BIOMARIN PHARMACEUTICAL INC
Form 10-K
February 29, 2016
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

Form 10-K

(Mark One)

x ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the fiscal year ended December 31, 2015

Or

¨TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the transition period from to .

Commission file number: 000-26727

BioMarin Pharmaceutical Inc.

(Exact name of registrant as specified in its charter)

Delaware	68-0397820
(State of other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
770 Lindaro Street San Rafael, California	94901
(Address of principal executive offices)	(Zip Code)

Registrant’s telephone number, including area code: (415) 506-6700

Securities registered pursuant to Section 12(b) of the Act:

Title of Each Class	Name of Each Exchange on Which Registered
Common Stock, $.001 par value	The NASDAQ Global Select Market

Securities registered under Section 12(g) of the Act:

None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes x No ¨

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ¨ No x

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes x No ¨

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes x No ¨

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§ 229.405 of this chapter) is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ¨

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer" "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	x	Accelerated filer	¨
Non-accelerated filer	¨ (Do not check if a smaller reporting company)	Smaller reporting company	¨

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act.) Yes ¨ No x

The aggregate market value of the voting and non-voting common stock held by non-affiliates of the registrant as of June 30, 2015 was $15.2 billion, based on the closing price reported for such date on the NASDAQ Global Select Market.

As of February 12, 2016, the registrant had 161,590,680 shares of common stock, par value $0.001, outstanding.

The documents incorporated by reference are as follows: Portions of the Registrant's Proxy Statement for our annual meeting of stockholders to be held June 6, 2016, are incorporated by reference into Part III.

BIOMARIN PHARMACEUTICAL INC.

2015 FORM 10-K ANNUAL REPORT

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Kyndrisa™ is our trademark. BioMarin®, Vimizim® Naglazyme®, Kuvan® and Firdapse® are our registered trademarks. Aldurazyme ® is a registered trademark of BioMarin/Genzyme LLC. All other brand names and service marks, trademarks and other trade names appearing in this report are the property of their respective owners.

Part I

FORWARD LOOKING STATEMENTS

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

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

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

Item 1. Business

Overview

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

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

We are conducting clinical trials on several investigational product candidates for the treatment of various diseases including: Kyndrisa (drisapersen), an exon-51 skipping compound for the potential treatment of Duchenne muscular dystrophy (DMD) amenable to exon 51 skipping; pegvaliase (formerly referred to as PEG PAL), an enzyme substitution therapy for the treatment of phenylketonuria (PKU); reveglucosidase alfa (formerly referred to as BMN 701), an enzyme replacement therapy for Pompe disease, a glycogen storage disorder; vosoritide (formerly referred to as BMN 111), a peptide therapeutic for the treatment of achondroplasia, the leading cause of dwarfism; BMN 044, BMN 045 and BMN 053 for the treatment of DMD (exons 44, 45 and 53); cerliponase alfa (formerly referred to as

BMN 190) for the treatment of late infantile neuronal ceroid lipofuscinosis (CLN2), a lysomal storage disorder primarily affecting the brain; and BMN 270, an AAV VIII vector and Factor VIII gene therapy drug development candidate, for the treatment of hemophilia A. We are conducting or planning to conduct preclinical development of several other product candidates for genetic and other metabolic diseases, including a novel fusion of alpha-N-acetyglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2) (formerly referred to as BMN 250), for the treatment of Sanfilippo B syndrome, or mucopolysaccharidosis type IIIB (MPS IIIB). We expect to initiate a Phase 1 study for NAGLU in the first half of 2016.

