BioMarin Doses First Participant in Phase 2 Study of Vosoritide for Treatment of Infants and Young Children with Achondroplasia

SAN RAFAEL, Calif., June 14, 2018 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ:BMRN) announced today that the company dosed the first participant in a global Phase 2 study for vosoritide, an analog of C-type Natriuretic Peptide (CNP), in infants and young children with achondroplasia, the most common form of disproportionate short stature in humans.

"Vosoritide represents an innovative therapy to treat the underlying cause of achondroplasia. At the molecular level, vosoritide corrects the signaling process that determines skeletal growth and proportionality of bones, while the body is still growing," said Hank Fuchs, M.D., President, Worldwide Research and Development at BioMarin. "We are pleased to have initiated this study in the youngest people with achondroplasia and are grateful to the children and their families who have been participating in our ongoing studies and those who are now participating in this Phase 2 study."

The Phase 2 study is a randomized, placebo-controlled study of vosoritide in approximately 70 infants and young children with achondroplasia ages zero to less than 60 months for 52 weeks. The study will be followed by a subsequent open-label extension. Children in this study will have completed a minimum three-month baseline study to determine their respective baseline growth prior to entering the Phase 2 study. The primary objectives of the study are to evaluate safety, tolerability, and the effect of vosoritide on height Z-scores, which is the number of standard deviations in relation to the mean height of age-matched, average stature children. The company also plans to augment the height Z-score data with assessments including proportionality, functionality, quality of life, sleep apnea, and foramen magnum dimension, as well as the advent of major illnesses and surgeries.

Vosoritide has been granted orphan drug designation in both the United States and Europe.

About Achondroplasia
Achondroplasia, a skeletal dysplasia and the most common form of disproportionate short stature in humans, is characterized by failure of normal conversion of cartilage into bone, which results in disproportionate short stature.

Beyond short stature, people with achondroplasia can experience serious health complications, including foramen magnum compression, spinal stenosis, sleep apnea, bowed legs, mid-face hypoplasia, permanent sway of the lower back, recurrent ear infections, and long term chronic pain. Some of these complications can result in invasive surgeries such as spinal cord decompression and straightening of bowed legs. In addition, studies show increased mortality at every age.[i,ii]

More than 80 percent 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.[iii]

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare disorders. The company's portfolio consists of seven commercialized products and multiple clinical and pre-clinical product candidates.

For additional information, please visit www.BMRN.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 program generally; the potential benefits of vosoritide for infants and young children; the continued clinical development of vosoritide; the timing, design and conduct of the planned Phase 2 study in infants and young children and of other ongoing and possible future studies of vosoritide; the expected results of such studies, the ability to use the primary objectives of the Phase 2 study to support the use of vosoritide in infants and young children; and actions by regulatory authorities. 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 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

Company's Securities and Exchange Commission (SEC) filings, including, without limitation, BioMarin's Quarterly Report on Form 10-Q for the quarter ended March 31, 2018, 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.

BioMarin® is a registered trademark of BioMarin Pharmaceutical Inc.

[i] Hecht JT, Francomano CA, Horton WA, Annegers JF. Am J Hum Genet. 1987; 41: 454-464.

[ii] Wynn J, King TM, Gabello MJ, Waller DK, Hecht JT. *Am J Med Genet A.* 2007; 143A: 2502-2511.

[iii] Waller DK, Correa A, Vo TM, Wang Y, Hobbs C, Langlois PH, Pearson K, Romitti PA, Shaw GM, Hecht JT. 2008. The population-based prevalence of achondroplasia and thanatophoric dysplasia in selected regions of the US. Am J Med Genet Part A 146A:2385–2389.

Contact:

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

SOURCE BioMarin Pharmaceutical Inc.
