**BioMarin to Present ROCTAVIAN™ (valoctocogene roxaparvovec) Data from Longest and Largest Hemophilia Gene Therapy Clinical Trial Program at the International Society on Thrombosis and Haemostasis (ISTH) 2023 Congress**

*New Data to be Presented for ROCTAVIAN from Phase 3 GENEr8-1 Study, Including Data on the Impact of ROCTAVIAN on Quality of Life and Musculoskeletal Health for Adults with Severe Hemophilia A*

SAN RAFAEL, Calif., June 22, 2023 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (Nasdaq: BMRN), a global biotechnology company dedicated to transforming lives through genetic discovery, today announced it will present new data from the ROCTAVIAN™ (valoctocogene roxaparvovec) gene therapy clinical trial program. These data will be shared this week in four oral presentations and two posters at the 31st Congress of the International Society on Thrombosis and Haemostasis (ISTH) in Montreal, Canada.

The U.S. Food and Drug Administration (FDA) has set a PDUFA Target Action Date of June 30, 2023, for the Company's Biologics License Application (BLA) for valoctocogene roxaparvovec gene therapy. The European Commission (EC) granted conditional marketing authorization under the brand name ROCTAVIAN on August 24, 2022.

"With our ROCTAVIAN clinical program, we are embarking on a new era in the treatment of people with severe hemophilia A," said Hank Fuchs, M.D., president of Worldwide Research and Development at BioMarin. "These data presented at ISTH will highlight the clinical impact of ROCTAVIAN for people living with severe hemophilia A more than three years post-infusion."

New data from the ongoing, global Phase 3 GENEr8-1 study – the largest to date for any hemophilia gene therapy – will highlight the impact of ROCTAVIAN on hemostatic efficacy, durability and quality of life of individuals with severe hemophilia A more than three years after infusion. Additional presentations will further explore the impact of ROCTAVIAN on the health and life experiences of people with hemophilia A after two years in the GENEr8-1 study using a tool called PROBE (Patient Reported Outcomes, Burdens and Experiences) and assess the impact of ROCTAVIAN on musculoskeletal health in people with severe hemophilia A.

Key ROCTAVIAN presentations at ISTH include:
Oral Presentations:

Bleeding, FVIII activity, and Safety 3 years after Gene Transfer with Valoctocogene Roxaparvovec: Results from GENEr8-1
OC 20.1
Sunday, June 25, 2023, 2:45 – 3 p.m., Eastern Time

Gene Therapy in Hemophilia A: the Impact of Valoctocogene Roxaparvovec on Patient Outcomes - Initial Results from Patient Reported Outcomes, Burdens and Experiences (PROBE) from the GENEr8-1 Trial
OC 20.2
Sunday, June 25, 2023, 3 – 3:15 p.m., Eastern Time

The Impact of Gene Therapy on the Musculoskeletal Health of Patients with Severe Hemophilia A
OC 20.4
Sunday, June 25, 2023, 3:30 – 3:45 p.m., Eastern Time

Stable Factor VIII Expression and Improvement in Bleeding Phenotype Following Early Childhood Treatment with Adeno-Associated Viral Gene Therapy in the Severe Hemophilia A Dog Model
OC 30.2
Monday, June 26, 2023, 11 – 11:15 a.m., Eastern Time

Posters:

Understanding the Requirement for Additional Factor VIII Infusion Associated with Novel Haemophilia A Treatments: An Expert Elicitation Exercise
PB0633
Monday, June 26, 2023, 6:30 – 7:30 p.m., Eastern Time

Quantitative Pharmacokinetic Model to Characterize and Extrapolate Long-Term FVIII Activity Levels in Patients with Severe Hemophilia A treated with Valoctocogene Roxaparvovec
PB0626
Monday, June 26, 2023, 6:30 – 7:30 p.m., Eastern Time

Robust Clinical Program
BioMarin has multiple clinical studies underway in its comprehensive gene therapy program for the treatment of severe hemophilia A. In addition to the global Phase 1/2 and Phase 3 GENER8-1 studies, the company is also conducting a single arm, open-label study to evaluate the efficacy and safety of ROCTAVIAN at a dose of 6e13 vg/kg with prophylactic corticosteroids in people with severe hemophilia A (Study 270-303). There is also an ongoing study with the 6e13 vg/kg dose of ROCTAVIAN in people with severe hemophilia A with pre-existing AAV5 antibodies (Study 270-203) and a study with the 6e13 vg/kg dose of ROCTAVIAN in people with severe hemophilia A with active or prior Factor VIII (FVIII) inhibitors (Study 270-205).

About Hemophilia A

Hemophilia A, also called FVIII deficiency or classic hemophilia, is an X-linked genetic disorder caused by missing or defective FVIII, 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.

People living with hemophilia A lack sufficient functioning FVIII 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% of the hemophilia A population. People with hemophilia A with moderate (FVIII levels 1-5%) or mild (FVIII levels 5-40%) disease show a much-reduced propensity to bleed. Individuals with severe hemophilia A are treated with a prophylactic regimen of intravenous FVIII infusions administered 2-3 times per week (100-150 infusions per year) or a bispecific monoclonal antibody that mimics the activity of FVIII administered 1-4 times per month (12-48 injections or shots 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.

About BioMarin

Founded in 1997, BioMarin is a global biotechnology company dedicated to transforming lives through genetic discovery. The company develops and commercializes targeted therapies that address the root cause of the genetic conditions. BioMarin's unparalleled research and development capabilities have resulted in eight transformational commercial therapies for patients with rare genetic disorders. The company's distinctive
approach to drug discovery has produced a diverse pipeline of commercial, clinical, and pre-clinical candidates that address a significant unmet medical need, have well-understood biology, and provide an opportunity to be first-to-market or offer a substantial benefit over existing treatment options. 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. (BioMarin), including, without limitation, statements about: the data BioMarin plans to present at the ISTH 2023 Congress, including the four oral and two poster presentations; the development of BioMarin's ROCTAVIAN (valoctocogene roxaparvovec) program generally; the impact of ROCTAVIAN for treating patients with severe hemophilia A, including ROCTAVIAN's impact on quality of life and musculoskeletal health; BioMarin embarking on a new era in the treatment of people with severe hemophilia A with ROCTAVIAN; and the duration and outcomes of the FDA's review of BioMarin's BLA for ROCTAVIAN, including expectations regarding the FDA's review of such BLA by the PDUFA Target Action Date of June 30, 2023. 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 ROCTAVIAN, 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, EU health authorities and other regulatory authorities; the content and timing of decisions by local and central ethics committees regarding clinical trials of ROCTAVIAN; BioMarin's ability to successfully manufacture ROCTAVIAN; 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 year ended March 31, 2023, 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.

BioMarin® is a registered trademark of BioMarin Pharmaceutical Inc., and ROCTAVIAN™ is a trademark of BioMarin Pharmaceutical Inc.