BioMarin's Biologics License Application for Valoctocogene Roxaparvovec Accepted for Priority Review by FDA with Review Action Date of August 21, 2020

No Advisory Committee Meeting Currently Planned to Review the Application

If approved, 1st Gene Therapy in U.S. for the Treatment of Any Type of Hemophilia

FDA Also Accepts Premarket Approval (PMA) Application for Companion Diagnostic Test, a 1st for a Gene Therapy

SAN RAFAEL, Calif., Feb. 20, 2020 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (Nasdaq: BMRN) today announced that the U.S. Food and Drug Administration (FDA) has accepted for Priority Review the Biologics License Application (BLA) to the FDA for its investigational AAV5 gene therapy, valoctocogene roxaparvovec, for adults with hemophilia A. This acceptance by the FDA marks the first marketing application accepted for a gene therapy product for any type of hemophilia in the United States.

The FDA has granted priority review designation to valoctocogene roxaparvovec, 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 Prescription Drug User Fee Act (PDUFA) action date is August 21, 2020. The FDA has informed the company that they are not currently planning to hold an advisory committee meeting to discuss the application.

The application is based on a Phase 3 interim analysis of study participants treated with investigational product manufactured by the to-be-commercialized process and three-year Phase 1/2 data. BioMarin has constructed, commissioned, and validated one of the first gene therapy manufacturing facilities of its kind in the world. This award-winning facility is located in Novato, California. Marketing authorization documentation has been included in the applications, and the facility is ready for inspection to support approval.

In addition, the FDA has accepted the premarket approval (PMA) application for an AAV5 total antibody assay intended as a companion diagnostic test for valoctocogene roxaparvovec. With a low prevalence of pre-existing immunity to AAV5, BioMarin estimates that approximately 80% of people with hemophilia A in the US do not have preexisting immunity to AAV5 that would make them ineligible for AAV5-mediated gene therapy treatment. The assay is produced by ARUP Laboratories, a leading national

reference laboratory and a nonprofit enterprise of the University of Utah and its Department of Pathology.

"Valoctocogene roxaparvovec has the potential to be the first gene therapy approved in any type of hemophilia and the acceptance of this application and its priority review status marks a significant milestone for gene therapies in general and for the hemophilia community specifically," said Hank Fuchs, M.D., President, Global Research and Development at BioMarin. "We recognize the decades of scientific research that has allowed us to reach this stage of development. As pioneers in gene therapy, we are proud of the medical and technological innovation represented in valoctocogene roxaparvovec, which is possible because of the scientists who did the early research, clinical investigators, the hemophilia community and the people who work here. We look forward to working with the FDA to bring this groundbreaking therapy to people with hemophilia A."

"The hemophilia community has been waiting for decades for gene therapies. The FDA acceptance of the filing and initiation of review for the first gene therapy for hemophilia A builds on years of scientific achievements in improving the standard of care for people with bleeding disorders," said Doris V. Quon M.D., Medical Director, Orthopaedic Hemophilia Treatment Center at The Orthopaedic Institute for Children. "As a treating physician, I look forward to the possibility of having more treatment options for people with hemophilia."

Regulatory Status in the U.S. and Europe

The FDA has granted valoctocogene roxaparvovec Breakthrough Therapy designation. Valoctocogene roxaparvovec has Orphan Drug designation from the FDA and the European Medicines Agency (EMA).

The 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 commenced 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 marks the first marketing application under review in Europe for a gene therapy product for any type of hemophilia.

Companion Diagnostic

The companion diagnostic is an AAV5 total antibody assay, which consists of a simple blood test to help identify patients most likely to respond to AAV5-based gene therapy. BioMarin implemented the test in multiple clinical studies evaluating valoctocogene roxaparvovec treatment of hemophilia A patients without antibodies to AAV5.

BioMarin partnered with ARUP, a laboratory with 35 years of experience supporting hospitals, physicians, and patients with unparalleled quality and service including two prior FDA companion diagnostic approvals, to develop this test. ARUP's experience in developing FDA-approved, single-source companion diagnostic tests is unique among reference laboratories.

"We are delighted to have partnered with BioMarin on its hemophilia A gene therapy program," said ARUP CEO Sherrie Perkins, MD, PhD. "Working with BioMarin on the simultaneous submission of the diagnostic and gene therapy applications aligns with our corporate commitment to providing outstanding patient care through cutting-edge diagnostic testing."

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 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.

About ARUP Laboratories

Founded in 1984, ARUP Laboratories is a leading national reference laboratory and a nonprofit enterprise of the University of Utah and its Department of Pathology. ARUP offers more than 3,000 tests and test combinations, ranging from routine screening tests to esoteric molecular and genetic assays. ARUP serves clients across the United States, including many of the nation's top university teaching hospitals and children's hospitals, as well as multihospital groups, major commercial laboratories, group purchasing organizations, military and other government facilities, and major clinics. In addition, ARUP is a worldwide leader in innovative laboratory research and development, led by the efforts of the ARUP Institute for Clinical and Experimental Pathology®.

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, the expected review procedures, and the timing of the FDA's acceptance of the premarket approval application for an AAV5 total antibody assay intended as a companion diagnostic test produced by ARUP Laboratories. 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 FDA, 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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