BioMarin Announces Phase 1 Results for BMN-111 for Achondroplasia

Phase 2 Trial Expected to Start in Mid-2013

NOVATO, Calif. , Sept. 26, 2012 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today the completion of a Phase 1 study for BMN-111, an analog of C-type Natriuretic Peptide (CNP), for achondroplasia. The company expects to initiate a Phase 2 trial in mid-2013.

The Phase 1 study was a two-part, double-blind, placebo-controlled study in healthy adult males. Part 1 examined a series of single subcutaneous doses and Part 2 included ten days of either fixed dosing or dose escalation. The primary objective of the study was to evaluate safety, tolerability and pharmacokinetics (PK) of single and multiple doses of BMN-111 in 48 healthy adult volunteers.

BMN-111 was generally well-tolerated. Mild, transient, self-limited hypotension was observed. The majority of these cases were asymptomatic, and only observed upon assumption of an upright posture following recumbence. No dose-limiting toxicities were identified outside of these cardiovascular findings. Systemic exposure to BMN-111 was similar at these doses to what has been observed to cause growth in healthy and disease model animals. All adverse events were of mild severity.

"We have identified a safe starting dose for treatment of achondroplastic children," stated Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "Further, we have identified the likely side effects of excessive exposure, at least in the short-term, and we do expect BMN-111 to be well-tolerated in children. We are presently evaluating options for further development in various forms of achondroplasia."

Based on the results of the Phase 1 study, the design of a Phase 2 proof-of-concept and dose finding study is underway. The goal of the program is to assess growth velocity as well as medical complications of achondroplasia, including non-growth endpoints. To better understand the nature of the disease, a study to collect consistent baseline growth measurements in children with achondroplasia was initiated in the second quarter of 2012. Participants in this study will be considered for enrollment in the Phase 2 study.

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.

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 patients 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 mucopolysaccharidosis VI (MPS VI), a product wholly developed and commercialized by BioMarin; Aldurazyme® (laronidase) for mucopolysaccharidosis I (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 GALNS (N-acetylgalactosamine 6-sulfatase), which is currently in Phase III clinical development for the treatment of MPS IVA, amifampridine phosphate (3,4-diaminopyridine phosphate), which is currently in Phase III clinical development for the treatment of LEMS in the U.S., PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase II clinical development for the treatment of PKU, BMN-701, a novel fusion protein of insulin-like growth factor 2 and acid alpha glucosidase (IGF2-GAA), which is currently in Phase I/II clinical development for the treatment of Pompe disease, BMN-673, a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase I/II clinical

development for the treatment of genetically-defined cancers, and BMN-111, a modified C-nutriuretic peptide, which is currently in Phase I clinical development for the treatment of achondroplasia. For additional information, please visit www.BMRN.com. Information on BioMarin's website is not incorporated by reference into this press release.

The BioMarin Pharmaceutical Inc. logo is available at http://www.globenewswire.com/newsroom/prs/?pkgid=11419

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 design of the planed 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 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; BioMarin's ability to secure clinical trial sites to perform the Phase 2 trial and the ability to enroll patients into those 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 Quarterly Report on Form 10-Q for the Quarter ended June 30, 2012. 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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