FDA Not Currently Planning to Hold Advisory Committee Meeting for BioMarin's Pegvaliase Biologics License Application (BLA)

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

SAN RAFAEL, Calif., Sept. 14, 2017 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (Nasdaq: BMRN) today announced that the U.S. Food and Drug Administration (FDA) has provided the company with the "Day-74" filing communication for its Biologics License Application (BLA) for 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 the letter, the FDA advised the Company that it is not currently planning to hold an advisory committee meeting to discuss the application. Additionally, the FDA indicated that the review is proceeding according to the Agency's internal review timelines described in their Guidance on Good Review Management Principles and Practices for PDUFA Products. The FDA comments based on its preliminary evaluation are generally in-line with the Company's expectations.

"We are pleased with our ongoing interactions with the FDA on the pegvaliase BLA. While the Agency has indicated they do not currently plan to hold an advisory committee meeting, the FDA may still consult with outside experts and patients in a similar fashion as they did when reviewing the BLA for our most recently approved treatment for a form of Batten disease," said Hank Fuchs, M.D., President Worldwide Research and Development at BioMarin. "We continue to work closely with the FDA as they review this application, and we are confident that we will be able to respond to the Agency's comments in a timely manner."

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. The PDUFA action date is February 28, 2018. However, the FDA has requested additional Chemistry, Manufacturing, and Controls (CMC) information, which we expect, when submitted, will be classified as a major amendment and result in a three month extension of the PDUFA date.

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 product candidate pegvaliase, and specifically about expectations regarding the BLA filing for pegvaliase with the FDA and the FDA's evaluation of such filing; expectations regarding an advisory committee meeting to discuss the application; 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 and timing of current and planned clinical trials of its product candidates; the content and timing of decisions by the FDA, the EMA and other regulatory authorities concerning pegvaliase; and those other risks detailed from time to time 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 June 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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