BioMarin Announces 1 Platform and 3 Poster Presentations at World Federation of Hemophilia 2018 World Congress

Platform Presentation to Highlight Data from Ongoing Phase 1/2 Study to Assess Long-Term Efficacy and Safety of Valoctocogene Roxaparvovec in Severe Hemophilia A

SAN RAFAEL, Calif., May 17, 2018 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that the company will present data in one platform presentation and three poster presentations at the World Federation of Hemophilia (WFH) 2018 World Congress from May 20-24 in Glasgow, Scotland. The platform presentation will highlight results from the ongoing Phase 1/2 study of its investigational gene therapy, valoctocogene roxaparvovec, for people with severe hemophilia A. The data set evaluates the long-term efficacy and safety of both the 6e13 vg/kg and 4e13 vg/kg doses of valoctocogene roxaparvovec.

"As a leader in gene therapy, we appreciate the opportunity to share important emerging data in this scientific forum. We are committed to studying gene therapy, which has the potential to revolutionize care for people with severe hemophilia A," said Hank Fuchs, M.D., President, Worldwide Research and Development at BioMarin. "We look forward to the scientific exchange at the WFH World Congress and to sharing our findings."

The platform presentation, "Achievement of normal factor VIII activity following gene transfer with valoctocogene roxaparvovec (BMN 270): long-term efficacy and safety results in patients with severe hemophilia A," will be presented by primary investigator John Pasi, M.B., Ch.B., Ph.D., Barts and the London School of Medicine and Dentistry, during the "Free Papers: Gene therapy" session from 10:15 – 11:45 AM GMT on Tuesday, May 22.

Presentation of the data at WFH follows the publication of 1.5 years of positive clinical data in the *New England Journal of Medicine* in December 2017 and the dosing of the first participants in the Phase 1/2 study evaluating people with severe hemophilia A with pre-existing AAV5 antibodies and the initiation of the global GENEr8-1 and GENEr8-2 Phase 3 registrational program.

In addition, BioMarin will present the following three posters:

Title	**Authors**
Poster #180: Feasibility study on the psychometric analysis and qualitative assessment of EQ-5D and Haemo-QoL-A May 21 from 15:45 -16:15 GMT	Jacob I, Burke T, Morgan G, Jain M, Camp C, O'Hara J
Poster #51: Prednisolone treatment does not regulate FVIII expression in mice treated with valactocogene roxaparvovec May 23 from 15;45 – 16:15 GMT	Fong S, Murphy R, Xie L, Zhang L, Bullens S, Handyside B, Liu S, Sihn C-R, Siso-Llonch S, Vitelli C, Harmon D, Bunting S
Poster #135: Relationship between treatment strategy and impairment in severe hemophilia May 23 from 15;45 – 16:15 GMT	O'Hara J, Jain M, Burke T, Camp C

Regulatory Status
The U.S. Food and Drug Administration (FDA) granted valoctocogene roxaparvovec Breakthrough Therapy Designation. The FDA's Breakthrough Therapy Designation program is intended to facilitate and expedite development and review of new drugs to address unmet medical need in the treatment of a serious condition. To qualify for Breakthrough Therapy Designation, preliminary clinical evidence must show that that the drug may demonstrate substantial improvement over existing therapies.

The European Medicines Agency (EMA) has granted access to its Priority Medicines (PRIME) regulatory initiative for valoctocogene roxaparvovec. To be accepted for PRIME, an investigational therapy has to show its potential to benefit patients with unmet medical needs based on early clinical data. PRIME focuses on medicines that may offer a major therapeutic advantage over existing treatments, or benefit patients with no treatment options. These medicines are

considered priority medicines within the European Union (EU).

BioMarin's valoctocogene roxaparvovec has also received orphan drug designation from the FDA and EMA for the treatment of severe hemophilia A. The Orphan Drug Designation program is intended to advance the evaluation and development of products that demonstrate promise for the diagnosis and/or treatment of rare diseases or conditions.

Gene Therapy Manufacturing
BioMarin has constructed one of the first gene therapy manufacturing facilities of its kind in the world, which is located in Novato, California. Good Manufacturing Practices (GMP) production of valoctocogene roxaparvovec has commenced and will support pivotal clinical development activities and anticipated commercial demand. This facility is capable of supporting approximately 2,000 to 3,000 patients per year, and the production process was developed in accordance with International Conference on Harmonisation guidance for Pharmaceuticals for Human Use facilitating worldwide registration with health authorities. Recently, the International Society for Pharmaceutical Engineering (ISPE) selected the company's gene therapy manufacturing facility as the 2018 Facility of the Year Category Winner for Project Execution.

About Hemophilia A
Hemophilia A, also called factor VIII (FVIII) deficiency or classic hemophilia, is a 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. People living with the disease are not able to form blood clots efficiently and are at risk for excessive bleeding from modest injuries, potentially endangering their life. People with severe hemophilia often bleed spontaneously 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 two to three times per week. Even with prophylactic regimens, many people still experience spontaneous bleeding events that result in progressive and debilitating joint damage.

About Gene Therapy
Gene therapy is a form of treatment designed to repair a genetic problem by adding a corrected copy of the defective gene. The functional gene is inserted into a vector, containing a small DNA sequence that acts as a delivery mechanism, providing the ability to deliver the functional gene to cells. The cells can then use the information to build the functional proteins that the body needs, potentially reducing or eliminating the cause of the disease.

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 six commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit www.BMRN.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: data presented at WFH, the development of BioMarin's valoctocogene roxaparvovec program generally; the potential benefit of valoctocogene roxaparvovec and of gene therapy generally for people with severe hemophilia A with pre-existing AAV5 antibodies and other sub-groups. 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: timing and results of the current and planned clinical trials of valoctocogene roxaparvovec; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities; our ability to successfully manufacture the product candidate for the clinical trials, and if approved, for the anticipated commercial demand of valoctocogene roxaparvovec; 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, 2018, 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.

Contacts:

Investors	Media
Traci McCarty	*Debra Charlesworth*
BioMarin Pharmaceutical Inc.	*BioMarin Pharmaceutical Inc.*
(415) 455-7558	*(415) 455-7451*

SOURCE BioMarin Pharmaceutical Inc.

---

https://investors.biomarin.com/2018-05-17-BioMarin-Announces-1-Platform-and-3-Poster-Presentations-at-World-Federation-of-Hemophilia-2018-World-Congress