, strategies and objectives of management for future operations;

factors that may affect our operating results;

new products or services;

the demand for our products;

our ability to consummate acquisitions and successfully integrate them into our operations;

future capital expenditures;

effects of current or future economic conditions or performance;

industry trends and other matters that do not relate strictly to historical facts or statements of assumptions underlying any of the foregoing; and

our estimates regarding our capital requirements and our need for additional financing.

The words anticipates, believes, estimates, expects, intends, may, plans, projects, will, would and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements that we make. We have identified some of the important factors that could cause future events to materially differ from our current expectations and they are described in this prospectus supplement under the caption Risk Factors as well as in our most recent Annual Report on Form 10-K. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or

investments we may make. We do not assume any obligation to update any forward-looking statement.

S-2

**Table of Contents**

**PROSPECTUS SUPPLEMENT SUMMARY**

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

**BioMarin Pharmaceutical Inc.**

**Overview**

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

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

We are conducting clinical trials on several investigational product candidates for the treatment of various diseases including: GALNS, an enzyme replacement therapy for the treatment of Mucopolysaccharidosis Type IV or Morquio Syndrome Type A, or MPS IV A, PEG-PAL, an enzyme substitution therapy for the treatment of phenylketonuria or PKU, BMN-701, an enzyme replacement therapy for Pompe disease, a glycogen storage disorder, BMN-673, an orally available poly (ADP-ribose) polymerase, or PARP inhibitor for the treatment of patients with certain cancers and BMN-111, a peptide therapeutic for the treatment of achondroplasia. We are conducting preclinical development of several other enzyme product candidates for genetic and other metabolic diseases, including BMN-190 for the treatment of late infantile neuronal ceroid lipofuscinosis, or LINCL, a form of Batten disease.

**Table of Contents**

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

Program	Indication	Orphan Drug Designation	Stage	Three Months Ended March 31, 2012	
				Total Net Product Revenues (in millions)	Research & Development Expense (in millions)
Naglazyme	MPS VI (1)	Yes	Approved	\$ 68.6	\$ 2.6
Aldurazyme (2)	MPS I (3)	Yes	Approved	\$ 12.0	\$ 0.5
Kuvan	PKU (4)	Yes	Approved	\$ 32.0	\$ 3.3
Firdapse (5)	LEMS (6)	Yes	Approved in the EU only	\$ 3.6	\$ 2.1
GALNS for MPS IV A	MPS IVA	Yes	Clinical Phase 3	N/A	\$ 24.7
PEG-PAL	PKU	Yes	Clinical Phase 2	N/A	\$ 8.8
BMN-701 for Pompe disease	POMPE (7)	Yes	Clinical Phase 1/2	N/A	\$ 6.9
BMN-673, PARP inhibitor for the treatment of patients with cancer	Not yet determined	Not yet determined	Clinical Phase 1/2	N/A	\$ 2.4
BMN-673, PARP inhibitor for the treatment of patients with hematological malignancies	Not yet determined	Not yet determined	Clinical Phase 1/2		
BMN-111, peptide therapeutic for the treatment of Achondroplasia	Achondroplasia	Yes	Clinical Phase 1	N/A	\$ 3.7

- (1) Mucopolysaccharidosis VI, or MPS VI
- (2) The Aldurazyme total product revenue noted above is the total product revenue recognized by us in accordance with the terms of our agreement with Genzyme Corporation. See *Commercial Products Aldurazyme* below for further discussion.
- (3) Mucopolysaccharidosis I, or MPS I
- (4) Phenylketonuria, or PKU
- (5) Marketing approval from the EMEA for Firdapse was granted in December 2009. We launched Firdapse in the EU in April 2010.
- (6) Lambert Eaton Myasthenic Syndrome, or LEMS
- (7) Pompe disease, a glycogen storage disorder

**Commercial Products*****Naglazyme***

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



## **Table of Contents**

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 and Turkey using our own sales force and commercial organization. Additionally, we use local distributors in several other regions to help us pursue registration and/or market Naglazyme on a named patient basis. Naglazyme net product sales for the three months ended March 31, 2012 were \$68.6 million, as compared to \$60.6 million for the three months ended March 31, 2011. Naglazyme net product sales for 2011 totaled \$224.9 million, as compared to \$192.7 million for 2010 and \$168.7 million for 2009.

### ***Kuvan***

Kuvan is a proprietary synthetic oral form of 6R-BH4, a naturally occurring enzyme co-factor for phenylalanine hydroxylase, or PAH, indicated for patients with PKU. Kuvan is the first drug for the treatment of PKU, which is an inherited metabolic disease that affects at least 50,000 diagnosed patients under the age of 40 in the developed world. We believe that approximately 30-50% of those with PKU could benefit from treatment with Kuvan. PKU is caused by a deficiency of activity of an enzyme, PAH, which is required for the metabolism of phenylalanine, or Phe. Phe is an essential amino acid found in all protein-containing foods. Without sufficient quantity or activity of PAH, Phe accumulates to abnormally high levels in the blood, resulting in a variety of serious neurological complications, including severe mental retardation and brain damage, mental illness, seizures and other cognitive problems.

Kuvan was granted marketing approval for the treatment of PKU in the U.S. in December 2007. We market Kuvan in the U.S. and Canada using our own sales force and commercial organization. Kuvan has been granted orphan drug status in the U.S., which confers seven years of market exclusivity in the U.S for the treatment of PKU, expiring in 2014. We expect that our patents will provide market exclusivity beyond the expiration of orphan status. Kuvan net product revenues were \$32.0 million for the three months ended March 31, 2012, as compared to \$26.7 million for the three months ended March 31, 2011. Kuvan net product sales for 2011 were \$116.8 million, as compared to \$99.4 million for 2010 and \$76.8 million for 2009.

In May 2005, we entered into an agreement with Merck Serono S.A. (Merck Serono) for the further development and commercialization of Kuvan and any other product containing 6R-BH4, and PEG-PAL for PKU. Through the agreement, as amended in 2007, Merck Serono acquired exclusive rights to market these products in all territories outside the U.S., Canada and Japan, and we retained exclusive rights to market these products in the U.S. and to market Kuvan in Canada. Merck Serono markets Kuvan in the EU and several other countries outside the U.S., Canada and Japan. Under the agreement with Merck Serono, we are entitled to receive royalties, on a country-by-country basis, until the later of the expiration of patent right licensed to Merck 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 4% on net sales of Kuvan by Merck Serono. We also sell Kuvan to Merck Serono at 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 months ended March 31, 2012, we earned \$0.5 million in net royalties on net sales of \$11.9 million of Kuvan by Merck Serono, compared to the three months ended March 31, 2011, when we earned \$0.3 million on their net sales of \$8.3 million. In 2011, we earned \$1.6 million in net royalties on net sales of \$40.4 million of Kuvan by Merck Serono, compared to 2010 when we earned \$0.9 million in net royalties on net sales of \$23.7 million. In 2009, we earned \$0.3 million in net royalties on net sales of \$6.9 million. We recorded collaborative agreement revenue associated with shared Kuvan development costs in the amounts of \$0.1 million for the three months ended March 31, 2012, \$0.5 million for 2011, \$0.7 million for 2010 and \$2.4 million for 2009.

