BioMarin Submits New Drug Application to U.S. Food and Drug Administration for Vosoritide to Treat Children with Achondroplasia

Potential first treatment for achondroplasia in the United States
European Regulatory Review Began on Aug. 13

SAN RAFAEL, Calif., Aug. 20, 2020 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) today announced that it has submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for vosoritide, an investigational, once daily injection analog of C-type Natriuretic Peptide (CNP) for children with achondroplasia, the most common form of disproportionate short stature in humans. BioMarin recently announced that the European Medicines Agency (EMA) validated the Company's Marketing Authorization Application (MAA) for vosoritide on Aug. 13, 2020.

"We are grateful to the children and families who have participated in the clinical studies to evaluate the potentially first pharmacological treatment option for children with achondroplasia. We are proud of our team and clinical partners, who have conducted extensive scientific and clinical research to address the underlying cause of the condition," said Hank Fuchs, M.D., President, Global Research and Development at BioMarin. "This is an important step to bring a treatment where none have existed before. We also recognize a broad range of views about treatment options, and we look forward to providing children with achondroplasia and their families a treatment choice with this potential new therapy."

"This regulatory submission is an important scientific and medical milestone for children with achondroplasia and their families," said Julie Hoover-Fong, MD, PhD, FACMG, Professor, McKusick-Nathans Department of Genetic Medicine and Director, Greenberg Center for Skeletal Dysplasias at Johns Hopkins University and an investigator in the vosoritide clinical program. "The extensive clinical program for vosoritide includes important natural history information, which has contributed to the body of scientific knowledge about achondroplasia and a potential treatment option for patients."

"This regulatory submission brings our community closer to accessing the first therapeutic choice for children and families," said Chandler Crews, Founder of The Chandler Project. "A well-researched drug treatment choice has the potential to be an important resource for the community and to increase our understanding of the scientific underpinnings of achondroplasia."

The Chandler Project is dedicated to the most common form of dwarfism and other congenital abnormalities and is a means for patients and parents of children with achondroplasia to find the correct resources for themselves and their child.

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 licensed 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: BioMarin's vosoritide development program generally and specifically about the Company's submission of an NDA for vosoritide to the FDA and the EMA's validation of the MAA for vosoritide, which began on August 13, 2020. 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: our ability to successfully manufacture vosoritide; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission 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.

Contacts:	
Investors	*Media*
Traci McCarty	*Debra Charlesworth*
BioMarin Pharmaceutical Inc.	*BioMarin Pharmaceutical Inc.*
(415) 455-7558	*(415) 455-7451*

SOURCE BioMarin Pharmaceutical Inc.
