BioMarin's Initial 6-Month Data from Phase 2 Study of Vosoritide (BMN 111) in Children with Achondroplasia Presented at the American Society for Bone and Mineral Research Annual 2015 Meeting

SAN RAFAEL, Calif., Oct. 12, 2015 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that Dr. Melita Irving, Clinical Geneticist, Guy's and St Thomas' NHS Foundation Trust, Evelina Children's Hospital London, UK, presented the initial six-month data from the first three cohorts of a Phase 2 proof-of-concept and dose-finding study of vosoritide (BMN 111), an analog of C-type Natriuretic Peptide, in children with achondroplasia at the American Society for Bone and Mineral Research Annual 2015 Meeting in Seattle, Washington. Achondroplasia is the most common form of human dwarfism.

The initial six-month data from the first three cohorts showed a 50 percent or 2.01 cm/year increase in mean annualized growth velocity (speed at which growth in children occurs) in the cohort of 10 patients receiving a 15 µg/kg dose of vosoritide daily for six months compared with their own pre-treatment growth velocity (p-value=0.01). Data suggests that vosoritide activity was sustained over six months of dosing as measured by increases in cyclic guanosine monophosphate (cGMP), a urinary marker of pharmacological activity. No serious or severe adverse events were observed and the most common adverse events reported were mild injection site reactions, asymptomatic hypotension and headache that were resolved without medical intervention (Grade 1). BioMarin previously announced these data in June this year and indicated that these data would be presented at an upcoming medical meeting.

"We are pleased to share this initial six-month data from the first three cohorts in a scientific forum, and we are grateful to the children, families and physicians who have participated in this study. By developing a therapy that addresses the root cause of achondroplasia, we hope to address the associated complications, such as disproportionate bone growth," said Hank Fuchs, M.D., Executive Vice President and Chief Medical Officer at BioMarin. "We are very encouraged with this initial data, and we look forward to working with health authorities and the patient community to advance vosoritide to the next stage of clinical development."

While the Phase 2 efficacy endpoint centers on annualized growth velocity after six months of treatment, BioMarin believes that growth velocity may be an important early indicator and that longer treatment may lead to improvement in many of the complications that can be associated with achondroplasia, such as disproportionality, though longer time of treatment is likely required to accumulate evidence to this effect.

Vosoritide is the designated generic name for BMN 111 and was issued by the International Nonproprietary Names (INN) system managed by the World Health Organization (WHO). Vosoritide has Orphan designation in both the United States and Europe.

**Phase 2 Study Design**

Children in this study completed or will have completed a minimum six-month natural history study to determine their respective baseline growth velocity prior to entering the Phase 2 study with vosoritide. The Phase 2 trial is an open-label, sequential cohort dose-escalation study of vosoritide in children with achondroplasia. In the current three dose cohorts, patients were treated with either 2.5 µg/kg, 7.5 µg/kg or 15 µg/kg/ daily, by subcutaneous injection. A total of 26 children with achondroplasia with an average age of 7.8 years were enrolled in the first three cohorts. Based on the safety profile observed to date across the three dose cohorts, all subjects participating in the study have now been switched to the dose of 15 µg/kg/ daily for the duration of the 18-month extension study. Additional cohorts for higher doses have been added to the study.
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. 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 invasive surgeries such as spinal cord decompression and straightening of bowed legs.

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, per the World FactBook 2014 edition which translates into approximately 96,000 potential patients. Vosoritide is being tested in children whose growth plates are still "open," typically those under 18 years of age. This is approximately 25 percent of people affected with achondroplasia. In the United States, Europe, Latin American and the Middle East, there is currently no licensed medicines for achondroplasia.

About BioMarin
BioMarin is a global biotechnology company that develops and commercializes innovative therapies for patients with serious and life-threatening rare and ultra-rare genetic diseases. 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.

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 final results of the Phase 2 trial 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 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 2014 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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