---

**Table of Contents**

***Aldurazyme***

Aldurazyme has been approved for marketing in the U.S., EU and other countries for patients with Mucopolysaccharidosis I, or MPS I. MPS I is a progressive and debilitating life-threatening genetic disease, for which no other drug treatment currently exists, that is caused by the deficiency of alpha-L-iduronidase, a lysosomal enzyme normally required for the breakdown of GAGs. Patients with MPS I typically become progressively worse and experience multiple severe and debilitating symptoms resulting from the build-up of carbohydrate residues in all tissues in the body. These symptoms include: inhibited growth, delayed and 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 a collaboration with Genzyme Corporation. 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 license 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 totaled \$12.0 million for the three months ended March 31, 2012 as compared to \$18.7 million for the three months ended March 31, 2011. Aldurazyme net product revenues totaled \$82.8 million for 2011 as compared to \$71.2 million for 2010 and \$70.2 million for 2009. The net product revenues for the three months ended March 31, 2012, and for each of the years ended December 31, 2011, 2010 and 2009 include \$18.4 million, \$74.2 million, \$68.0 million and \$61.8 million, respectively, of royalty revenue on net Aldurazyme sales by Genzyme. Royalty revenue from Genzyme is based on 39.5% to 44.0% of net Aldurazyme sales by Genzyme, which totaled \$45.9 million for the three months ended March 31, 2012, \$185.2 million for 2011, \$166.8 million for 2010 and \$155.1 million for 2009. For the three months ended March 31, 2012, previously recognized Aldurazyme net product transfer revenue of \$6.4 million reflects previous shipments of Aldurazyme to Genzyme. Incremental Aldurazyme net product transfer revenue of \$8.6 million, \$3.2 million and \$8.4 million for 2011, 2010 and 2009, respectively, reflect incremental shipments of Aldurazyme to Genzyme to meet future product demand. In the future, to the extent that Genzyme Aldurazyme inventory quantities on hand remain consistent, we expect that our total Aldurazyme revenues will approximate the 39.5% to 50% royalties on net product sales by Genzyme.

***Firdapse***

In conjunction with our acquisition of Huxley Pharmaceuticals, Inc. (Huxley) we acquired the rights to Firdapse in October 2009, a proprietary form of 3,4-diaminopyridine (amifampridine phosphate), or 3,4-DAP for the treatment of Lambert Eaton Myasthenic Syndrome, or LEMS. Firdapse was originally developed by AGEPS, the pharmaceutical unit of the Paris Public Hospital Authority, or AP-HP, and sublicensed to Huxley from EUSA Pharma in April 2009. 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.

## **Table of Contents**

We launched Firdapse on a country by country basis in Europe beginning in April 2010. Firdapse net product revenues for the three months ended March 31, 2012 were \$3.6 million as compared to \$3.1 million for the three months ended March 31, 2011. Firdapse net product revenues in 2011 were \$13.1 million, compared to \$6.4 million in 2010. We also continue to develop Firdapse for the possible treatment of LEMS in the U.S. and initiated a Phase 3 clinical trial in the second quarter of 2011. This Phase 3 study is a double-blind, placebo-controlled randomized discontinuation study followed by an open-label extension period in approximately 30 patients across 11 sites worldwide. The primary objective of the study is to evaluate the efficacy and safety, including the long-term safety, of Firdapse. The primary efficacy variable is the Quantitative Myasthenia Gravis score and the secondary efficacy variable is the timed 25-foot walk test. We are also exploring other options with the Firdapse program, including the potential outlicense of certain rights in the U.S. or elsewhere.

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**

### ***GALNS***

We are developing GALNS, an enzyme replacement therapy for the treatment of MPS IV A, a lysosomal storage disorder. In November 2008, we announced the initiation of a clinical assessment program for patients with MPS IV A. We initiated a Phase 1/2 clinical trial of GALNS in the first half of 2009. The objectives of the Phase 1/2 study were to evaluate safety, pharmacokinetics, and pharmacodynamics and to identify the optimal dose of GALNS for future studies. The results reported in April 2010 showed clinically meaningful improvements in two measures of endurance (6-minute walk distance and 3-minute stair climb) were achieved at both 24 weeks and 36 weeks as compared to baseline. Clinically meaningful improvements in two measures of pulmonary function (forced vital capacity and maximum voluntary ventilation) were achieved at 36 weeks as compared to baseline and keratan sulfate levels decreased shortly after the initiation of treatment and fell further as the study progressed. In February 2011, we announced the initiation of a pivotal Phase 3 clinical trial for GALNS for the treatment of MPS IV A. This Phase 3 trial is a randomized, double-blind, placebo-controlled study designed to evaluate the efficacy and safety of GALNS in patients with MPS IV A. The trial is being conducted at approximately 30 centers worldwide including Brazil, Japan, Taiwan, most Western European countries, Canada and the U.S. In March 2012, we announced that enrollment for this trial had been completed, with 176 patients enrolled. This trial will explore doses of two milligrams per kilogram per week and two milligrams per kilogram every other week for a treatment period of 24 weeks. We expect to report top-line results from the study in the fourth quarter of 2012.

## **Table of Contents**

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

### ***PEG-PAL***

PEG-PAL is an investigational enzyme substitution therapy that we are developing as a subcutaneous injection and is intended for those patients with PKU who do not respond to Kuvan. In preclinical models, PEG-PAL produced a rapid, dose-dependent reduction in blood phenylalanine, or Phe levels, the same endpoint that was used in the Kuvan studies. In June 2009, we announced results from a Phase 1 open-label, single-dose, dose-escalation clinical trial of PEG-PAL for PKU. Significant reductions in blood Phe levels were observed in all patients in the fifth dosing cohort of the Phase 1 trial. In addition, there are no serious immune reactions observed and mild to moderate injection-site reactions were in line with our expectations. In September 2009, we initiated a Phase 2, open-label dose finding clinical trial of PEG-PAL. The primary objective of this clinical trial is to optimize the dose and schedule that produces the most favorable safety profile and Phe reduction. The secondary objectives of the clinical trial are to evaluate the safety and tolerability of multiple dose levels of PEG-PAL, to evaluate the immune response to PEG-PAL, and to evaluate steady-state pharmacokinetics in all patients and accumulation of PEG-PAL in a subset of patients enrolled in this clinical trial. In the ongoing Phase 2 study, the rate of discontinuation due to adverse events remains low and virtually all patients who are able to achieve a therapeutic dose have their blood Phe levels lowered to less than 600 micromoles per liter, the target of therapeutic efficacy. Mild to moderate self limiting injection site reactions are the most commonly reported toxicity. In April 2011 we initiated an extension of the Phase 2 study to find the quickest and safest induction dosing regimen to an efficacious maintenance dose. This study is ongoing. We expect to report results from the Phase 2 trial in the third quarter of 2012 and, if successful, to initiate a Phase 3 clinical trial of PEG-PAL in 2013 after meetings with regulatory authorities.

### ***BMN-673***

BMN-673 is a PARP inhibitor, a class of molecules that has shown clinical activity against cancers involving defects in DNA repair that we are investigating for the treatment of certain cancers. In January 2011, we announced the initiation of a Phase 1/2 clinical trial for BMN-673 for the treatment of patients with solid tumors. The clinical trial is an open-label study of once-daily, orally-administered BMN-673 in approximately 70 patients ages 18 and older with advanced or recurrent solid tumors. The primary objective of the study is to establish the maximum tolerated dose of daily oral BMN-673. The secondary objective of the study is to establish the safety, pharmacokinetic profile and recommended Phase 2 dose. Over twenty patients have been dosed in the solid tumor study, and we have not yet determined the maximum tolerated dose. Top-line results are expected in the second half of 2012. In July 2011, we initiated a Phase 1/2 clinical trial for BMN-673 for the treatment of patients with advanced hematological malignancies. This clinical trial is a two-arm, open-label dose escalation study to determine the maximum tolerated dose and to assess the safety, pharmacokinetics, pharmacodynamics and preliminary efficacy of once-daily, orally-administered BMN-673 in patients with acute myeloid leukemia, myelodysplastic syndrome, chronic lymphocytic leukemia or mantle cell lymphoma. This study is expected to enroll approximately 80 patients. Top-line results are expected in the first quarter of 2013.

