BioMarin Demonstrates Enduring Commitment to Advancing the Standard of Care in Hemophilia A at 15th Annual Congress of the European Association for Haemophilia and Allied Disorders (EAHAD) February 2-4

Latest Safety and Efficacy Data from Valoctocogene Roxaparvovec Studies, Largest and Longest Development Program for any Gene Therapy in Hemophilia A

Oral Presentation to Highlight 2-Year Analysis of Ongoing Global Phase 3 GENEr8-1 Study

SAN RAFAEL, Calif., Feb. 2, 2022 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) today announced that the Company will be presenting data in several presentations on valoctocogene roxaparvovec, an investigational gene therapy for the treatment of adults with severe hemophilia A at the 15th Annual Virtual Congress of the European Association for Haemophilia and Allied Disorders (EAHAD) February 2-4. Notably, there will be an oral presentation of data from the two-year analysis of an ongoing global Phase 3 study, GENEr8-1, the largest Phase 3 gene therapy study in severe hemophilia A with 134 participants and the safety data of the overall program.

On Friday February 4 at 17:10 Central European Time (CET), Prof. Johnny Mahlangu, Professor in Haematology and Head of School of Pathology in the Faculty of Health Sciences of the University of the Witwatersrand and the National Health Laboratory Service in Johannesburg, South Africa, will close the *Latest Clinical Trial Results* session presenting "Efficacy and safety of valoctocogene roxaparvovec gene transfer for severe haemophilia A: Results from the GENEr8-1 two-year analysis." The entire session consisting of six presentations will be chaired by EAHAD President, Prof. Flora Peyvandi and will cover the latest developments in the field of hematology.

Further demonstrating the Company's commitment to advancing the standard of care in severe hemophilia A, BioMarin also will present three posters, which will be available to meeting participants at any time during EAHAD.

Poster Title	**Authors**
Health-related quality of life following valoctocogene roxaparvovec gene therapy for severe haemophilia A in the	O'Mahony B, Mahlangu J, Peerlinck K, Wang JD, Lowe G, Tan C, Tran h, Khoo TL, Cockrell E, Pepperell D, Chambost H, Lopez Fernandez MF, Kazmi R, Majerus E, Skinner M, Klamroth R, Quinn J, Yu H, Wong W, Lawal

phase 3 trial GENEr8-1	A, Robinson T, Kim B
Valoctocogene roxaparvovec gene transfer in participants with HIV	Ragni M, Majerus E, Giermasz A, Fong S, Yates B, Scheeler S, Razon L, Liu S, Yu H, Reddy D, Robinson T
Qualitative research evaluating patient preference for haemophilia therapy	Miesbach W, Valentino L, Noone D, Forsyth K, Bullinger M, Dashiell-Aje E, Newman V, Hawes C, Hawley S, Lewis H, Latibeaudiere-Gardner D, Quinn J

"We are looking forward to sharing these transformational data from the largest and longest development program for gene therapy in severe hemophilia A at EAHAD," said Hank Fuchs, M.D., President of Worldwide Research and Development at BioMarin. "We are committed to helping the community understand the safety and efficacy of this novel investigational treatment."

The 15th Congress of the European Association for Haemophilia and Allied Disorders is taking place from February 2 - 4, 2022. Under the direction of the Organizing Committee, the EAHAD 2022 Virtual Congress aims to continue being a locus for the latest innovations and advances in the field of hemophilia and bleeding disorders.

About Hemophilia A

People living with hemophilia A lack sufficient 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 (FVIII levels <1%) often experience painful, spontaneous bleeds into their muscles or joints. Individuals with the most severe form of hemophilia A make up approximately 50 percent of the hemophilia A population. People with hemophilia A with moderate (FVIII 1-5%) or mild (FVIII 5-40%) disease show a much-reduced propensity to bleed. The standard of care for individuals with severe 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 breakthrough 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 EAHAD

The European Association for Haemophilia and Allied Disorders (EAHAD) is a multidisciplinary association of healthcare professionals who provide care for individuals with hemophilia and other bleeding disorders. Its members include hematologists, internists, pediatricians, nurses, physiotherapists, laboratory scientists and researchers from across Europe.

Since its establishment in 2007, EAHAD has worked to improve the situation of people living with hemophilia and other bleeding disorders.

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., including without limitation, statements about: the oral presentation at EAHAD where the results from the GENEr8-1 two-year analysis will be presented along with the safety data from the overall program, the data presented at EAHAD, including the oral presentation and three posters, the development of BioMarin's valoctocogene roxaparvovec program generally, the impact of valoctocogene roxaparvovec gene therapy for treating patients with severe hemophilia A and the ongoing clinical programs generally. 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 data and additional data from the continuation of these trials and the entire development program, including further assessment of safety events, any potential adverse events observed in the continuing monitoring of the patients in the clinical trials; the content and timing of decisions by the FDA, the EMA 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; 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 Annual and quarterly Reports on Forms 10-K and 10-Q, 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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