BioMarin Announces Acceptance of Late Breaking Abstract at the International Society on Thrombosis and Haemostasis (ISTH) 2019 Congress in Melbourne, Australia from July 6-10, 2019

Abstract Covers Efficacy and Safety of AAV Gene Therapy Over 3 Years with Valoctocogene Roxaparvovec in Severe Hemophilia A

SAN RAFAEL, Calif., June 26, 2019 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today that Professor John Pasi, M.B., Ch.B., Ph.D., from Barts and the London School of Medicine and Dentistry and principal investigator for the valoctocogene roxaparvovec Phase 1/2 study (BMN 270-201) and the Phase 3 study (BMN 270-301) will present data in a late breaking abstract session at the International Society on Thrombosis and Haemostasis (ISTH) 2019 Congress being held July 6-10, 2019 in Melbourne, Australia. More than 5,000 experts from over 90 countries in the field will convene at the Melbourne Convention Exhibition Centre (MCEC) to present the latest basic, translational and clinical research and discuss the diagnostic and therapeutic advances in bleeding and clotting disorders.

"We are pleased to share clinical data in a late breaking abstract on our novel investigational gene therapy treatment for severe hemophilia A at the leading scientific event for thrombosis, hemostasis and vascular biology," said Hank Fuchs, M.D., President Worldwide Research and Development at BioMarin. "Researching a gene therapy treatment for people with severe hemophilia A has the potential to address a significant unmet medical need and advance the standard of care."

Founded in 1969, the ISTH is the leading worldwide not-for-profit organization dedicated to advancing the understanding, prevention, diagnosis and treatment of thrombotic and bleeding disorders. ISTH is an international professional membership organization with more than 5,000 clinicians, researchers and educators working together to improve the lives of patients in more than 100 countries around the world. Among its highly regarded activities and initiatives are education and standardization programs, research activities, yearly congresses, peer-reviewed publications, expert committees and World Thrombosis Day on 13 October.

Oral Presentation – Late Breaking Abstract Session

Title	Presenter
First-in-human Evidence of Durable Therapeutic Efficacy and Safety of AAV Gene Therapy Over Three-years with Valoctocogene Roxaparvovec for Severe Haemophilia A (BMN 270-201 Study) **Presentation:** Monday, July 8 10:45 – 12:00 (AEST or GMT +10) **Location:** Plenary Hall	John Pasi, M.B., Ch.B., Ph.D., Barts and the London School of Medicine and Dentistry

Presentation Number: LB 01.2	

About Hemophilia A

People living with hemophilia A lack enough FVIII protein to help their blood clot and are at risk for painful, potentially life-threatening bleeds from even modest injuries. Additionally, people with severe hemophilia A often experience painful, spontaneous bleeds into their muscles or joints. The standard of care for the 43 percent of individuals with hemophilia A who are severely affected is a prophylactic regimen of Factor VIII infusions administered intravenously two to three times per week. 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 (FVIII) 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 is born with 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 Statements

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc.(BioMarin), including without limitation, statements about the development of BioMarin's valoctocogene roxaparvovec program generally; the Phase 1/2 study with valoctocogene roxaparvovec; and the impact of valoctocogene roxaparvovec gene therapy for treating people with severe hemophilia A;. 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, including final analysis of the above interim data; additional data from the continuation of this Phase 1/2 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; errors and deficiencies in our durability modeling; 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 March 31, 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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