BioMarin Provides Highlights of 5 Years of Clinical Data from Ongoing Phase 1/2 Study of Valoctocogene Roxaparvovec with the Longest Duration of Clinical Experience for a Gene Therapy in Hemophilia A

All Study Participants in 6e13 vg/kg and 4e13 vg/kg Dose Cohorts Remain off Factor VIII Prophylactic Therapy

95% Reductions in Mean Annualized Bleed Rate (ABR) and 96% Reduction in Mean Annualized Factor VIII Usage Through Year 5 in 6e13 vg/kg Dose Cohort

92% Reductions in Mean ABR and 95% Reduction in Mean Annualized Factor VIII Usage Through Year 4 in 4e13 vg/kg Dose Cohort

MAA Submission to EMA on Track for June 2021

Data to be Shared in Oral Presentation at Upcoming International Society on Thrombosis and Haemostasis (ISTH) 2021 Virtual Congress (July 17-21)

SAN RAFAEL, Calif., May 19, 2021 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today an update to its previously reported results from an open-label Phase 1/2 study of valoctocogene roxaparvovec, an investigational gene therapy treatment for adults with severe hemophilia A. The Company plans to share the data during an oral presentation at the upcoming International Society on Thrombosis and Haemostasis (ISTH) 2021 Virtual Congress (July 17-21).[i]

Five-year and four-year post-treatment follow-up of the 6e13 vg/kg and 4e13 vg/kg cohorts, respectively, shows a sustained treatment benefit of valoctocogene roxaparvovec. All participants in both cohorts remain off

prophylactic Factor VIII treatment. Mean cumulative annualized bleed rates (ABR) remain less than one in the 6e13 vg/kg cohort and substantially below pre-treatment baseline levels; the mean ABR in year five for the 6e13 vg/kg cohort was 0.7 with an ABR reduction of 95% and Factor VIII use reduction of 96% through five years, compared to pre-infusion. The mean ABR in year four for the 4e13 vg/kg cohort was 1.7 with a mean cumulative ABR reduction of 92% and Factor VIII use reduction of 95% through four years, compared to pre-infusion. Factor VIII activity levels declined commensurate with the most recent years' observations and continue to remain in a range to provide hemostatic efficacy.

"The latest data update in this ongoing study represents the longest duration of clinical experience for any gene therapy in hemophilia A and demonstrates hemostatic control with valoctocogene roxaparvovec out to five years in the majority of patients in this study. With this prolonged period of observation, we gain further insight about the long-term relationship between Factor VIII expression following a single infusion of gene therapy, and the low levels of bleeds in the absence of prophylactic therapy," said Professor Michael Laffan, faculty of Medicine, Department of Immunology and Inflammation at Imperial College London, Director of the Hammersmith Hospital Haemophilia Centre, and Chief Investigator for the valoctocogene roxaparvovec Phase 1/2 study. "As a treating physician and the physician who dosed these study participants some five or more years ago, I am heartened that most of these patients have been free from bleeds and the burden of regular infusions for such a long period of time in the ongoing research into valoctocogene roxaparvovec."

"We are encouraged by the consistent and dramatic bleed control observed for up to five years in this study, representing a milestone in the development of gene therapy in this patient population. We believe these data combined with the compelling Phase 3 data we shared earlier this year, demonstrating superiority to Factor VIII prophylaxis in reducing annualized bleeding rate,

provide cogent evidence to support a determination of clinical benefit," said Hank Fuchs, M.D., President, Worldwide Research and Development at BioMarin. "It is promising that Factor VIII levels continue to remain in a range to provide hemostatic efficacy for the vast majority of patients for a meaningful period of time. Given the continued decline in Factor VIII expression, future investigation of variability and re-dosing remain critical objectives. It continues to be an honor and a privilege to be developing the potential first gene therapy in hemophilia A."

