BioMarin Presents Findings from Studies of Valoctocogene Roxaparvovec, Investigational Gene Therapy, at the International Society on Thrombosis and Haemostasis (ISTH) 2022 Congress July 9-12, Including 4 Oral and 2 Poster Presentations

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

European Commission Approval for Valoctocogene Roxaparvovec Expected Q3 2022

SAN RAFAEL, Calif., July 11, 2022 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) today announced that it presented four oral presentations and two poster presentations on valoctocogene roxaparvovec, an investigational gene therapy for the treatment of adults with severe hemophilia A, at the International Society on Thrombosis and Haemostasis (ISTH) 2022 World Congress from July 9-12 in London.

"With each passing year, the breadth of data supporting valoctocogene roxaparvovec increasingly demonstrate the positive impact gene therapy treatment may provide people with severe hemophilia A. These presentations at ISTH offer supportive evidence of long-term hemostatic efficacy, consistent safety results in clinical studies, efficacy from our pivotal study that is consistent with propensity scoring, the clearance of the vector, and on health-related quality of life over six years," said Hank Fuchs, M.D., President of Worldwide Research and Development at BioMarin. "We look forward to an anticipated approval of valoctocogene roxaparvovec in Europe and providing a therapy that could represent an important and valuable treatment choice for patients with severe Hemophilia A by offering the potential to reduce both the burden of the disease as well as the burden of treatment."

"BioMarin continues to increase and share important data about investigational valoctocogene roxaparvovec that may be useful for patients and physicians to evaluate therapeutic options based on an individual's unique circumstances," said one of the presenters Professor Johnny Mahlangu, a study investigator and Professor in Haematology and Head of School of Pathology in the Faculty of Health Sciences of the University of the Witwatersrand in Johannesburg, South Africa.

Presentation of these data at ISTH follows the Committee for Medicinal Products for

Human Use (CHMP) adopting a positive opinion recommending conditional marketing authorization (CMA) for valoctocogene roxaparvovec for adults with severe hemophilia A. Valoctocogene roxaparvovec is the first gene therapy to be recommended for approval in Europe for hemophilia A. A final approval decision, typically consistent with the CHMP recommendation, is expected from the European Commission in Q3 2022.

BioMarin's presentations at ISTH include:

Oral Presentations

Comparative effectiveness of valoctocogene roxaparvovec and prophylactic factor VIII replacement estimated through propensity scoring

Anthony J. Hatswell, PhD, Director and Analyst, Delta Hat Limited, Nottingham, UK

Sunday July 10, 2022, 3:45 PM - 4:00 PM BST

Relationship between transgene-produced FVIII and bleeding rates 2 years after gene transfer with valoctocogene roxaparvovec: Results from GENEr8-1

Professor Johnny Mahlangu, Professor of Haematology and Head of School of Pathology in the Faculty of Health Sciences of the University of the Witwatersrand in Johannesburg, South Africa.

Sunday July 10, 2022, 3:15 PM - 3:30 PM BST

Hemostatic results for up to 6 years following treatment with valoctocogene roxaparvovec, an AAV5-hFVIII-SQ gene therapy for severe hemophilia A

Professor Michael Laffan, faculty of Medicine, Department of Immunology and Inflammation at Imperial College London, Director of the Hammersmith Hospital Haemophilia Centre

Sunday July 10, 2022, 3:00 PM - 3:15 PM BST

Innate and Adaptive Immune Responses to Adeno-associated viral Gene Therapy in the Severe Hemophilia A Dog Model

Paul Batty, MBBS, PhD, Associate Professor at University College London and an Honorary

Consultant in Haemostasis and Thrombosis at the Katharine Dormandy Haemophilia and Thrombosis Centre (Royal Free Hospital, London, UK)

Saturday, July 9, 2022, 2:00 PM – 2:15 PM BST

Posters

Comparability of bleeding outcomes by prophylactic FVIII replacement intensity: A post hoc analysis of a noninterventional study of men with severe hemophilia A

Charlotte Camp, MSc, Associate Director, Health Economics and Outcomes Research, BioMarin

Monday July 11, 2022, 6:30 PM - 7:30 PM BST

Blood Biodistribution and Vector Shedding of Valoctocogene Roxaparvovec in People with Severe Hemophilia A: Results from the Phase 3 GENEr8-1 Trial

Suresh Agarwal, PhD, MS, RPh, Director, Clinical Pharmacology, BioMarin

Sunday July 10, 2022, 6:30 PM - 7:30 PM

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 ISTH, including the four oral and two poster presentations, the development of BioMarin's valoctocogene roxaparvovec program generally, the impact of valoctocogene roxaparvovec gene therapy for treating patients with severe hemophilia A, a final approval decision for valoctocogene roxaparvovec, typically consistent with the CHMP recommendation, expected from the European Commission in Q3 2022 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 data from 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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