BioMarin to Present Findings from Ongoing Studies of Valoctocogene Roxaparvovec, Investigational Gene Therapy, at the World Federation of Hemophilia 2022 World Congress, May 8-11, 2022, Including Five Platform Presentations

Ongoing Clinical Development Program Represents Largest and Longest Development Program for any Gene Therapy in Hemophilia A, Demonstrates Commitment to Advancing Care for People with Hemophilia A

SAN RAFAEL, Calif., May 5, 2022 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ:BMRN) today announced five platform presentations and one poster presentation on valoctocogene roxaparvovec, an investigational gene therapy for the treatment of adults with severe hemophilia A, at the World Federation of Hemophilia (WFH) 2022 World Congress from May 8-11 in Montreal, Canada.

"We're pleased to provide updated data on durability of effect in patients treated over two years ago, on molecular contributors to variability, on hepatotoxicity and the role of immunosuppression, on the potential for integration-related oncogenicity, and on health-related quality of life. We continue to learn about the potential for investigational valoctocogene roxaparvovec to transform lives and the optimal way to manage patients through their journey," said Hank Fuchs, M.D., President of Worldwide Research and Development at BioMarin. "We are progressing our regulatory efforts with the intent to deliver a therapy that may represent an important and valuable treatment choice for patients with severe Hemophilia A, as no presently available treatment offers such freedom from prophylaxis and reduced bleeding frequency at the same time."

"BioMarin is generating valuable data about investigational valoctocogene roxaparvovec that potentially could enable shared decision making between physicians and patients on what is the best therapy in each personal situation," said Professor Wolfgang Miesbach, Head of the Department of Coagulation Disorders and the Comprehensive Care Haemophilia Centre at the Goethe University Hospital in Frankfurt/Main, Germany.

Presentation of data at WFH follows positive two-year results from the ongoing, global phase 3 GENEr8-1 study of valoctocogene roxaparvovec presented at a medical meeting earlier in the year, as well as publication of one-year results from the pivotal clinical trial in the *New England Journal of Medicine* in March 2022.

BioMarin's presentations at WFH include:

Platform Presentations

Immune suppression following gene therapy in Hemophilia
Professor Wolfgang Miesbach, Head of the Department of Coagulation Disorders and the Comprehensive Care Haemophilia Centre, Goethe University Hospital, Frankfurt/Main, Germany
Monday, May 9, 2022, 1:30 -2:30 PM ET

Exploratory analyses of healthy liver biopsies and a single case of parotid acinar cell carcinoma do not identify a role for valoctocogene roxaparvovec vector insertion in altering cell growth
Kevin Eggan, Group Vice President, Head of Research and Early Development / BioMarin
Monday, May 9, 2022, 1:30 -2:30 PM ET

Health-related quality of life over 2 years following valoctocogene

roxaparvovec adeno-associated virus gene transfer for severe hemophilia A: Results from GENEr8-1
Dr. Amy Dunn, Director of Pediatric Hematology, Nationwide Children's Hospital, Columbus, Ohio
Tuesday, May 10, 2022, 1:30-2:30 PM ET

Human liver biopsy analysis showed interindividual variability in transgene mRNA and protein production following adeno-associated virus gene therapy for hemophilia A
Sylvia Fong, Head of Hematology Research, BioMarin
Wednesday, May 11, 2022, 1:30 -2:30 PM ET

Interim 52-week analysis of immunogenicity to the vector capsid and transgene-expressed human FVIII in GENEr8-1, a phase 3 clinical study of valoctocogene roxaparvovec, an AAV5-medicated gene therapy for hemophilia A
Brian Long, Principal Scientist, Clinical Immunology, BioMarin
Wednesday, May 11, 2022, 4-5 PM ET

Poster Presentation

Use of immunosuppressives in patients with hemophilia receiving gene therapy: Evidence generation using a mixed-methods approach
Professor Wolfgang Miesbach, Head of the Department of Coagulation Disorders and the Comprehensive Care Haemophilia Centre, Goethe University Hospital, Frankfurt/Main, Germany

BioMarin-Sponsored Symposia

Gene Therapy Clinical Trial Patient Journey: A Look Into Shared Decision

Making
Monday, May 9, 12:15 – 1:15 PM ET

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 BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare diseases and medical conditions. The Company selects product candidates for diseases

and conditions that represent a significant unmet medical need, have well-understood biology and provide an opportunity to be first-to-market or offer a significant benefit over existing products. The Company's portfolio consists of seven commercial products and multiple clinical and preclinical product candidates for the treatment of various diseases. For additional information, please visit www.biomarin.com.

Forward Looking Statements

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including without limitation, statements about: the data presented at WFH, including the five platform presentations, one poster, and one BioMarin sponsored symposia, 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 potential to transform the lives of these patients 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 factors detailed in BioMarin's filings with the Securities and Exchange Commission (SEC), including, without limitation, the factors contained under the caption

"Risk Factors" in BioMarin's Quarterly Report on Form 10-Q for the quarter ended March 31, 2022 as such factors may be updated by any subsequent reports. 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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https://investors.biomarin.com/2022-05-05-BioMarin-to-Present-Findings-from-Ongoing-Studies-of-Valoctocogene-Roxaparvovec,-Investigational-Gene-Therapy,-at-the-World-Federation-of-Hemophilia-2022-World-Congress,-May-8-11,-2022,-Including-Five-Platform-Presentations