BioMarin Enrolls First Participant in Phase 3 Trial of Vosoritide for Treatment of Children with Achondroplasia

SAN RAFAEL, Calif., Dec. 12, 2016 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (NASDAQ:BMRN) announced today that the company has initiated a global Phase 3 study for vosoritide, an analog of C-type Natriuretic Peptide (CNP), in children with achondroplasia, the most common form of dwarfism. The first child enrolled in the study was at a site in Australia.

"Based on the positive results seen in our Phase 2 study, we look forward to continuing to investigate vosoritide," said Hank Fuchs, M.D., President, Worldwide Research and Development at BioMarin. "We are grateful to the children and their families, who have participated in the earlier study and are participating in this Phase 3 study."

The Phase 3 study is a randomized, placebo-controlled study of vosoritide in approximately 110 children with achondroplasia ages 5-14 for 52 weeks. The study will be followed by a subsequent open-label extension. Children in this study will have completed a minimum six-month baseline study to determine their respective baseline growth velocity prior to entering the Phase 3 study. Vosoritide is being tested in children whose growth plates are still open. This is approximately 25 percent of people with achondroplasia.

The primary endpoint of the study is the change in growth velocity from baseline over one year in children treated compared to placebo. The company also plans to augment the growth velocity data with assessments of proportionality and functionality.

In addition, BioMarin is planning a separate Phase 2 study evaluating the effect of vosoritide in infants and toddlers. Vosoritide has been granted orphan drug designation in both the United States and Europe.

About Achondroplasia
Achondroplasia, the most common form of human dwarfism, 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, 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 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 five 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., including, without limitation, statements about: the development of vosoritide; the continued clinical development of vosoritide; the timing, design and conduct of the planned Phase 3 and Phase 2 studies; the expected results of such studies, the ability to use the primary endpoint to support registration of vosoritide; 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 patients 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 factors detailed in BioMarin's filings with the Securities and Exchange Commission, including, without limitation, the factors contained under the caption "Risk Factors" in BioMarin's 2015 Annual Report on Form 10-K, and the factors contained in BioMarin's reports on Form 10-Q. Stockholders are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin is under no obligation, and expressly disclaims any obligation to update or alter any forward-looking statement, whether as a result of new information, future events or otherwise.

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[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:
Traci McCarty
BioMarin Pharmaceutical Inc.
(415) 455-7558

Media:
Debra Charlesworth
BioMarin Pharmaceutical Inc.
(415) 455-7451

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