European Medicines Agency Validates BioMarin's Marketing Authorization Application for Vosoritide to Treat Children with Achondroplasia

Potential first medicine to treat Achondroplasia in EU

SAN RAFAEL, Calif., Aug. 13, 2020 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today that the European Medicines Agency (EMA) validated the Company's Marketing Authorization Application (MAA) 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. The MAA review will commence on August 13, 2020.

The company remains on track to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) in the third quarter of 2020. Vosoritide has Orphan Drug designation from the FDA and the EMA.

"The acceptance of the vosoritide application for review by the EMA is the culmination of years of scientific and clinical research to bring the potential first pharmacological treatment option for children with achondroplasia," said Hank Fuchs, M.D., President Worldwide Research and Development at BioMarin. "Throughout our journey, we have worked together with advocacy groups and physicians to address the underlying cause and associated complications of achondroplasia through a robust data set for regulators to evaluate the safety and efficacy of vosoritide. We are committed to providing children with achondroplasia and their families a treatment option and look forward to continue working with the European regulatory authorities."

"The extensive research conducted in the Phase 2 and 3 clinical trials, along with a natural history study, has provided more scientific and medical knowledge around skeletal dysplasia, especially achondroplasia, than we've ever had before," said Melita Irving, Clinical Geneticist at Guy's and St Thomas' NHS Foundation Trust, London, UK and investigator for the vosoritide clinical program at the Evelina London Children's Hospital. "These clinical studies provide a much better understanding of how to treat the underlying conditions associated with achondroplasia more effectively and less invasively, and with no drug therapy options previously available, vosoritide has the potential to make a meaningful impact on the daily lives of these children."

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 planned submission of an NDA to the FDA in the third quarter of 2020 for vosoritide and its anticipation that the review of the MAA for vosoritide by the EMA will begin 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.

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