BioMarin Provides Updates on Progress in Gene Therapy Programs

SAN RAFAEL, Calif., Feb. 17, 2022 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced updates on its investigational gene therapy programs in clinical development. In February 2022, the Company received additional requests from the Food and Drug Administration (FDA) for information needed to resolve the clinical hold of the PHEARLESS Phase 1/2 study of BMN 307 issued in September 2021. BMN 307 is an AAV5-human phenylalanine hydroxylase (hPAH) gene therapy being studied in adults with phenylketonuria (PKU). The FDA has requested data from additional non-clinical studies to assess the theoretical oncogenic risk to human study participants, which is expected to take several quarters. The company will communicate next steps for the program when available.

"As leaders in the development of gene therapies, a novel treatment modality, it is our responsibility to answer new questions that arise for the benefit of patients, physicians, regulatory bodies, and for the field in general. Patient safety is our utmost priority," said Hank Fuchs, M.D., President, Worldwide Research and Development at BioMarin. "With new technologies in healthcare, we anticipate that both health authorities and developers will seek to characterize and evaluate potential safety-related signals to enable a more comprehensive assessment of these potential risks to patients. We remain grateful to all of the participants and investigators in our gene therapy studies, as well as for the support from patient advocacy groups."

The Company also announced that enrollment has completed in the ongoing Phase 3 Study 270-303 to evaluate the safety and effectiveness of valoctocogene roxaparvovec, an AAV5 gene therapy, in combination with prophylactic corticosteroids in people with severe hemophilia A. Results from the 52-week analysis from this study are expected in 1H 2023.

BioMarin has multiple clinical studies underway in its comprehensive gene therapy program for the treatment of hemophilia A that remain ongoing and/or continue to enroll participants, including the global Phase 3 study GENEr8-1 and the ongoing Phase 1/2 dose escalation study. In addition, the Company is running a Phase 1/2 Study with the 6e13 vg/kg 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. Finally, the Company announced that its Phase1/2 HAERMONY study to

evaluate BMN 331, an investigational AAV5-mediated gene therapy for people living with hereditary angioedema (HAE) is open for enrollment. In addition, the FDA granted Orphan Disease Designation status to BMN 331.

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 seven commercialized products and multiple clinical and preclinical product candidates. For additional information, please visit www.biomarin.com. Information on such a 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 impact of the preclinical findings on the BMN 307 clinical program, the predictability of the preclinical findings on human patients, the development of BioMarin's BMN 307 program generally, including the impact on the timing and process for regulatory interactions and decisions, the timing on when next steps for the BMN 307 program will be announced, the expectation that the data requested by the FDA from additional non-clinical studies to assess the theoretical oncogenic risk to human study participants in the BMN 307 clinical trial will take several quarters to complete and the results from the Phase 3 study to evaluate the safety and effectiveness of valoctocogene roxaparvovec in combination with prophylactic corticosteroids in people with severe hemophilia A expected in the first half of 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: the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities; uncertainties inherent in research and development, the outcome of ongoing review of clinical and preclinical data of BMN 307, BMN 331 and valoctocogene roxaparvovec; the outcome of additional experiments related to the preclinical findings; the results and timing of current and future clinical trials related to BMN 307, BMN 331, and valoctocogene roxaparvovec; any potential adverse events observed in the monitoring of the patients in the clinical trials for these programs; 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" and elsewhere in BioMarin's Securities and Exchange Commission (SEC) filings, including

BioMarin's Annual and quarterly Reports on Forms 10-K and 10-Q, 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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