### ***BMN-701***

BMN-701 is a novel fusion of insulin-like growth factor 2 and alpha glucosidase (IGF2-GAA) in development for Pompe disease. We acquired the BMN-701 program in August 2010 in connection with the acquisition of ZyStor Therapeutics, Inc. (ZyStor). In January 2011, we announced the initiation of a Phase 1/2 clinical

## **Table of Contents**

trial for BMN-701. This clinical trial is an open-label study to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamic and clinical activity of BMN-701 administered as an intravenous infusion every two weeks at doses of 5 milligrams per kilogram, 10 milligrams per kilogram and 20 milligrams per kilogram. We expect to enroll approximately 30 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. We are now dosing patients in the 20 milligram per kilogram cohort. BMN-701 has been generally well-tolerated with a safety profile consistent with other enzyme replacement therapies. Pompe disease is a lysosomal storage disorder caused by a deficiency in GAA, which prevents cells from adequately degrading glycogen. This results in the storage of glycogen in lysosomes, particularly those in muscle cells, thereby damaging those cells and causing progressive muscle weakness which in turn can result in death due to pulmonary or cardiac insufficiency. We expect to report top-line results from this study in the fourth quarter of 2012.

### ***BMN-111***

BMN-111 is a peptide therapeutic in development for the treatment of achondroplasia. In January 2012, we announced the initiation of a Phase 1 clinical trial for BMN-111. The primary objective of the Phase 1 clinical trial is 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. We expect to report results from this trial in the third quarter of 2012. We expect to start the Phase 2 study in pediatric patients in the fourth quarter of 2012 or the first quarter of 2013.

### **Company Information**

We were incorporated in Delaware in October 1996 and began operations on March 21, 1997. Our principal executive offices are located at 105 Digital Drive, Novato, California 94949 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](http://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, DC 20549 or by calling the SEC at 1-800-SEC-0330. Additionally, these reports are available at the SEC's website at <http://www.sec.gov>. Information contained in our website is not part of this or any other report that we file with or furnish to the SEC.

**Table of Contents**

**THE OFFERING**

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

Issuer	BioMarin Pharmaceutical Inc.
Common stock to be offered	6,500,000 shares
Common stock to be outstanding after the offering	122,181,825 shares
Option to purchase additional shares	The underwriters have an option to purchase up to 650,000 additional shares of our common stock. The underwriters may exercise this option at any time within 30 days from the date of this prospectus supplement.
Use of Proceeds	We intend to apply the net proceeds of this offering for general corporate purposes, including working capital and research and development. 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 flexibility 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 the offering is based on 115,681,825 shares of common stock outstanding as of March 31, 2012.	
The number of shares of common stock to be outstanding after the offering does not take into account:	

15,537,214 shares of our common stock issuable upon exercise of outstanding options issued under our stock option plans at a weighted average exercise price of \$22.90 per share as of March 31, 2012;

1,415,086 shares of our common stock issuable upon the conversion of our \$23.5 million 2.50% convertible subordinated notes due 2013;

Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 424B5

15,956,385 shares of our common stock issuable upon the conversion of our \$324.9 million 1.875% convertible subordinated notes due 2017; and

S-10

**Table of Contents**

an aggregate of 21,059,525 shares of our common stock available for future equity awards under our stock option plans as of March 31, 2012.

S-11



## Table of Contents

### **RISK FACTORS**

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

#### **Risk Related to Our Business**

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

We must obtain and maintain regulatory approval to market and sell our drug products in the U.S. and in jurisdictions outside of the U.S. In the U.S., we must obtain FDA approval for each drug that we intend to commercialize. The FDA approval process is typically lengthy and expensive, and approval is never certain. Products distributed abroad are also subject to government regulation by international regulatory authorities. Naglazyme, Aldurazyme and Kuvan have received regulatory approval to be commercially marketed and sold in the U.S., EU and other countries. Firdapse has received regulatory approval to be commercially marketed only in the EU. 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.

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

After any of our products receive regulatory approval, they remain subject to ongoing regulation, which can impact, among other things product labeling, manufacturing practices, adverse event reporting, storage, distribution, advertising and promotion, and record keeping. If we do not comply with the applicable regulations, the range of possible sanctions includes issuance of adverse publicity, product recalls or seizures, fines, total or partial suspensions of production and/or distribution, suspension of marketing applications, and enforcement actions, including injunctions and civil or criminal prosecution. The FDA and comparable international regulatory agencies can withdraw a product's approval under some circumstances, such as the failure to comply with regulatory requirements or unexpected safety issues. Further, the FDA often requires post-marketing testing and surveillance to monitor the effects of approved products. The FDA and comparable international regulatory agencies may condition approval of our product candidates on the completion of such post-marketing clinical studies. These post-marketing studies may suggest that a product causes undesirable side effects or may present a risk to the patient. If data we collect from post-marketing studies suggest that one of our approved products may present a risk to safety, the government authorities could withdraw our product approval, suspend production or place other marketing restrictions on our products. If regulatory sanctions are applied or if regulatory approval is

## **Table of Contents**

delayed or withdrawn, the value of our company and our operating results will be adversely affected. Additionally, we will be unable to generate revenue from the sale of these products, our potential for generating positive cash flow will be diminished and the capital necessary to fund our operations will be increased.

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

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

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

Even though we have obtained orphan drug designation for certain of our products and product candidates and even if we obtain orphan drug designation for our future product candidates, due to the uncertainties associated with developing pharmaceutical products, we may not be the first to obtain marketing approval for any particular orphan indication. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process.

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

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

## **Table of Contents**

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

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

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

slow or insufficient patient enrollment;

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

longer treatment time required to demonstrate efficacy;

lack of sufficient supplies of the product candidate;

adverse medical events or side effects in treated patients;

lack of effectiveness of the product candidate being tested; and

regulatory requests for additional clinical trials.

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

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

Since we began operations in March 1997, we have been engaged in very substantial research and development and operated at a net loss until 2008. Although we were profitable in 2008 and 2010, we operated at a net loss in 2009 and 2011. 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 2012 and may operate at an annual net loss beyond 2012. Our future profitability depends on our marketing and selling of Naglazyme, Kuvan and Firdapse, the successful 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



---

**Table of Contents**

with others, our spending on our development programs and the impact of any possible future business development transactions. The extent of our future losses and the timing of profitability are highly uncertain. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

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

Before we can begin commercial manufacture of our products, we, or our contract manufacturers, must obtain regulatory approval of our manufacturing facilities, processes and quality systems. In addition, our pharmaceutical manufacturing facilities are continuously subject to inspection by the FDA, the State of California and international regulatory authorities, before and after product approval. Our manufacturing facilities have been inspected and licensed by the State of California for pharmaceutical manufacture and have been approved by the FDA, the EC, and health agencies in other countries for the manufacture of Aldurazyme, and by the FDA and EC for the manufacture of Naglazyme. In addition, our third-party manufacturers' facilities involved with the manufacture of Naglazyme, Kuvan, Firdapse and Aldurazyme have also been inspected and approved by various regulatory authorities.

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

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

**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.**

We may require additional financing to fund our future operations, including the commercialization of our approved drugs and drug product candidates currently under development, preclinical studies and clinical trials, and potential licenses and acquisitions. We may be unable to raise additional financing, if needed, due to a variety of factors, including our financial condition, the status of our product programs, and the general condition of the financial markets. If we fail to raise additional financing if we need such funds, we may have to delay or terminate some or all of our product development programs and our financial condition and operating results will be adversely affected.

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

our ability to successfully market and sell Naglazyme, Kuvan and Firdapse;

Genzyme's ability to continue to successfully commercialize Aldurazyme;

the progress and success of our preclinical studies and clinical trials (including studies and the manufacture of materials);

the timing, number, size and scope of our preclinical studies and clinical trials;

**Table of Contents**

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 stock purchase agreements with the former stockholders of Huxley, LEAD Therapeutics, Inc. (LEAD) and ZyStor 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 believe that our cash, cash equivalents and short-term investment securities at March 31, 2012 will be sufficient to meet our operating and capital requirements for the foreseeable future based on our current long-term business plans. These estimates are based on assumptions and estimates, which may prove to be wrong. 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 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.

