# BioMarin Submits Marketing Authorization Application to European Medicines Agency for Vosoritide to Treat Children with Achondroplasia

***If approved, vosoritide would be the first medicine to treat Achondroplasia in EU***

SAN RAFAEL, Calif., July 23, 2020 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today that the company submitted a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) 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. Subject to completion of EMA's validation check, BioMarin anticipates the start of the MAA review to commence in August 2020.

The marketing application is based on the outcomes from the randomized, double-blind, placebo-controlled Phase 3 study evaluating the efficacy and safety of vosoritide, announced in December 2019, and further supported by the long-term safety and efficacy from the ongoing Phase 2 and Phase 3 extension studies, and extensive natural history data. If approved, vosoritide would be the first medicine for the treatment of achondroplasia in Europe.

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.

"Years of scientific research have led to this important point in the development of the potentially first pharmacological treatment option for children with achondroplasia.  We have worked alongside patient advocacy groups from around the world throughout the development, and we appreciate the implications of developing a treatment option for this community, recognizing that this potential new treatment would offer a choice for families who have a child with achondroplasia," said Hank Fuchs, M.D., President Worldwide Research and Development at BioMarin.  "Our goal is to provide a treatment option that addresses the underlying cause of the condition and over time demonstrate a reduction of complications that may result from achondroplasia.  We respect the depth and breadth of views among the community about treatment options and have sought to be scientifically rigorous in providing a robust data set for regulators to evaluate the safety and efficacy of vosoritide.  We remain grateful to the physicians and families who have participated in our studies that have helped increase the scientific understanding of

this investigational treatment."

"This is an important milestone bringing the achondroplasia community one step closer to a potential therapy," said Klaus Mohnike, Professor of Paediatrics at Magdeburg University Hospital in Germany and investigator for the vosoritide clinical program. "Our scientific and medical knowledge around skeletal dysplasias and achondroplasia in particular continues to increase, which can help us treat the underlying cause of the condition and potentially make a meaningful impact on the lives of children with achondroplasia."

"It is of critical importance that children with achondroplasia and their families have drug treatment options to advance the standard of care for this condition where currently none are available," said Carmen Alonso Alvarez, Managing Director of Fundacion ALPE Foundation. "We look forward to expanding treatment options that can contribute to the improvement of the health and well-being of children with achondroplasia."

**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 in August 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: final analysis of the Phase 3 data, results and timing of current and planned preclinical studies and clinical trials of vosoritide; 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 March 31, 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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