BioMarin Announces Acceptance of Late Breaking Abstract at the International Society on Thrombosis and Haemostasis 2017 Congress

Oral Presentation to Include Interim Results from its Ongoing Phase 1/2 BMN 270 Clinical Program, a Gene Therapy for Hemophilia A
Conference call and web-cast to be held July 11th at 2:30pm CEST/8:30am ET

SAN RAFAEL, Calif., June 16, 2017 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today that the company will present data in a late breaking abstract session at the International Society on Thrombosis and Haemostasis (ISTH) 2017 Congress being held July 8-13, 2017 in Berlin, Germany. The presentation will report interim results from a Phase 1/2 study of BMN 270, an AAV5-FVIII Gene transfer in severe hemophilia.

"We feel privileged to be presenting the only human data in gene therapy for hemophilia A at ISTH," said Hank Fuchs, M.D., President Worldwide Research and Development at BioMarin. "We are committed to further our ongoing clinical program with BMN 270, which has the potential to change the course of hemophilia A treatment, the most common form of the disease, and look forward to sharing our latest findings."

ISTH is a global not-for-profit membership organization advancing the understanding, prevention, diagnosis and treatment of thrombotic and bleeding disorders. The congress hosts the world's leading experts on thrombosis, hemostasis and vascular biology, presenting the most recent advances to improve patient care.

Oral Presentation – Late Breaking Abstract Session

Title	**Authors**
Interim Results from a Phase 1/2 AAV5-FVIII Gene Transfer in Patients with Severe Hemophilia A **Presentation:** July 11 at 9:30-9:38 AM CEST	J. Pasi, S. Rangarajan, L. Walsh, W. Lester, D. Perry, B. Madan, H. Yu, G.F. Pierce, W.Y. Wong

Conference Call to be held Tuesday, July 11th at 2:30pm CEST/8:30am ET

Interested parties may access a live webcast of the conference call via the investor section of the BioMarin website, www.biomarin.com. The Late Breaker slide presentation will be available to download in advance of the call. A replay of the call will be archived on the site for one week following the call.

U.S. / Canada Dial-in Number: (866) 502-9859
International Dial-in Number: (574) 990-1362
Conference ID: 41581309

Replay Dial-in Number: (855) 859-2056
Replay International Dial-in Number: (404) 537-3406
Conference ID: 41581309

About Hemophilia A

Hemophilia A is a genetic disease caused by the deficiency of clotting factor VIII. It is the most common type of hemophilia and occurs much more frequently in males; incidence is estimated at 1 in 4,000-5,000 male births. People born with hemophilia produce little or no clotting factors. The two main types of hemophilia are A and B. People with hemophilia A are missing or have low levels of clotting factor VIII.

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for patients with serious and life-threatening rare and ultra-rare genetic diseases. The Company's portfolio consists of six commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit www.BioMarin.com.

Forward Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: our research on hemophilia A treatment and our potential to change the course of hemophilia A treatment. 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 BMN 270, including final analysis of the Phase 1/2 data; any potential adverse events observed in the continuing monitoring of the patients in the Phase 1/2 trial; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities; a potential decision by the EMA to remove BMN 270 from the PRIME scheme or the accelerated assessment framework; 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 factors detailed in BioMarin's filings with the Securities and Exchange Commission, including, without limitation, the factors contained under the caption "Risk Factors" in BioMarin's 2016 Annual Report on Form 10-K, and the factors contained in BioMarin's reports on Form 10-Q. Stockholders are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin is under no obligation, and expressly disclaims any obligation to update or alter any forward-looking statement, whether as a result of new information, future events or otherwise.

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SOURCE BioMarin Pharmaceutical Inc.