Improvements in manufacturing processes typically are very difficult to achieve and are often very expensive and may require extended periods of time to develop. If we contract for manufacturing services with an unproven process, our contractor is subject to the same uncertainties, high standards and regulatory controls, and may therefore experience difficulty if further process development is necessary.

Even a developed manufacturing process can encounter difficulties. Problems may arise during manufacturing for a variety of reasons, including human error, mechanical breakdowns, problems with raw



## Table of Contents

materials, 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 and Aldurazyme, 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 and Aldurazyme 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 and Aldurazyme or our third-party manufacturer's ability to manufacture Kuvan or Firdapse.**

Our Galli Drive facility located in Novato, California is our only manufacturing facility for Naglazyme and Aldurazyme. It is located in the San Francisco Bay Area near known earthquake fault zones and is vulnerable to significant damage from earthquakes. We, and the third-party manufacturers with whom we contract and our single-source suppliers of raw materials, 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 and Aldurazyme, or to have Kuvan or Firdapse manufactured, could be seriously, or potentially completely impaired, and our Naglazyme, Kuvan, Aldurazyme and Firdapse commercialization efforts and revenue from the sale of Naglazyme, Kuvan, Aldurazyme and Firdapse 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.



## **Table of Contents**

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

Numerous factors could cause interruptions in the supply of our finished products, including:

timing, scheduling and prioritization of production by our contract manufacturers or a breach of our agreements by our contract manufacturers;

labor interruptions;

changes in our sources for manufacturing;

the timing and delivery of shipments;

our failure to locate and obtain replacement manufacturers as needed on a timely basis; and

conditions affecting the cost and availability of raw materials.

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

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

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

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

Third-party payers, such as government or private health care insurers, carefully review and increasingly challenge the prices charged for drugs. Reimbursement rates from private companies vary depending on the



## **Table of Contents**

third-party payer, the insurance plan and other factors. Reimbursement systems in international markets vary significantly by country and by region, and reimbursement approvals must be obtained on a country-by-country basis.

Reimbursement in the EU must be negotiated on a country-by-country basis and in many countries the product cannot be commercially launched until reimbursement is approved. The negotiation process in some countries can exceed 12 months.

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

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

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

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

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

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

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

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

We expect that coverage and reimbursement may be increasingly restricted both in the U.S. and internationally. The escalating cost of health care has led to increased pressure on the health care industry to

## Table of Contents

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 reduce the value of our intellectual property portfolio. As part of these cost containment measures, some countries have imposed or threatened to impose revenue caps limiting the annual volume of sales of Naglazyme. To the extent that these caps are significantly below actual demand, our future revenues and gross margins may be adversely affected.

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

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

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

Several provisions of the new law, which have varying effective dates, may affect us and will likely increase certain of our costs. For example, the Medicaid rebate rate was increased and the volume of rebated drugs has been expanded to include beneficiaries in Medicaid managed care organizations. Among other things, the PPACA also expands the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance; includes a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or donut hole, and imposes a new fee on certain manufacturers and importers of branded prescription drugs (excluding orphan drugs under certain conditions). The law also revised the definition of average manufacturer price for reporting purposes, which could increase the amount of the Medicaid drug rebates paid to states. Substantial new provisions affecting compliance also have been added, which may require us to modify our business practices with health care practitioners. For example, beginning in 2013, drug manufacturers will be required to report information on payments or transfers of value to physicians and teaching hospitals, as well as investment interests held by physicians and their immediate family members during the preceding calendar year. Failure to submit required information may result in civil monetary penalties. Although the statute requires reporting by March 31, 2013 of payments and other transfers of value made in calendar year 2012, the CMS has proposed not to require manufacturers to begin collecting required information until 90 days after publication of a final rule. This means that the initial report due on March 31, 2013 may only need to cover a portion of calendar year 2012.

The reforms imposed by the new law will significantly impact the pharmaceutical industry; however, the full effects of the PPACA cannot be known until these provisions are implemented and the CMS and other federal and state agencies issue applicable regulations or guidance. Moreover, in the coming years, additional changes could be made to governmental healthcare programs that could significantly impact the success of our products or product candidates. We will continue to evaluate the PPACA, as amended, the implementation of regulations or guidance related to various provisions of the PPACA by federal agencies, as well as trends and changes that may be encouraged by the legislation and that may potentially have an impact on our business over

## **Table of Contents**

time. The cost of implementing more detailed record keeping systems and otherwise complying with these regulations could substantially increase our costs. The changes to the way our products are reimbursed by the CMS could reduce our revenues. Both of these situations could adversely affect our results of operations.

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

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

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

We are subject to various federal and state health care fraud and abuse laws, including antikickback laws, false claims laws and laws related to ensuring compliance. The federal health care program antikickback 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 antikickback statute. However, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor. 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 antikickback statute, some of which apply to referral of patients for health care services reimbursed by any source, not just governmental payers. In addition, the State of California and several other states have passed laws that require pharmaceutical companies to comply with both the April 2003 Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers and the PhRMA Code on Interactions with Healthcare Professionals.

Neither the government nor the courts have provided definitive guidance on the application of some of these laws to our business. Law enforcement authorities are increasingly focused on enforcing these laws, and it is possible that some of our practices may be challenged under these laws. While we believe we have structured our business arrangements to comply with these laws, it is possible that the government could allege violations

## **Table of Contents**

of, or convict us of violating, these laws. If we are found in violation of one of these laws, we are required to pay a penalty or are suspended or excluded from participation in federal or state health care programs, our business, financial condition and results of operation may be adversely affected.

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

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

changes in international regulatory and compliance requirements that could restrict BioMarin's ability to manufacture, market and sell its products;

political and economic instability;

diminished protection of intellectual property in some countries outside of the U.S.;

trade protection measures and import or export licensing requirements;

difficulty in staffing and managing international operations;

differing labor regulations and business practices;

potentially negative consequences from changes in or interpretations of tax laws;

changes in international medical reimbursement policies and programs;

financial risks such as longer payment cycles, difficulty collecting accounts receivable and exposure to fluctuations in foreign currency exchange rates; and

regulatory and compliance risks that relate to maintaining accurate information and control over sales and distributors' and service providers' activities that may fall within the purview of the Foreign Corrupt Practices Act.

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

As we continue to expand our existing international operations, we may encounter new risks. For example, as we focus on building our international sales and distribution networks in new geographic regions, we must continue to develop relationships with qualified local distributors and trading companies. If we are not successful in developing and maintaining these relationships, we may not be able to grow sales in these geographic regions. These or other similar risks could adversely affect our revenue and profitability.

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

## Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 424B5

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.

S-22

## Table of Contents

The patent positions of biopharmaceutical products are complex and uncertain. The scope and extent of patent protection for some of our products and product candidates are particularly uncertain because key information on some of our product candidates has existed in the public domain for many years. The composition and genetic sequences of animal and/or human versions of Naglazyme, Aldurazyme, and many of our product candidates have been published and are believed to be in the public domain. The chemical structure of BH4 and 3,4-DAP have also been published. Publication of this information may prevent us from obtaining or enforcing patents relating to our products and product candidates, including without limitation composition-of-matter patents, which are generally believed to offer the strongest patent protection.

We own or have licensed patents and patent applications related to Naglazyme, Kuvan, Aldurazyme and Firdapse and certain of our product candidates. 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. For example, we may not have developed a method for treating a disease before others developed identical or similar methods, in which case we may not receive a granted patent.

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. 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 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 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.

In addition, competition may also seek intellectual property protection for their technology. Due to the amount of intellectual property in our field of technology, we cannot be certain that we do not infringe intellectual property rights of competitors or that we will not infringe intellectual property rights of competitors granted or created in the future. For example, if a patent holder believes our product infringes their patent, the patent holder may sue us even if we have received patent protection for our technology. If someone else claims we infringe their intellectual property, we would face a number of issues, including the following:

Defending a lawsuit, which takes significant time and resources 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, 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



## Table of Contents

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.

