BioMarin Doses First Patient in Phase 2 Trial With BMN 111 for the Treatment of Children With Achondroplasia

SAN RAFAEL, Calif., Jan. 14, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that it has dosed the first child in the Phase 2 trial with BMN 111, an analog of C-type Natriuretic Peptide (CNP), for the treatment of children with achondroplasia. Achondroplasia is the most common form of disproportionate short stature or dwarfism.

"BMN 111 is representative of BioMarin's core competency of developing life-altering therapies that address unmet medical needs," stated Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "In this Phase 2 study, we hope to see improvements in bone growth similar to what was observed in our preclinical models, and resulting improvements in the medical complications of achondroplasia that occur as a result of disproportionate bone growth. We believe treatment with BMN 111 for achondroplastic children will be well-tolerated and could potentially address the underlying cause of this condition and lead to benefits in the lives of these patients."

The Phase 2 study is an open-label, sequential cohort, dose-escalation study of BMN 111 in children who are 5-14 years old. The primary objective of this study is to assess the safety and tolerability of daily subcutaneous doses of BMN 111 administered for 6 months. The secondary objectives will include an evaluation of change in annualized growth velocity, changes in absolute growth parameters, changes in body proportions and other medically relevant and functional aspects of achondroplasia, such as sleep apnea and joint range of motion. Prior to enrolling in the Phase 2 study, all patients will have participated in a 6 month natural history study to determine baseline growth velocity data. This is an international study that will enroll approximately 24 subjects for a treatment duration of 6 months.

About Achondroplasia

Achondroplasia is the most common form of human dwarfism and is characterized by failure of normal conversion of cartilage into bone. It is caused by an autosomal dominant activating mutation in the fibroblast growth factor receptor 3 (FGFR3) gene, a negative regulator of bone growth. Eighty percent of cases are the result of a spontaneous mutation, and ninety-eight percent of those cases have a G380R mutation. Clinical manifestations of the disease include short stature, cervico-medullary compression, sleep apnea, bowed legs, frontal bossing and mid-face hypoplasia, permanent sway of the lower back, spinal stenosis, recurrent ear infections and obesity, all of which are related to the disproportionate growth which is characteristic of the condition.

The rate of incidence of achondroplasia is one in 15,000 to one in 40,000 live births, with approximately 18,000 to 24,000 people in the U.S. and Europe combined.

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises four approved products and multiple clinical and pre-clinical product candidates. Approved products include Naglazyme® (galsulfase) for MPS VI, a product wholly developed and commercialized by BioMarin; Aldurazyme® (laronidase) for MPS I, a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; Kuvan® (sapropterin dihydrochloride) Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany; and Firdapse® (amifampridine), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS). Product candidates include VIMIZIM™ (N-acetylgalactosamine 6-sulfatase), formally referred to as GALNS, which successfully completed Phase 3 clinical development for the treatment of MPS IVA, PEG PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase 3 clinical development for the treatment of PKU, BMN 673, a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase 3 clinical development for the treatment of germline BRCA breast cancer, BMN 701, a novel fusion of acid alpha glucosidase (GAA) with a peptide derived from insulin like growth factor 2, which is currently in Phase 1/2 clinical development for the treatment of Pompe disease, BMN 111, a modified C-natriuretic peptide, which is currently in Phase 1 clinical development for the treatment of achondroplasia and BMN 190, a recombinant human tripeptidyl peptidase-1 (rhTPP1) for the treatment of late-infantile neuronal ceroid lipofuscinosis (CLN2), a form of Batten Disease. 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 BioMarin's BMN 111 program generally and the timing and results of the planned Phase 2 trial of BMN 111. 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 BMN 111; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities; our ability to successfully manufacture the product candidate for the preclinical and clinical trials; 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 2012 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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