

BioMarin Receives Anticipated Notification of PDUFA Extension for Pegvaliase Biologics License Application (BLA) to May 28, 2018

Regulatory Review Process Proceeding In-line with Company's Expectations

SAN RAFAEL, Calif., Dec. 22, 2017 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (Nasdaq: BMRN) today announced that the U.S. Food and Drug Administration (FDA) will require additional time to complete its review of the Biologics License Application (BLA) for its investigational therapy pegvaliase, a PEGylated recombinant phenylalanine ammonia lyase enzyme product, to reduce blood phenylalanine (Phe) levels in adult patients with phenylketonuria (PKU) who have uncontrolled blood Phe levels on existing management. In a notice received from the FDA, the Prescription Drug User Fee Act (PDUFA) Goal Date for pegvaliase has been extended by three months to May 28, 2018. Due to the Memorial Day weekend, the Action Goal Date will be May 25, 2018.

On Aug. 29, 2017, when the FDA accepted BioMarin's BLA and granted priority review status, the company announced that the FDA had requested additional information on Chemistry, Manufacturing and Controls (CMC), which was likely to be classified as a major amendment to the BLA and result in a three month extension of the PDUFA date. As expected, the FDA designated the receipt of this additional information as a major amendment to the application thus extending the PDUFA action date by three months.



"We are pleased with our ongoing interactions with the FDA on the pegvaliase BLA. We appreciate the FDA's ability to expedite review through priority designation, particularly for this complex disease and treatment," said Hank Fuchs, M.D., President Worldwide Research and Development at BioMarin. "We continue to work closely with the FDA and look forward to the possibility of bringing this important treatment to patients."

The FDA has granted priority review designation to pegvaliase, which is granted to drugs that treat a serious condition and, if approved, would provide a significant improvement in safety or effectiveness of the treatment, prevention, or diagnosis of a serious condition.

About Pegvaliase

Pegvaliase is an investigational study drug that substitutes the deficient PAH enzyme in PKU with the PEGylated version of the enzyme phenylalanine ammonia lyase, to break down Phe. It is being developed as a potential treatment for adults with inadequately controlled blood Phe levels in the study. In clinical studies, treatment with subcutaneous pegvaliase substantially reduced blood Phe compared to placebo using a randomized withdrawal study design, and led to long-term maintenance of Phe reduction in the majority of adult patients with PKU. Pegvaliase was administered using a dosing regimen that achieved a manageable safety profile, consisting primarily of immune-mediated responses, including anaphylaxis, for which robust risk management measures effective in clinical trials will be proposed.

For additional information regarding the investigational product pegvaliase, please contact BioMarin Medical Information at medinfo@bmrn.com.

About Phenylketonuria

Phenylketonuria (PKU), or phenylalanine hydroxylase (PAH) deficiency, is a genetic disorder affecting approximately 50,000 diagnosed patients in the developed world and is caused by a deficiency of the enzyme PAH. This enzyme is required for the metabolism of Phe, an essential amino acid found in most protein-containing foods. If the active enzyme is not present in sufficient quantities, Phe accumulates to abnormally high levels in the blood and becomes toxic to the brain, resulting in a variety of complications including severe intellectual disability, seizures, tremors, behavioral problems and psychiatric symptoms. As a result of newborn screening efforts implemented in the 1960s and early 1970s, virtually all individuals with PKU under the age of 40 in developed countries are diagnosed at birth and treatment is implemented soon after. PKU can be managed with a Phe-restricted diet, which is supplemented by low-protein modified foods and Phe-free medical foods; however, the strict diet is difficult for most patients to adhere to the extent needed for achieving adequate control of blood Phe levels.

To learn more about PKU, please visit www.PKU.com. Information on this website is not incorporated by reference into this press release.

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare disorders. The company's portfolio consists of six commercialized products and multiple clinical and pre-clinical product candidates.

For additional information, please visit www.BMRN.com. Information on BioMarin's website is not incorporated by reference into this press release.

Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: BioMarin's development programs for pegvaliase generally, and specifically about expectations regarding the BLA filing for pegvaliase with the FDA and the FDA's evaluation of such filing; the potential outcome of the review of such filings; and the possible approval of such product candidate. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. These risks and uncertainties include, among others: results of current and planned clinical trials of pegvaliase; the content and timing of decisions by the U.S. Food and Drug Administration, the European Medicines Agency and other regulatory authorities; our ability to manufacture sufficient quantities of pegvaliase for clinical trials, commercial launch and other preapproval requirements; and those factors detailed in BioMarin's filings with the Securities and Exchange Commission, including, without limitation, the factors contained under the caption "Risk Factors" and elsewhere in the Company's Securities and Exchange Commission (SEC) filings, including the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2017, and future filings and reports by the Company. The Company undertakes no duty or obligation to update any forward-looking statements contained in this press release as a result of new information, future events or changes in its expectations.

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