Recent Developments

Regulatory Review of Kyndrisa

In January 2016 the U.S. Food and Drug Administration (the FDA) issued a complete response letter to our New Drug Application (NDA) for Kyndrisa for the treatment of DMD amenable to exon 51 skipping. The FDA issues a complete response letter to indicate that the review cycle for an application is complete and that the application is not ready for approval in its present form. The FDA concluded that the standard of substantial evidence of effectiveness for Kyndrisa had not been met. We are reviewing the complete response letter and will work with the FDA to determine the appropriate next steps regarding this application. A Marketing Authorization Application (MAA) for Kyndrisa for the treatment of DMD amenable to exon 51 skipping remains under review in the EU. We anticipate that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) will provide an opinion for our MAA for Kyndrisa in the second quarter of 2016. If the CHMP opinion is positive, the MAA will be referred to the European Commission (EC). The EC is expected to render a final decision for Kyndrisa in the second half of 2016. If the MAA is approved by the EC, we would receive marketing authorization for Kyndrisa in the EU. Furthermore, if we receive approval in the EU, we plan to roll out our international registration strategy for Kyndrisa using the EU approval as the basis of multiple international applications.

Acquisition of Rights to PKU Franchise from Ares Trading S.A. (Merck Serono)

On October 1, 2015 we entered into a Termination and Transition Agreement with Ares Trading S.A. (Merck Serono), as amended and restated on December 23, 2015 (the A&R Kuvan Agreement), to terminate the Development, License and Commercialization Agreement, dated May 13, 2005, as amended (the License Agreement), between us and Merck Serono, including the license to Kuvan granted in the License Agreement from us to Merck Serono. Also on October 1, 2015, we and Merck Serono entered into a Termination Agreement (the Pegvaliase Agreement) to terminate the license to pegvaliase granted in the License Agreement from us to Merck Serono. On January 1, 2016, pursuant to the A&R Kuvan Agreement and the Pegvaliase Agreement, we completed the acquisition from Merck Serono and its affiliates of certain rights and other assets, and the assumption from Merck Serono and its affiliates of certain liabilities, in each case with respect to Kuvan and pegvaliase. As a result, we acquired all global rights to Kuvan and pegvaliase from Merck Serono, with the exception of Kuvan in Japan. Previously, we had exclusive rights to Kuvan in the U.S. and Canada and pegvaliase in the U.S. and Japan.

Pursuant to the A&R Kuvan Agreement, in December 2015 we paid a deposit on this transaction totaling $371.8 million and we may pay Merck Serono up to a maximum of €60.0 million, in cash, if future sales milestones are met. Pursuant to the Pegvaliase Agreement, we may also pay Merck Serono up to a maximum of €125.0 million, in cash, if future development milestones are met.

We and Merck Serono have no further rights or obligations under the License Agreement with respect to pegvaliase. The License Agreement will continue in effect in order to complete the transfer of certain assets related to Kuvan, the majority of which occurred in January 2016. Accordingly, we continue to rely on Merck Serono to provide critical transition services for sales and distribution of Kuvan until marketing authorizations can be transferred in approximately 13 remaining countries, but in no event later than December 31, 2016.

Sale of Talazoparib to Medivation, Inc.

On October 6, 2015 we completed the sale of talazoparib (formerly referred to as BMN 673), an orally available poly-ADP ribose polymerase (PARP) inhibitor for the treatment of patients with certain cancers, to Medivation, Inc. (Medivation), under which Medivation acquired the worldwide rights to talazoparib in exchange for an upfront payment of $410.0 million and up to an additional $160.0 million upon the achievement of regulatory and sales-based milestones, as well as mid-single digit percentage royalties for talazoparib.

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Paragraph IV Notice Letter from Dr. Reddy's Laboratories and Par Pharmaceutical, Inc.

