FDA Grants Breakthrough Therapy Designation for BioMarin's Valoctocogene Roxaparvovec (formerly BMN 270), an Investigational Gene Therapy for Hemophilia A

Global Phase 3 Program to Begin Before Year End

SAN RAFAEL, Calif., Oct. 26, 2017 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today that the U.S. Food and Drug Administration (FDA) granted valoctocogene roxaparvovec (formerly BMN 270) Breakthrough Therapy Designation. The FDA's Breakthrough Therapy Designation program is intended to facilitate and expedite development and review of new drugs to address unmet medical need in the treatment of a serious condition. To qualify for Breakthrough Therapy Designation, preliminary clinical evidence must show that that the drug may demonstrate substantial improvement over existing therapies. BioMarin expects to initiate enrollment of a global Phase 3 program before the end of the year.

"The news of the FDA granting Breakthrough Therapy Designation coupled with EU PRIME designation granted in early 2017 by EMA, demonstrates the strong support of global health authorities for valoctocogene roxaparvovec and its expedited development and registration pathway," said Hank Fuchs, M.D., President, Worldwide Research and Development at BioMarin. "There is a tremendous need to achieve normal steady state Factor VIII levels to eliminate spontaneous bleeding, to avoid the complications of suboptimally corrected bleeding disorder, to improve quality of life and enable patients to live to their fullest potential."

Breakthrough Therapy Designation status was granted based on the data from an ongoing BioMarin Phase 1/2 study, which evaluated safety and efficacy of valoctocogene roxaparvovec. Interim results of the study were provided at the company's R&D Day for investors last week.

The global Phase 3 program includes two studies with valoctocogene roxaparvovec, one with the 4e13 vg/kg dose and one with the 6e13 vg/kg dose. The studies will each likely include approximately 40 patients. In addition, the Company has commissioned its commercial gene therapy manufacturing facility to supply both clinical and commercial drug.

Valoctocogene Roxaparvovec Regulatory Status

Earlier this month, the Company announced that the FDA completed its review of the IND application for valoctocogene roxaparvovec and concluded that it can proceed. The IND application included 52-week data at the 6e13 vg/kg dose and the protocol for the Phase 3 study using the 6e13 vg/kg dose. The protocol for the second Phase 3 study using the 4e13 vg/kg dose has also been submitted to the FDA. Both studies may now begin in the US, following approval from institutional review boards.

Phase 3 Clinical Trial Applications have also been approved by the UK Medicines and Healthcare Products Regulatory Agency (MHRA) for both study protocols, one for the 6e13 vg/kg and one for the 4e13 vg/kg.

The European Medicines Agency (EMA) has granted access to its Priority Medicines (PRIME) regulatory initiative for valoctocogene roxaparvovec. To be accepted into PRIME, an investigational therapy has to show its potential to benefit patients with unmet medical needs based on early clinical data. PRIME focuses on medicines that may offer a major therapeutic advantage over existing treatments, or benefit patients with no treatment options. These medicines are considered priority medicines within the European Union (EU).

BioMarin's valoctocogene roxaparvovec has also received orphan drug designation from the FDA and EMA for the treatment of hemophilia A. The Orphan Drug Designation program is intended to advance the evaluation and development of products that demonstrate promise for the diagnosis and/or treatment of rare diseases or conditions.

Gene Therapy Manufacturing

BioMarin has constructed one of the largest gene therapy manufacturing facilities in the world, which is located in Novato, California. Good Manufacturing Practices (GMP) production of valoctocogene roxaparvovec has commenced and will support clinical development activities and anticipated commercial demand. This facility is capable of supporting approximately 2,000 patients per year, and the production process was developed in accordance with International Conference on Harmonisation guidance for Pharmaceuticals for Human Use

facilitating worldwide registration with health authorities.

About Hemophilia A

Hemophilia A, also called Factor VIII (FVIII) deficiency or classic hemophilia, is a genetic disorder caused by missing or defective Factor VIII, a clotting protein. Although it is passed down from parents to children, about 1/3 of cases are caused by a spontaneous mutation, a new mutation that was not inherited. As an X-linked disorder, hemophilia A mostly affects males, occurring in approximately 1 in 5,000 male births. People living with the disease are not able to form blood clots efficiently and are at risk for excessive bleeding from modest injuries, potentially endangering their life. People with severe hemophilia often bleed spontaneously into their muscles or joints. The standard of care for the 43% of hemophilia A patients who are severely affected, is a prophylactic regimen of Factor VIII infusions three times per week. Even with prophylactic regimens, many patients still experience microbleeds and spontaneous bleeding events that result in progressive joint damage

About Gene Therapy at BioMarin

Gene therapy is a treatment designed to alter a genetic problem by adding a corrected copy of the defective gene. The functional gene is inserted into a vector – containing a DNA sequence coding for a specific protein – that acts as a delivery mechanism, providing the ability to deliver the functional gene to cells. The cells can then use the information to build the functional protein that the body needs, potentially reducing or eliminating the cause of the disease. Currently, gene therapy for the treatment of hemophilia A is available only as part of a clinical trial. The AAV approach to gene therapy has been advanced at the University College London (UCL) in the treatment of hemophilia B. At UCL, the data regarding this technology supports the ability to correct bleeding and to be generally well tolerated for greater than four years in a continuing clinical trial.

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 the development of BioMarin's valoctocogene roxaparvovec program generally, the timing and expected design of future clinical trials and announcements from those studies and trials, including BioMarin's Phase 3 program with valoctocogene roxaparvovec, the adequacy of production of valoctocogene roxaparvovec in the Company's commercial gene therapy manufacturing facility and the expected regulatory actions related to valoctocogene roxaparvovec. 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: BioMarin's success in the commercialization of its commercial products, results and timing of current and planned preclinical studies and clinical trials of valoctocogene roxaparvovec, including final analysis of the interim data; the continued clinical experiences of the patients in the current studies; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities; the content and timing of decisions by local and central ethics committees regarding the clinical trials; our ability to successfully manufacture the product candidate for the preclinical and clinical trials; and those other risks detailed from time to time under the caption "Risk Factors" and elsewhere in BioMarin's Securities and Exchange Commission (SEC) filings, including BioMarin's Quarterly Report on Form 10-Q for the quarter ended June 30, 2017, and future filings and reports by BioMarin. BioMarin 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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