Redesigning our product so it does not infringe the intellectual property rights of competitors may not be possible or could require substantial funds and time.

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

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

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

**The USPTO has issued three patents to a third-party that relate to alpha-L-iduronidase and a related patent has been issued in Canada. If we are not able to successfully challenge these patents or a related patent in Japan, if it issues, we may be prevented from producing Aldurazyme in countries with issued patents unless and until we obtain a license.**

The USPTO has issued three patents to Women's and Children's Hospital Adelaide that cover composition-of-matter, isolated genomic nucleotide sequences, vectors including the sequences, host cells containing the vectors, and method of use claims for human, recombinant alpha-L-iduronidase. Aldurazyme is based on human, recombinant alpha-L-iduronidase. Corresponding patent applications were filed in Europe, Japan and Canada. The European patent application was rejected over prior art, was withdrawn and cannot be re-filed. The corresponding Japanese application was finally rejected and cannot be re-filed. A corresponding Canadian patent issued and covers enzyme, pharmaceutical composition, nucleic acid encoding the enzyme, host cells and vectors. We believe that these patents are invalid or not infringed on a number of grounds. However, under U.S. law, issued patents are entitled to a presumption of validity, and a challenge to the U.S. patents may be unsuccessful. Even if we are successful, challenging the patents may be expensive, require our management to devote significant time to this effort and may adversely impact marketing of Aldurazyme in the U.S. and Canada.

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

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

## **Table of Contents**

If the MMS Agreement is terminated for breach, the breaching party will transfer its interest in BioMarin/Genzyme LLC, or the LLC, to the non-breaching party, and the non-breaching party will pay a specified buyout 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.

**Our strategic alliance with Merck Serono may be terminated at any time by Merck Serono, and if it is terminated, our expenses could increase and our operating performance could be adversely affected.**

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. 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 Canada. Merck Serono may terminate the agreement forming our strategic alliance with them at any time by giving 90 days prior written notice if such termination occurs prior to the commercialization of any of the products licensed under our agreement, or by giving 180 days prior written notice if such termination occurs after the commercialization of such a product. Either Merck Serono or we may terminate our strategic alliance under certain circumstances, including if the other party is in material breach of the agreement and does not remedy the breach within a specified period of time, or has suffered certain financial difficulties, including filing for bankruptcy or making an assignment for the benefit of creditors. Although we are not currently in breach of the agreement and we believe that Merck Serono is not currently in breach of the agreement, there is a risk that either party could breach the agreement in the future. Upon a termination of the agreement by Merck Serono by giving notice or by us for a material breach by Merck Serono, all rights licensed to us under the agreement become irrevocable and fully-paid except in those countries where restricted by applicable law or for all intellectual property that Merck Serono does not own.

Upon a termination of the agreement by Merck Serono for a material breach by us or based on our financial difficulty, or upon the expiration of the royalty term of the products licensed under the agreement, all

## **Table of Contents**

rights licensed to Merck Serono under the agreement become irrevocable and fully-paid upon the payment of amounts due by Merck Serono to us which accrued prior to the expiration of the royalty term, except in those countries where restricted by applicable law or for all intellectual property that we do not own and for which we do not have a royalty-free license. Upon a termination of the agreement for a material breach by us or for our financial difficulty, all rights and licenses granted by Merck Serono to us under or pursuant to the agreement will automatically terminate. If the agreement is terminated by either Merck Serono or us, and we continue the development and commercialization of products related to that agreement, we would be responsible for 100% of future development costs and all costs relating to the assumption of commercial responsibility for the marketing and selling of products related to that agreement, and accordingly our expenses would increase and our operating performance may be adversely affected.

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

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

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

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

The Hatch Waxman Act permits the FDA to approve abbreviated new drug applications, or ANDAs, for generic versions of branded drugs. We refer to this process as the ANDA process. The ANDA process permits competitor companies to obtain marketing approval for a drug with the same active ingredient for the same uses but does not generally require the conduct and submission of clinical equivalency studies for that product. In place of such clinical studies, an ANDA applicant usually needs only to submit data demonstrating that its product is bioequivalent to the branded product based on pharmacokinetic studies. Pursuant to the Hatch Waxman Act, companies were able to file an ANDA application for the active ingredient in Kuvan at any time after December 2011. At present, we have no information that any other party has filed or is preparing to file an ANDA for Kuvan.

The Hatch Waxman Act requires an applicant for a drug that relies, at least in part, on our data regarding the safety and efficacy of Kuvan, to notify us of their application and potential infringement of our patent rights.

## **Table of Contents**

Upon receipt of this notice we would have 45 days to bring a patent infringement suit in federal district court against the company seeking approval for its product. The discovery, trial and appeals process in such suits can take several years. If such a suit is commenced, the Hatch Waxman Act provides a 30-month stay on the FDA's approval of the competitor's application. If the litigation is resolved in favor of the applicant or the challenged patent expires during the 30-month stay period, the stay is lifted and the FDA's review of the application may be completed. Such litigation is often time-consuming and costly and may result in generic competition if such patent(s) are not upheld or if the generic competitor does not infringe such patent(s). However, generic versions of Kuvan would be prohibited until the expiration of orphan drug exclusivity in 2014 or 2015, depending on if we receive pediatric exclusivity.

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

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

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

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

Our future growth and success will depend in large part of 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 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.

## **Table of Contents**

### **The integration of our Chief Financial Officer into our management team may interfere with our operations.**

We have recently hired a new Chief Financial Officer. To integrate into our company, our CFO must spend a significant amount of time learning our business model and our management system. Our CFO must do this in addition to performing his regular duties. If we fail to complete this integration in an efficient manner, our business and prospects will suffer.

### **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 and financial and administrative systems. Our staff, financial resources, systems, procedures or controls may be inadequate to support our operations and our management may be unable to manage successfully future market opportunities or our relationships with customers and other third parties.

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

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

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

We are exposed to the potential product liability risks inherent in the testing, manufacturing and marketing of human pharmaceuticals. We maintain insurance against product liability lawsuits for commercial sale of our products and for the clinical trials of our product candidates. Pharmaceutical companies must balance the cost of insurance with the level of coverage based on estimates of potential liability. Historically, the potential liability associated with product liability lawsuits for pharmaceutical products has been unpredictable. Although we believe that our current insurance is a reasonable estimate of our potential liability and represents a commercially reasonable balancing of the level of coverage as compared to the cost of the insurance, we may be subject to claims in connection with our clinical trials and commercial use of Naglazyme, Kuvan, Aldurazyme and Firdapse, or our clinical trials for PEG-PAL, GALNS, BMN-701, BMN-673 or BMN-111 for which our insurance coverage may not be adequate.

The product liability insurance we will need to obtain in connection with the commercial sales of our product candidates if and when they receive regulatory approval may be unavailable in meaningful amounts or at a reasonable cost. In addition, while we continue to take what we believe are appropriate precautions, we may be unable to avoid significant liability if any product liability lawsuit is brought against us. If we are the subject of a successful product liability claim that exceeds the limits of any insurance coverage we obtain, we may incur substantial charges that would adversely affect our earnings and require the commitment of capital resources that might otherwise be available for the development and commercialization of our product programs.