We received a paragraph IV notice letter, dated October 3, 2014, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL), notifying us that DRL had filed an abbreviated new drug application (ANDA) seeking approval of a proposed generic version of Kuvan 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). Additionally, we received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying us that Par has filed an ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book. Together with Merck & Cie, on March 6, 2015 we filed lawsuits against both DRL and Par in the U.S. District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan tablets and seeking an injunction to prevent Par from introducing a generic version of Kuvan tablets that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of each ANDA in accordance with the Hatch-Waxman Act, which expires in July 2017. In response, DRL and Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

In September 2015, we entered into a settlement agreement with DRL that resolved the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral tablets. Under the terms of the settlement agreement, we have granted DRL a non-exclusive license to our Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100 mg tablets in the U.S. for the indications approved for Kuvan beginning at a confidential date in the future, but which is more than five years from the settlement date, or earlier under certain circumstances.

The settlement with DRL does not affect the case against Par, and the litigation against Par is still pending. The parties submitted opening claim construction briefs on January 14, 2016, and responsive claim construction briefs are due on March 4, 2016. The Court has not yet set a date for trial in the litigation against Par.

We also received a paragraph IV notice letter, dated January 14, 2016, from Par, notifying us that Par has filed a separate ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral powder prior to the expiration of our patents listed in the FDA's Orange Book. On February 22, 2016, we filed a lawsuit against Par in the U.S. District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan powder and seeking an injunction to prevent Par from introducing a generic version of Kuvan powder that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2018.

Acquisition of Prosensa Holding N.V.

On January 29, 2015, we completed the acquisition of Prosensa Holding N.V. (Prosensa), a public limited liability company organized under the laws of the Netherlands, for a total purchase price of $751.5 million. In connection with the acquisition of Prosensa, we recognized transaction costs of $9.7 million, of which $7.0 million and $2.7 million, respectively, was recognized in the years ended December 31, 2015 and 2014.

Prosensa was an innovative biotechnology company engaged in the discovery and development of ribonucleic acid (RNA)-modulating therapeutics for the treatment of genetic disorders. Prosensa's primary focus was on rare neuromuscular and neurodegenerative disorders with a large unmet medical need, including subsets of patients with DMD, myotonic dystrophy and Huntington's disease. Prosensa's clinical portfolio of RNA-based product candidates was focused on the treatment of DMD. Each of Prosensa's DMD compounds has been granted orphan drug status in the U.S. and the EU, including Prosensa's lead product, Kyndrisa.

In connection with our acquisition of Prosensa, we made cash payments totaling $680.1 million, which consisted of $620.7 million for approximately 96.8% of Prosensa's ordinary shares (the Prosensa Shares), $38.6 million for the options that vested pursuant to our tender offer for the Prosensa Shares and $20.8 million to the remaining Prosensa shareholders that did not tender their shares under the tender offer. Additionally, for each Prosensa Share, we issued one non-transferable contingent value right (CVR), which represents the contractual right to receive a cash payment of up to $4.14 per Prosensa Share, or an aggregate of approximately $160.0 million (undiscounted), upon the achievement of certain product approval milestones. The fair value of the CVRs and acquired in-process research and development (IPR&D) on the acquisition date was $71.4 million and $772.8 million, respectively. The acquisition date fair value of the CVRs and IPR&D was estimated by applying a probability-based income approach utilizing an appropriate discount rate. Key assumptions include a discount rate and various probability factors. See Note 13 to the accompanying Condensed Consolidated Financial Statements for additional discussion regarding fair value measurements of the CVRs, which is included in contingent acquisition consideration payable.

Summary of Commercial Products and Major Development Programs

A summary of our various commercial products and major development programs, including key metrics as of December 31, 2015, is provided below:

Commercial Products	Indication	U.S. Orphan Drug Exclusivity Expiration	EU Orphan Drug Exclusivity Expiration	2015 Total Net Product Revenues (in millions)	2015 Research & Development Expense (in millions)
Vimizim	MPS IV A [1]	2021	2024	$ 228.1	$ 45.7
Naglazyme	MPS VI [2]	Expired	Expired	$ 303.1	$ 12.8
Kuvan	PKU [3]	Expired	2020 [4]	$ 239.3	$ 15.4
Aldurazyme [5]	MPS I [6]	Expired	Expired	$	