BioMarin Provides 2 Years of Clinical Data in 6e13 vg/kg Dose from Ongoing Phase 1/2 Study in Valoctocogene Roxaparvovec Gene Therapy for Severe Hemophilia A at World Federation of Hemophilia 2018 World Congress

Eliminated Need for Prophylaxis and No Spontaneous Bleeds in Year 2

Quality of Life Scores Continue to Increase in Year 2

Protocol of Global GENEr8-1 (Phase 3) Pivotal Study Amended to Evaluate Superiority Compared to Standard of Care with Increased Enrollment to 130 Participants Anticipated by 1Q 2019

Conference Call and Webcast to be Held Tuesday, May 22, 2018 at 8:30 AM Eastern

SAN RAFAEL, Calif., May 22, 2018 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today an update to its previously reported results of an open-label Phase 1/2 study of valoctocogene roxaparvovec (formerly BMN 270), an investigational gene therapy treatment for severe hemophilia A. The updated results were presented during an oral presentation at the World Federation of Hemophilia (WFH) 2018 World Congress in Glasgow, Scotland by John Pasi, M.B., Ch.B., Ph.D., from Barts and the London School of Medicine and Dentistry and primary investigator for this Phase 1/2 study.

Previously, the Company provided updated data on the 4e13 vg/kg dose cohort and 6e13 vg/kg dose cohort on Dec. 9, 2017 at the American Society of Hematology (ASH) Annual Meeting.

The data presented at WFH is the most current data (Apr. 16, 2018 cut off) and includes 104 weeks of data for the 6e13 vg/kg cohort and 52 weeks of data for

the 4e13 vg/kg cohort.

In the 6e13 vg/kg cohort, the data showed continued and substantial reductions in bleeding requiring Factor VIII infusions with a 97% reduction in mean Annualized Bleed Rate (ABR), with no spontaneous bleeds and elimination of all bleeds in target joints in the second year. 71% and 86% of participants had zero bleeds requiring Factor VIII infusions in years 1 and 2 respectively compared to 14%, who had zero bleeds requiring Factor VIII infusions for a year at baseline. There was a 96% reduction in mean FVIII usage through week 104. Quality of life as measured by the six-domain Haemo-QoL-A instrument rapidly improved across all domains by up to 17.3 points in mean over baseline through the second year. This is well above the 5.2 point increase considered to be the minimal clinically important difference.

The 4e13 vg/kg cohort also showed a substantial reduction in bleeding requiring Factor VIII infusions with a 92% reduction in ABR. 83% of participants had zero bleeds requiring Factor VIII infusions following treatment for a year compared to 17%, who had zero bleeds requiring Factor VIII infusions for a year at baseline. Mean Factor VIII usage decreased by 98%. Consistent with the reduction in ABR and FVIII usage, quality of life showed mean improvement by 3.8 to 6.3 points.

For the 6e13 vg/kg cohort, mean Factor VIII activity levels from week 20 through 104 have been consistently within the normal or near normal range and no participant was above the upper limit of normal at week 104, expressed as a percentage of normal factor activity in blood. At 104 weeks post-infusion, mean Factor VIII activity level of the 6e13 vg/kg cohort is within the normal range at 59%, and the median is near normal at 46%.

For the 4e13 vg/kg cohort, mean Factor VIII activity levels from week 20 through 52 have been consistently within the mild range, expressed as a

percentage of normal factor activity in blood. At 52 weeks post-infusion, mean and median Factor VIII activity levels of the 4e13 vg/kg cohort are 32%.

The Company will be updating the protocol for the GENEr8-1 study evaluating the 6e13 vg/kg dose and will power the study to evaluate superiority to the current standard of care, Factor VIII prophylaxis. The study will now include 130 participants (an increase of 90 from the original 40).

"In order to make this option available with the urgency and data support that people with severe hemophilia A deserve, we plan to raise the sample size of our registrational study, Gener8-1 with the 6e13 dose to demonstrate benefits well beyond prophylactic factor use," said Hank Fuchs, M.D., President, Worldwide Research and Development at BioMarin.