Safety Summary

Overall, the safety profile of valoctocogene roxaparvovec in the Phase 1/2 study remains consistent with previously reported data with no delayed-onset treatment related adverse events. All participants continue to remain off corticosteroids since the first year. No participants developed inhibitors to Factor VIII, and no participants withdrew from the study. No participants have developed thrombotic events. The most common adverse events associated with valoctocogene roxaparvovec occurred early after a single infusion and included short-lived infusion-associated reactions and transient, asymptomatic, and mild to moderate rise in the levels of certain proteins and enzymes measured in liver function tests with no long-lasting clinical sequelae.

Regulatory Status

In Europe, BioMarin plans to submit a Marketing Authorization Application (MAA) for valoctocogene roxaparvovec for the treatment of severe hemophilia A with one-year results from the Phase 3 GENEr8-1 study to the European Medicines Agency (EMA) in June 2021 based on positive feedback from EMA earlier this year.

In the United States, BioMarin plans to submit two-year follow-up safety and efficacy data on all study participants from the GENEr8-1 study in response to FDA's request for these data to support their benefit-risk assessment of valoctocogene roxaparvovec. BioMarin is targeting a Biologics License Application (BLA) submission in the second quarter of 2022 assuming favorable study results, followed by an expected six-month review procedure by the FDA.

The FDA granted Regenerative Medicine Advanced Therapy (RMAT) designation to valoctocogene roxaparvovec in March 2021. RMAT is an expedited program intended to facilitate development and review of regenerative medicine therapies, such as valoctocogene roxaparvovec, that are intended to address an unmet medical need in patients with serious conditions. The RMAT designation is complementary to Breakthrough Therapy Designation, which the Company received in 2017.

In addition to the RMAT Designation and Breakthrough Therapy Designation, BioMarin's valoctocogene roxaparvovec also has 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.

Robust Clinical Program

BioMarin has multiple clinical studies underway in its comprehensive gene therapy program for the treatment of hemophilia A. In addition to the global Phase 3 study GENEr8-1 and the ongoing Phase 1/2 dose escalation study, the Company is actively enrolling participants in a Phase 3b, single arm, open-label study to evaluate the efficacy and safety of valoctocogene roxaparvovec at a dose of 6e13 vg/kg with prophylactic corticosteroids in people with severe

hemophilia A. The Company is running a Phase 1/2 Study with the 6e13kg/vg dose of valoctocogene roxaparvovec in approximately 10 participants with pre-existing AAV5 antibodies, as well as another Phase 1/2 Study with the 6e13 vg/kg dose of valoctocogene roxaparvovec in people with hemophilia A with active or prior FVIII inhibitors.

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 patients with 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 Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including without limitation, statements about: (i) the development of BioMarin's valoctocogene roxaparvovec program generally, (ii) the impact of valoctocogene roxaparvovec gene therapy for treating patients with severe hemophilia A, (iii) our plans to submit a Marketing Authorization Application (MAA) for valoctocogene roxaparvovec for the treatment of severe hemophilia A with one-year results from the Phase 3 GENEr8-1 study to the European Medicines Agency (EMA) in June 2021 based on positive feedback from EMA earlier this year, (iv) the planned updates of the Phase 1/2 study including the Company's upcoming oral presentation of the data at ISTH 2021, (v) our plans to submit two-year follow-up safety and efficacy data on all study participants from the GENEr8-1 study in response to FDA's request for these data to support their benefit-risk assessment of valoctocogene roxaparvovec, (vi) our target Biologics License Application (BLA) submission date in the second quarter of 2022 assuming favorable study results, followed by an expected six-month review procedure by the FDA, (vii) the potential approval and commercialization of valoctocogene roxaparvovec for the treatment of severe hemophilia A, including timing of such approval decisions.

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 patients in the Phase 1/2 trial; the content and timing of decisions by the FDA, the European Commission and other regulatory authorities, including the potential impact of the COVID-19 pandemic on the regulatory authorities' abilities to issue such decisions and the timing of such decisions; the content and timing of decisions by local and central ethics committees regarding the clinical trials; BioMarin's 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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2021, 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.

BioMarin® is a registered trademark of BioMarin Pharmaceutical Inc.

[i] *ISTH provided approval to BioMarin to release information on the Company sharing data at the ISTH Virtual Congress to be held from July 17-21, 2021.*

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