BioMarin Initiates Phase 1 Trial for BMN-111 for the Treatment of Achondroplasia

NOVATO, Calif., Feb. 16, 2012 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today the initiation of a Phase 1 study in healthy volunteers for BMN-111, an analog of C-type Natriuretic Peptide (CNP), for the treatment of achondroplasia. The company expects to report results from this trial in the third quarter of 2012.

"BMN-111 has demonstrated benefits in moderately and severely affected animal models and has been shown to have a wide therapeutic index based on new nonclinical data," stated Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "We aim to conduct a Phase 1 trial in healthy volunteers to inform the starting dose and regimen to be employed in a subsequent Phase 2 pediatric study scheduled to start in the fourth quarter of 2012 or first quarter of 2013. With seven programs in the clinic, the most ever in the history of the company, we look forward to many potentially value-creating clinical milestones this year."

The Phase 1 study is a two-part, double-blind, placebo-controlled study to evaluate the safety, tolerability and pharmacokinetics of single and multiple subcutaneous doses of BMN-111 administered in up to 74 healthy adult volunteers.

About Achondroplasia

Achondroplasia is the most common form of human dwarfism and is characterized by failure of normal conversion of cartilage into bone. Achondroplasia 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, craniomedullary compression, apnea, bowed legs, frontal bossing and midface 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.
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 timing and conduct of the Phase I clinical trial of BMN-111; and the timing of a possible Phase 2 clinical trial of BMN-111. These risks and uncertainties include, among others: enrollment in and results of the Phase 1 clinical trial of BMN-111; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities concerning BMN-111; 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 2010 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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