Valoctocogene Roxaparvovec Safety

Overall, valoctogogene roxaparvovec has been well-tolerated by participants across all doses, including the two participants who received the lowest doses of 6e12 and 2e13 vg/kg, respectively. No participants developed inhibitors to Factor VIII, and no participants withdrew from the study. The most common adverse events (AEs) across all dose cohorts were alanine aminotransferase (ALT) elevation (11 participants, 73%); arthralgia (9 participants, 60%); aspartate aminotransferase elevation (8 participants, 53%); headache (7 patients, 47%); back pain and upper respiratory tract infection (6 participants, 40%), and fatigue, insomnia and pain in extremity (5 subjects, 33%). Two participants reported serious adverse events (SAEs) during the study. One participant was hospitalized for observation after developing Grade 2 pyrexia with myalgia and headache within 24 hours of receiving valoctocogene roxaparvovec. The event resolved within 48 hours following treatment with paracetamol, an over-the-counter treatment for pain and fever. The event was

assessed as related to valoctocogene roxaparvovec. The other SAE was assessed as not related to valoctocogene roxaparvovec, attributed to a planned knee surgery to treat hemophilic arthropathy, and Grade 1 in severity. No complications were reported.

Registrational Studies Underway; GENEr8-1 Amended to Evaluate Superiority

The global Phase 3 program includes two studies with valoctocogene roxaparvovec, one with the 6e13 vg/kg dose (GENEr8-1) and one with the 4e13 vg/kg dose (GENEr8-2). With the new goal of evaluating superiority of valoctocogene roxaparvovec to the current standard of care, prophylactic therapy, the sample size of the GENEr8-1 study will be increased to approximately 130 total participants from 40 participants. The amended study is now powered to evaluate superiority to the current standard of care. Enrollment completion in the newly amended GENEr8-1 study is expected in the first quarter of 2019.

GENEr8-2, the ongoing Phase 3 study with the 4e13 vg/kg dose, remains unchanged with an N=40. The GENEr8-2 study is expected to complete enrollment one to two quarters after GENEr8-1 in 2019.

BioMarin now has six clinical studies underway in its comprehensive gene therapy program for the treatment of severe hemophilia A. In addition to the two global Phase 3 studies GENEr8-1 and GENEr8-2, the Company recently began a Phase 1/2 Study with the 6E13kg/vg dose of valoctocogene roxaparvovec in approximately 10 participants with pre-existing AAV5 antibodies. Two additional and separate studies, one to study seroprevalence in people with severe hemophilia A and one non-interventional study to determine baseline characteristics in people with hemophilia A, are ongoing around the world. Participants in the Phase 1/2 dose escalation study updated

today at the WFH 2018 meeting will continue to be monitored as part of the global program underway.

Valoctocogene roxaparvovec investigational product from BioMarin's commercial scale manufacturing facility will be used in all BioMarin interventional studies going forward. Product to support the additional participants in the GENEr8-1 has been produced and is immediately available.

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 this manufacturing facility will support pivotal clinical development activities and anticipated commercial demand, if valoctocogene roxaparvovec is approved. 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.

Conference Call and Webcast to be Held May 22 at 8:30 AM Eastern Time

Interested parties may access a live video webcast that will include audio and slides of the presentations via the investor section of the BioMarin website. A replay of the meeting will be archived on the site for one week.

For those who choose not to listen and view the event via webcast, dial-in information for the audio portion of the webcast can be accessed using:

U.S. / Canada Dial-in Number: (866) 502-9859
International Dial-in Number: (574) 990-1362
Conference ID: 4273016

Replay Dial-in Number: (855) 859-2056

Replay International Dial-in Number: (404) 537-3406
Conference ID: 4273016

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 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.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.(BioMarin), including without limitation, statements about the development of BioMarin's valoctocogene roxaparvovec program generally; the timing of BioMarin's clinical studies and trials and announcements from BioMarin's Phase 3 program and Phase1/2 study with valoctocogene roxaparvovec; the impact of valoctocogene roxaparvovec gene therapy for treating people with severe hemophilia A; the potential for valoctocogene roxaparvovec to bring Factor VIII levels to normal, near normal or mild, and to reduce or eliminate bleeds, reduce the number of Factor VIII infusions, and improve the quality of life for people with severe hemophilia A; statements about BioMarin's amendment to the protocol for the GENE r8-1 study evaluating the 6e13 vg/kg dose with the goal of evaluating superiority of valoctocogene roxaparvovec to the current standard of care and the planned increase of participants in the clinical trial; the adequacy of production of valoctocogene roxaparvovec in the Company's commercial gene therapy manufacturing facility; the ongoing Phase 1/2 study, Phase 1/2 study in people with AAV5+, or other possible future clinical studies of valoctocogene roxaparvovec. 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 interim data; any potential adverse events observed in the continuing monitoring of the participants in the clinical trials; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission 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 for the clinical trials and commercially, if approved; 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.

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