European Medicines Agency Validates BioMarin's Marketing Authorization Application for Valoctocogene Roxaparvovec to Treat Severe Hemophilia A

Potential 1st Gene Therapy in Europe Directed at Any Type of Hemophilia
Application to be Reviewed Under Accelerated Assessment

SAN RAFAEL, Calif., Dec. 23, 2019 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today that the European Medicines Agency (EMA) validated the Company's Marketing Authorization Application (MAA) for its investigational gene therapy, valoctocogene roxaparvovec, for adults with severe hemophilia A. The MAA review will commence in January 2020 under accelerated assessment.

Recognizing valoctocogene roxaparvovec for its potential to benefit patients with unmet medical needs, EMA granted access to its Priority Medicines (PRIME) regulatory initiative in 2017 and recently granted BioMarin's request for accelerated assessment of this MAA, potentially shortening the review period. This submission is based on an interim analysis of study participants treated in an ongoing Phase 3 study with material from the to-be-commercialized process and updated three-year Phase 1/2 data. This submission marks the first marketing application submission in Europe for a gene therapy product for any type of hemophilia.

Accelerated assessment reduces the time-frame for the EMA Committee for Medicinal Products for Human Use (CHMP) and Committee for Advanced Therapies (CAT) to review a MAA for an Advanced Therapy Medicinal Product (ATMP). Applications are eligible for accelerated assessment if the CHMP and CAT decide the product is of major interest for public health, particularly from the point of view of therapeutic innovation. Evaluating a MAA under the EMA centralized procedure can take up to 210 days, not counting clock stops when applicants are requested to provide additional information. On request, the CHMP and CAT can reduce the time-frame to 150 days if the applicant provides sufficient justification for an accelerated assessment, although an application initially designated for accelerated assessment can revert to the standard procedure during the review for a variety of reasons. The decision to grant accelerated assessment has no impact on the eventual CHMP and CAT opinion on whether a marketing authorization should be granted.

The U.S. Food and Drug Administration (FDA) has granted valoctocogene roxaparvovec Breakthrough Therapy designation. Valoctocogene roxaparvovec has Orphan Drug designation from the FDA and the EMA.

"We are pleased that the agency has recognized the potential scientific advancement that valoctocogene roxaparvovec could bring to people with severe hemophilia A," said Hank Fuchs, M.D., President, Global Research and Development at BioMarin. "We continue to move thoughtfully and urgently through the regulatory review process to deliver a treatment that we believe has the potential to make a meaningful difference to people with hemophilia A. We also believe that the field of gene therapy offers the possibility of changing the practice of medicine in many rare genetic diseases like hemophilia A."

About Hemophilia A

People living with hemophilia A lack enough functioning Factor VIII protein to help their blood clot and are at risk for painful and/or potentially life-threatening bleeds from even modest injuries. Additionally, people with the most severe form of hemophilia A often experience painful, spontaneous bleeds into their muscles or joints. Individuals with the most severe form of hemophilia A make up approximately 43 percent of the hemophilia A population. The standard of care for such individuals with hemophilia A is a prophylactic regimen of replacement Factor VIII infusions administered intravenously up to two to three times per week or 100 to 150 infusions per year. Despite these regimens, many people continue to experience bleeds, resulting in progressive and debilitating joint damage, which can have a major impact on their quality of life.

Hemophilia A, also called Factor VIII deficiency or classic hemophilia, is an X-linked 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. Approximately 1 in 10,000 people have Hemophilia A.

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for serious and life-threatening rare and ultra-rare genetic diseases. The Company's portfolio consists of seven commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit www.biomarin.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. (BioMarin), including without limitation, statements about development of BioMarin's valoctocogene roxaparvovec program generally and; the timing of the regulatory activities in the U.S and Europe, including validation and timing of potential approvals and the expected review procedures. 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 preclinical studies and clinical trials of valoctocogene roxaparvovec; additional data from the continuation of the Phase 1/2 trial and the Phase 3 trial, any potential adverse events observed in the continuing monitoring of the participants in the clinical trials; 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 valoctocogene roxaparvovec for the clinical trials and commercially, if approved; 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 September 30, 2019, 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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