## **Table of Contents**

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

We rely significantly on our information technology and manufacturing infrastructure to effectively manage and maintain our inventory and internal reports, to manufacture and ship products to customers and to timely invoice them. Any failure, inadequacy, or interruption of that infrastructure or security lapse of that technology, including cybersecurity incidents could harm our ability to operate our business effectively. Our ability to manage and maintain our inventory and internal reports, to manufacture and ship our products to customers and timely invoice them depends significantly on our enterprise resource planning, production management, and other information systems. Cybersecurity attacks in particular are evolving and include, but are not limited to, malicious software, attempts to gain unauthorized access to data and other electronic security breaches that could lead to disruptions in systems, misappropriation of our confidential or otherwise protected information and corruption of data. Cybersecurity incidents resulting in the failure of our enterprise resource planning system, production management or other systems to operate effectively or to integrate with other systems, or a breach in security or other unauthorized access of these systems, may affect our ability to manage and maintain our inventory and internal reports, and result in delays in product fulfillment and reduced efficiency of our operations. A breach in security, unauthorized access resulting in misappropriation, theft, or sabotage with respect to our proprietary and confidential information, including research or clinical data could require significant capital investments to remediate any such failure, problem or breach, all of which could adversely affect our business, financial condition and results of operations.

**Our business is affected by macroeconomic conditions.**

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

For the year ended December 31, 2011, approximately 4.5% of our net product revenues were from the Southern European countries of Italy, Spain, Portugal and Greece. Approximately, 15.4% of our total accounts receivable as of December 31, 2011 related to such countries. If the financial conditions of these countries continues to decline, a substantial portion of the receivables may be uncollectable, which would adversely affect our results of operations. Additionally, if one or more of these countries were unable to purchase our products, our revenue would be adversely affected.

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

**Table of Contents**

**Risks Related to this Offering and Ownership of Our Common Stock**

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

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

product sales and profitability of Naglazyme, Aldurazyme, Kuvan and Firdapse;

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

progress of our product candidates through the regulatory process;

results of clinical trials, announcements of technological innovations or new products by us or our competitors;

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

developments or disputes concerning patent or proprietary rights;

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

economic conditions in the U.S. or abroad;

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

actual or anticipated fluctuations in our operating results; and

changes in our assessments or financial estimates by securities analysts.

In the past, following periods of large price declines in the public market price of a company's securities, securities class action litigation has often been initiated against that company. Litigation of this type could result in substantial costs and diversion of management's attention and resources, which would hurt our business. Any adverse determination in litigation could also subject us to significant liabilities. In addition, the current decline in the financial markets and related factors beyond our control, including the credit and mortgage crisis in the U.S. and worldwide, may cause our stock price to decline rapidly and unexpectedly.

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

## Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 424B5

We are incorporated in Delaware. Certain anti-takeover provisions of Delaware law and our charter documents as currently in effect may make a change in control of our company more difficult, even if a change in control would be beneficial to the stockholders. Our anti-takeover provisions include provisions in our certificate of incorporation providing that stockholders' meetings may only be called by our Board of Directors and provisions in our bylaws providing that the stockholders may not take action by written consent and requiring that stockholders that desire to nominate any person for election to our Board of Directors or to make any proposal with respect to business to be conducted at a meeting of our stockholders be submitted in appropriate form to our Secretary within a specified period of time in advance of any such meeting. Additionally,

S-30



**Table of Contents**

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 have not determined the specific allocation of the net proceeds of this offering. Our management will have broad discretion in the application of the net proceeds from this offering and could spend the proceeds in ways that do not necessarily improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business or financial condition, cause the price of our common stock to decline and delay product development.

**Table of Contents**

**USE OF PROCEEDS**

We expect to receive approximately \$       million from the sale of 6,500,000 shares of our common stock in this offering, based on the price set forth on the cover page of this prospectus, or \$       million if the underwriters exercise their option to purchase additional shares in full, after deducting the estimated offering expenses that we are to pay.

We intend to apply the net proceeds of this offering for general corporate purposes, including working capital and research and development. 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 flexibility in applying these proceeds. Until we use the net proceeds of this offering, we intend to invest the funds in short term, interest bearing instruments or other investment grade securities.

S-32

**Table of Contents****PRICE RANGE OF COMMON STOCK**

Our common stock is listed on the Nasdaq Global Select Market under the symbol BMRN. The following table shows the high and low closing sale prices for our common stock as reported by the Nasdaq Global Select Market during the periods indicated:

	<b>High</b>	<b>Low</b>
<b>Year Ended December 31, 2010</b>		
First Quarter	\$ 23.81	\$ 18.95
Second Quarter	\$ 24.71	\$ 18.33
Third Quarter	\$ 23.09	\$ 18.24
Fourth Quarter	\$ 28.25	\$ 21.82
<b>Year Ended December 31, 2011</b>		
First Quarter	\$ 28.29	\$ 23.46
Second Quarter	\$ 28.46	\$ 24.93
Third Quarter	\$ 31.87	\$ 24.02
Fourth Quarter	\$ 35.38	\$ 30.07
<b>Year Ended December 31, 2012</b>		
First Quarter	\$ 38.34	\$ 33.68
Second Quarter (through May 29, 2012)	\$ 39.06	\$ 32.13

The last reported sale price of our common stock on the Nasdaq Global Select Market on May 29, 2012 was \$39.06 per share. As of May 29, 2012, there were 63 holders of record of our common stock. Additionally, as of May 29, 2012, options to acquire 16,668,460 shares of our common stock were outstanding under our stock option plans.

**Table of Contents**

**DIVIDEND POLICY**

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

S-34

**Table of Contents**

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

The following is a summary of certain material U.S. federal income tax considerations relating to the purchase, ownership and disposition of our common stock applicable to non-U.S. holders as we define that term below. This discussion is not a complete analysis of all of the potential U.S. federal income tax consequences relating thereto, nor does it address any tax consequences arising under any state, local or foreign tax laws, or any other U.S. federal tax laws. This summary is based upon the provisions of the Internal Revenue Code of 1986, as amended (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 (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 a partnership or any other entity taxable as a partnership, or any of the following:

an individual 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;

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 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;

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;

foreign persons or entities, except to the extent specifically set forth below;

persons that are partnerships or other pass-through entities;

persons that own, or are deemed to own, more than 5% of our company;

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

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

S-35

---

**Table of Contents**

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

**Distributions on Common Stock**

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

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

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

To claim the benefit of a tax treaty or an exemption from withholding because the dividends are effectively connected with the conduct of a trade or business in the U.S., a non-U.S. holder must either (a) provide a properly executed IRS Form W-8BEN or Form W-8ECI (as applicable) before the payment of dividends or (b) if our common stock is held through certain foreign intermediaries, satisfy the relevant certification requirements of applicable U.S. Treasury regulations. These forms must 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 regular graduated U.S. federal income tax rates in much the same manner as if the non-U.S. holder were a U.S. person and, if the non-U.S. holder is a foreign corporation, the branch profits tax described above may also apply;

---

## **Table of Contents**

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

our common stock constitutes a United States real property interest by reason of our status as a United States real property holding corporation (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 (currently at a rate of 28%) 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**

On February 8, 2012, the Treasury Department issued proposed regulations relating to the Foreign Account Tax Compliance Act, or FATCA, which was enacted in March of 2010. As a general matter, FATCA



**Table of Contents**

imposes a 30% withholding tax on dividends on, and gross proceeds from the sale or other disposition of, our common stock if paid to a foreign entity unless (i) if the foreign entity is a foreign financial institution, the foreign entity undertakes certain due diligence, reporting, withholding, and certification obligations, (ii) if the foreign entity is not a foreign financial institution, the foreign entity identifies certain of its U.S. investors, or (iii) the foreign entity is otherwise excepted under FATCA. Although this legislation currently applies to applicable payments made after December 31, 2012, under the proposed regulations, withholding is required (i) with respect to dividends on our common stock paid on or after January 1, 2014, and (ii) with respect to gross proceeds from a sale or other disposition of our common stock that occurs on or after January 1, 2015. Notwithstanding the foregoing, the proposed regulations will not be effective until issued in final form. There can be no assurance either as to when final regulations relating to FATCA will be issued or as to the particular form that those final regulations might take. If withholding is required under FATCA on a payment related to our common stock, investors that otherwise would not be subject to withholding (or that otherwise would be entitled to a reduced rate of withholding) on such payment generally will be required to seek a refund or credit from the IRS to obtain the benefit of such exemption or reduction (provided that such benefit is available). We will not pay any additional amounts in respect of amounts withheld under FATCA. Prospective investors should consult their tax advisors regarding the effect of FATCA in their particular circumstances.

