BioMarin Submits Biologics License Application to U.S. Food and Drug Administration for Valoctocogene Roxaparvovec to Treat Hemophilia A

1st Marketing Application Submission in U.S. for Gene Therapy Directed at Any Type of Hemophilia

SAN RAFAEL, Calif., Dec. 23, 2019 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today that the company submitted a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for its investigational AAV gene therapy, valoctocogene roxaparvovec, for adults with hemophilia A. Subject to completion of the FDA's filing review, BioMarin anticipates the BLA review to commence in February 2020. BioMarin will provide an update in February 2020.

This submission is based on a Phase 3 interim analysis of study participants treated with material from the to-be-commercialized process, and the three-year Phase 1/2 data. This submission marks the first marketing application submission for a gene therapy product for any type of hemophilia in the United States.

The FDA has granted valoctocogene roxaparvovec Breakthrough Therapy designation. Valoctocogene roxaparvovec has Orphan Drug designation from the FDA and the European Medicines Agency (EMA). Today, the EMA validated the Company's Marketing Authorization Application (MAA) to the EMA. BioMarin anticipates the start of the MAA review to commence in January 2020 under accelerated assessment.

"We look forward to working with the FDA as we seek marketing authorization for the potential first gene therapy for hemophilia A. We are grateful for the high level of interest from the health authorities in this investigational treatment," said Hank Fuchs, M.D., President, Global Research and Development at BioMarin. "We especially appreciate the study participants and investigators, who have made this milestone a reality. Our hope is one day very soon to deliver a transformative treatment that has the potential to change the way hemophilia A is treated."

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 company's regulatory submissions 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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