BioMarin Announces Presentation of Vosoritide Phase 3 Data in Children with Achondroplasia at the American Society for Bone and Mineral Research 2020 Annual Meeting

SAN RAFAEL, Calif., Sept. 11, 2020 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) today announced that Lynda Polgreen, MD, MS, Lundquist Institute for Biomedical Innovation at Harbor-UCLA Medical Center, Torrance, CA, USA will present data from the randomized, double-blind, phase 3, placebo-controlled, multicenter trial for vosoritide, an investigational analog of C-type Natriuretic Peptide (CNP), in children aged 5 to 18 years with achondroplasia at the American Society for Bone and Mineral Research (ASBMR) Annual 2020 Meeting. The data will be presented during a virtual oral presentation on Saturday, September 12 at 11:50am ET. Achondroplasia is the most common form of disproportionate short stature in humans.

"We are pleased to share this phase 3 clinical data with the research community at this important conference and contribute to the growing body of scientific information about vosoritide, an investigational therapeutic to address the underlying molecular pathology in children with achondroplasia," said Hank Fuchs, M.D., President, Worldwide Research and Development at BioMarin. "This study is part of a robust clinical program, and we are grateful to the participating children, families and physicians."

Regulatory Status

BioMarin has previously announced that the European Medicines Agency (EMA) validated the Company's Marketing Authorization Application and that a New Drug Application (NDA) for vosoritide has been submitted to the U.S. Food and Drug Administration (FDA). Vosoritide has also received orphan drug

designation from the FDA and EMA for the treatment of children with achondroplasia. 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.

Description of Phase 3 Study

The global Phase 3 study was a randomized, double-blind, placebo-controlled study of vosoritide in 121 children with achondroplasia aged 5 to 14 for 52 weeks. (The enrollment age criteria were 5 to 18 per the study protocol). Vosoritide is being tested in children whose growth plates are still open. This is approximately 25% of people with achondroplasia. Children in the Phase 3 study completed a minimum six-month baseline study to determine their baseline growth velocity prior to entering the Phase 3 study. The primary endpoint of the study was the change in growth velocity from baseline over one year in children treated with vosoritide compared to placebo. Children in the study will continue to be evaluated in an ongoing open-label extension study where all study participants receive active treatment until the children participating in this study reach final adult height.

About Achondroplasia

Achondroplasia, the most common form of disproportionate short stature in humans, is characterized by slowing of endochondral ossification, which results in disproportionate short stature and disordered architecture in the long bones, spine, face and base of the skull. This condition is caused by a mutation in the fibroblast growth factor receptor 3 gene (*FGFR3*), a negative regulator of bone growth. Beyond disproportionate short stature, people with achondroplasia can experience serious health complications, including foramen magnum compression, sleep apnea, bowed legs, mid-face hypoplasia, permanent sway

of the lower back, spinal stenosis and recurrent ear infections. Some of these complications can result in the need for invasive surgeries such as spinal cord decompression and straightening of bowed legs. In addition, studies show increased mortality at every age.

More than 80% of children with achondroplasia have parents of average stature and have the condition as the result of a spontaneous gene mutation. The worldwide incidence rate of achondroplasia is about one in 25,000 live births. Vosoritide is being tested in children whose growth plates are still "open", typically those under 18 years of age. This is approximately 25% of people with achondroplasia. In the U.S., Europe, Latin America, the Middle East, and most of Asia Pacific, there are currently no approved medicines for achondroplasia.

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 Statement

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 vosoritide development program generally, the regulatory review of the marketing applications for vosoritide by the U.S. Food and Drug Administration (FDA) and the European Medicines Authority (EMA), the continued clinical development of

vosoritide and the timing and conduct of such clinical program. *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 vosoritide; our ability to enroll participants into such clinical trials, our ability to successfully manufacture vosoritide; the content and timing of decisions by the FDA, EMA and other regulatory authorities concerning vosoritide; and those other risks and uncertainties detailed from time to time under the caption "Risk Factors" and elsewhere in the BioMarin's Securities and Exchange Commission (SEC) filings, including, without limitation, BioMarin's Quarterly Report on Form 10-Q for the quarter ended June 30, 2020, and future SEC 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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