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

**Table of Contents****UNDERWRITING**

Merrill Lynch, Pierce, Fenner & Smith Incorporated and Barclays Capital Inc. are the underwriters in connection with this offering. Subject to the terms and conditions described 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.

<b>Underwriter</b>	<b>Number of Shares</b>
Merrill Lynch, Pierce, Fenner & Smith Incorporated	
Barclays Capital Inc.	
<b>Total</b>	<b>6,500,000</b>

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 nondefaulting underwriters may be increased or the underwriting agreement may be terminated.

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.

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.

**Commissions and Discounts**

The underwriters are purchasing the shares of common stock from us at \$ \_\_\_\_\_ per share (representing approximately \$ \_\_\_\_\_ aggregate proceeds to us, before we deduct our out-of-pocket expenses of approximately \$394,000, or approximately \$ \_\_\_\_\_ if the underwriters' option to purchase additional shares described below is exercised in full). The underwriters may offer the shares of common stock from time to time for sale in one or more transactions on the Nasdaq Global Select Market, in the over-the-counter market, through negotiated transactions or otherwise at market prices prevailing at the time of sale, at prices related to prevailing market prices or at negotiated prices. In connection with the sale of the shares of common stock offered hereby, the underwriters may be deemed to have received compensation in the form of underwriting discounts. The underwriters may effect such transactions by selling shares of common stock to or through dealers, and such dealers may receive compensation in the form of discounts, concessions or commissions from the underwriters and / or purchasers of shares of common stock for whom they may act as agents or to whom they may sell as principal.

**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 650,000 additional shares of our common stock at the price 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.

## **Table of Contents**

### **Nasdaq Global Select Market Listing**

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

The transfer agent and registrar for our common stock is BNY Mellon Shareowner Services.

### **No Sales of Similar Securities**

We and our executive officers and directors have agreed, with certain limited exceptions, not to sell or transfer any of our common stock or any securities convertible into or exercisable or exchangeable for our common stock for 60 days after the date of this prospectus supplement without first obtaining the prior written consent of the underwriter. 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 a registration statement 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 that transfers, in whole or in part, the economic consequence of ownership of any of our common stock whether any such swap or transaction is to be settled by delivery of shares or other securities, in cash or otherwise.

The restrictions described in the preceding paragraph do not apply to our issuance of:

any shares of our common stock upon the exercise of an option or warrant or the conversion of a security outstanding as of the date of this prospectus supplement;

any shares of our common stock or options to purchase our common stock pursuant to existing employee benefit plans referred to in this prospectus supplement;

any shares of our common stock in connection with any joint venture, partnering or other arrangement with any strategic investor or partner of ours; or

any shares of our common stock in connection with any acquisition made by us.

As to our executive officers and directors, the restrictions do not apply to:

bona fide gifts;

transfers to a trust for the direct or indirect benefit of the individuals subject to the 60 day restriction or the immediate family of any such individual (for purposes of the lock-up agreement, immediate family shall mean any relationship by blood, marriage or adoption, not more remote than first cousin), provided that:

- (1) the underwriter receives a signed lock-up agreement for the balance of the 60 day restriction period from each donee, trustee, distributee, or transferee, as the case may be;
- (2) any such transfer shall not involve a disposition for value;
- (3) such transfers are not required to be reported in any public report or filing with the SEC, or otherwise; and

S-40

---

**Table of Contents**

- (4) the individual subject to the lockup does not otherwise voluntarily effect any public filing or report regarding such transfers;

transfers to us for tax withholding purposes in connection with vesting of equity awards that are subject to a taxable event upon vesting;

transfers to us upon repurchase pursuant to existing employee benefit plans referred to in this prospectus supplement; or

sales pursuant to previously established 10b5-1 trading plans.

The 60 day restriction does not apply to the exercise of stock options held by directors and officers (provided that the shares of common stock received upon exercise shall continue to be restricted by the lockup agreement).

Furthermore, we and our officers and directors subject to the restriction may sell shares of our common stock purchased on the open market following this offering if and only if (i) such sales are not required to be reported in any public report or filing with the SEC, or otherwise, and (ii) a public filing or report regarding such sales is not otherwise voluntarily made.

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.

**Price Stabilization and Short Positions**

In connection with the 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 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 the 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 the offering. Stabilizing transactions consist of various bids for or purchases of shares of common stock made by the underwriters in the open market to peg, fix or maintain the price of our common stock 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.

## **Table of Contents**

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 representatives 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.

### **Other Relationships**

The underwriters and their affiliates have in the past and may in the future provide us with investment banking and advisory services. From time to time, the underwriters and certain of their affiliates may in the future engage in transactions with, and perform investment banking and/or commercial banking services, for us and our affiliates in the ordinary course of business. The underwriters or their affiliates have received and 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 express independent research views in respect of such securities or financial instruments and may hold, or recommend to clients that they acquire, long and/or short positions in such securities and instruments.

### **Electronic Distribution**

In connection with the offering, the underwriters or securities dealers may distribute this prospectus supplement and the accompanying prospectus by electronic means, such as email.

### **Notice to Prospective Investors in the European Economic Area**

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive (each, a Relevant Member State), with effect from and including the date on which the Prospectus Directive is implemented in that Relevant Member State (the Relevant Implementation Date), no offer of shares may be made to the public in that Relevant Member State other than:

- A. to any legal entity which is a qualified investor as defined in the Prospectus Directive;
- B. to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150, natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the underwriters; or
- C. in any other circumstances falling within Article 3(2) of the Prospectus Directive,

## **Table of Contents**

provided that no such offer of shares shall require the Company or the underwriter to publish a prospectus pursuant to Article 3 of the Prospectus Directive or supplement a prospectus pursuant to Article 16 of the Prospectus Directive.

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

The Company, the underwriter and its affiliates will rely upon the truth and accuracy of the foregoing representation, acknowledgement and agreement.

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

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

### **Notice to Prospective Investors in the United Kingdom**

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

**Table of Contents**

**Notice to Prospective Investors in Switzerland**

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

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

**Notice to Prospective Investors in the Dubai International Financial Centre**

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



**Table of Contents**

**LEGAL MATTERS**

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

**EXPERTS**

The consolidated financial statements of BioMarin Pharmaceutical Inc. and subsidiaries as of December 31, 2011 and 2010, and for each of the years in the three-year period ended December 31, 2011, and management's assessment of the effectiveness of internal control over financial reporting as of December 31, 2011 have been incorporated by reference herein and in the registration statement in reliance upon the reports of KPMG LLP, independent registered public accounting firm, incorporated by reference herein, and upon the authority of said firm as an experts in accounting and auditing.

The financial statements of BioMarin/Genzyme LLC at December 31, 2010 and for the years ended December 31, 2010 and 2009 incorporated in this prospectus supplement by reference to the Annual Report on Form 10-K of BioMarin Pharmaceutical Inc. for the year ended December 31, 2011 have been so incorporated in reliance on the report of PricewaterhouseCoopers LLP, independent accountants, given on the authority of said firm as experts in auditing and accounting.

S-45

**Table of Contents**

**PROSPECTUS**

## **Common Stock**

We may offer and sell shares of our common stock from time to time in one or more offerings.

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

Our principal executive offices are located at 105 Digital Drive, Novato, California 94949, and our telephone number is (415) 506-6700.

Our common stock is listed on the Nasdaq Global Select Market under the symbol **BMRN** . On May 29, 2012, the last reported sale price of our common stock was \$39.06 per share.

**Investing in our common stock involves various risks. See the sections entitled RISK FACTORS on page 3 and CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS on page 4. Additional risks associated with an investment in us will be described in the related prospectus supplements.**

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

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

**The date of this prospectus is May 30, 2012.**

**Table of Contents**

**TABLE OF CONTENTS**

	<b>Page</b>
<u>About this Prospectus</u>	1
<u>BioMarin Pharmaceutical Inc.</u>	2
<u>Risk Factors</u>	3
<u>Cautionary Note Regarding Forward-Looking Statements</u>	4
<u>Where You Can Find More Information</u>	5
<u>Information Incorporated by Reference</u>	6
<u>Use of Proceeds</u>	7
<u>Description of Capital Stock</u>	8
<u>Plan of Distribution</u>	9
<u>Legal Matters</u>	11
<u>Experts</u>	11

**Table of Contents**

**ABOUT THIS PROSPECTUS**

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

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

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

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

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

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

**Table of Contents**

**BIOMARIN PHARMACEUTICAL INC.**

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

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

We are conducting clinical trials on several investigational product candidates for the treatment of various diseases including: GALNS, an enzyme replacement therapy for the treatment of Mucopolysaccharidosis Type IV or Morquio Syndrome Type A, or MPS IV A, PEG-PAL, an enzyme substitution therapy for the treatment of phenylketonuria or PKU, BMN-701, an enzyme replacement therapy for Pompe disease, a glycogen storage disorder, BMN-673, an orally available poly (ADP-ribose) polymerase, or PARP inhibitor for the treatment of patients with certain cancers and BMN-111, a peptide therapeutic for the treatment of achondroplasia. We are conducting preclinical development of several other enzyme product candidates for genetic and other metabolic diseases, including BMN-190 for the treatment of late infantile neuronal ceroid lipofuscinosis, or LINCL, a form of Batten disease.

We were incorporated in Delaware in October 1996 and began operations on March 21, 1997. Our principal executive offices are located at 105 Digital Drive, Novato, California 94949 and our telephone number is (415) 506-6700.

**Table of Contents**

**RISK FACTORS**

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

**Table of Contents**

**CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS**

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

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

our expectations with respect to regulatory submissions and approvals and our clinical trials;

any projection or expectation of earnings, revenue or other financial items;

the plans, strategies and objectives of management for future operations;

factors that may affect our operating results;

new products or services;

the demand for our products;

our ability to consummate acquisitions and successfully integrate them into our operations;

future capital expenditures;

effects of current or future economic conditions or performance;

industry trends and other matters that do not relate strictly to historical facts or statements of assumptions underlying any of the foregoing;

our success in pending litigation;

our estimates regarding our capital requirements and our need for additional financing.

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

## Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 424B5

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



**Table of Contents**

**WHERE YOU CAN FIND MORE INFORMATION**

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

**Table of Contents**

**INFORMATION INCORPORATED BY REFERENCE**

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

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

Our Annual Report on Form 10-K for the year ended December 31, 2011, as filed with the SEC on February 22, 2012;

Our Quarterly Report on Form 10-Q for the quarter ended March 31, 2012, as filed with the SEC on April 30, 2012;

Our Definitive Proxy Statement on Schedule 14A filed with the SEC on March 23, 2012;

Our Current Reports on Form 8-K, as filed with the SEC on January 9, 2012, February 22, 2012, May 9, 2012 and May 24, 2012;

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

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

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

BioMarin Pharmaceutical Inc.

Attention: Corporate Secretary

105 Digital Drive

Novato, CA 94949

(415) 506-6700

Any statement contained in a document incorporated or deemed to be incorporated by reference in this prospectus shall be deemed modified, superseded or replaced for purposes of this prospectus to the extent that a statement contained in this prospectus, or in any subsequently filed document that also is deemed to be



**Table of Contents**

incorporated by reference in this prospectus, modifies, supercedes or replaces such statement. Any statement so modified, superceded or replaced shall not be deemed, except as so modified, superceded or replaced, to constitute a part of this prospectus.

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

**USE OF PROCEEDS**

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

**Table of Contents**

**DESCRIPTION OF CAPITAL STOCK**

Our authorized common stock consists of 250,000,000 shares, \$0.001 par value per share, and 1,000,000 shares of preferred stock, \$0.001 par value per share. At May 28, 2012, there were 116,514,244 shares of our common stock issued and outstanding. The approximate number of stockholders of record of our common stock as of May 28, 2012 was 62.

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

We may issue up to 999,886 shares of preferred stock in one or more classes or series within a class as may be determined by our Board of Directors, who may establish, from time to time, the number of shares to be included in each class or series, may fix the designation, powers, preferences and rights of the shares of each such class or series and any qualifications, limitations or restrictions thereof, and may increase or decrease the number of shares of any such class or series without any further vote or action by the stockholders. Any preferred stock so issued by the Board of Directors may rank senior to the common stock with respect to the payment of dividends or amounts upon liquidation, dissolution or winding up of the company, or both. In addition, any such shares of preferred stock may have class or series voting rights. Moreover, under certain circumstances, the issuance of preferred stock or the existence of the unissued preferred stock might tend to discourage or render more difficult a merger or other change in control of us.

**Table of Contents**

**PLAN OF DISTRIBUTION**

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

The applicable prospectus supplement will describe the terms of the offering of our common stock, including:

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

the purchase price of the shares of our common stock and the proceeds we will receive from the sale;

any underwriting discounts and other items constituting underwriters' compensation;

any offering price at which our shares of common stock may be offered to the public;

any over-allotment options under which underwriters may purchase additional shares of common stock from us;

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

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

We may distribute the shares of our common stock from time to time in one or more transactions at:

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

market prices prevailing at the time of sale;

prices related to such prevailing market prices; or

negotiated prices.

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

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

## Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 424B5

Subject to specific limited conditions, the underwriters will be obligated to purchase all of the shares of common stock offered by the applicable prospectus supplement. Any public offering price and any discounts or concessions allowed or reallocated or paid to dealers may change from time to time.

## **Table of Contents**

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

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

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

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

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

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

We cannot guarantee the liquidity of the trading markets for our shares of common stock.

Until the distribution of shares of our common stock is completed, SEC rules may limit the underwriters from bidding for and purchasing our common stock. However, the underwriters may engage in transactions that stabilize the price of the common stock, such as bids or purchases to peg, fix or maintain that price. These transactions may include short sales, stabilizing transactions and purchases to cover positions created by short sales. Short sales involve the sale by the underwriters of more shares than are listed on the cover of this prospectus supplement. Covered short sales are sales made in an amount not greater than the underwriters' option to purchase additional shares from us in the related offering. The underwriters may reduce the short



## **Table of Contents**

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

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

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

## **LEGAL MATTERS**

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

## **EXPERTS**

The consolidated financial statements of BioMarin Pharmaceutical Inc. and subsidiaries as of December 31, 2011 and 2010, and for each of the years in the three-year period ended December 31, 2011, and management's assessment of the effectiveness of internal control over financial reporting as of December 31, 2011 have been incorporated by reference herein and in the registration statement in reliance upon the reports of KPMG LLP, independent registered public accounting firm, incorporated by reference herein, and upon the authority of said firm as an expert in accounting and auditing.

The financial statements of BioMarin/Genzyme LLC at December 31, 2010 and for the years ended December 31, 2010 and 2009 incorporated in this prospectus by reference to the Annual Report on Form 10-K of BioMarin Pharmaceutical Inc. for the year ended December 31, 2011 have been so incorporated in reliance on the report of PricewaterhouseCoopers LLP, independent accountants, given on the authority of said firm as experts in auditing and accounting.

Table of Contents

**6,500,000 Shares**

**Common Stock**

**PROSPECTUS SUPPLEMENT**

**BofA Merrill Lynch**

**Barclays**

**, 2012**