BioMarin Initiates Phase 3 Trial for PEG-PAL for the Treatment of PKU

SAN RAFAEL, Calif., June 5, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that it has initiated the Phase 3 program for PEG-PAL (PEGylated recombinant Phenylalanine Ammonia Lyase) for the treatment of phenylketonuria (PKU).

"In the Phase 2 trial, PEG-PAL was shown to dramatically reduce blood Phe levels, and we are hopeful that we will achieve the same outcome with the Phase 3 program," stated Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "Adult patients with PKU and patients on the severe end of the disease spectrum still represent a very high unmet medical need. With PEG-PAL, it may be possible to provide a treatment benefit to this population."

The Phase 3 study (165-301) is an open-label, multi-center study to assess the safety and tolerability of an induction, titration and maintenance dose regimen of PEG-PAL self-administered by approximately 90 naïve adult PKU subjects. The primary objective of the 165-301 study is to characterize the safety and tolerability of PEG-PAL during induction, titration, and maintenance dosing. The secondary objective of the study is to evaluate blood Phe levels during induction, titration, and maintenance dosing.

After completion of the open label 165-301 study, subjects are expected to enroll in 165-302, a Phase 3 double-blind, placebo-controlled, randomized discontinuation study to evaluate the efficacy and safety of PEG-PAL self-administered by adults with PKU. The study will also enroll approximately 60 subjects from the Phase 2 program who are currently being treated with PEG-PAL. The primary objective of the 165-302 study is to evaluate blood Phe levels. The secondary objective of this study is to evaluate changes in neuropsychiatric assessments as measured by the Inattentive portion of the Attention Deficit and Hyperactivity Disorder Rating Scale (ADHD-RS) and the Profile of Mood States (POMS). These will be administered at baseline, four and eight weeks.

About PKU

PKU, a genetic disorder affecting approximately 50,000 diagnosed patients in the developed world, is caused by a deficiency of the enzyme phenylalanine hydroxylase. PAH is required for the metabolism of phenylalanine, an essential amino acid found in most protein-containing foods. If the active enzyme is not present in sufficient quantities, Phe accumulates to abnormally high levels in the blood and becomes toxic to the brain, resulting in a variety of complications including severe mental retardation and brain damage, mental illness, seizures, tremors, and limited cognitive ability. As a result of newborn screening efforts implemented in the 1960s and early 1970s, virtually all PKU patients under the age of 40 in developed countries have been diagnosed at birth. Currently, PKU can only be managed by a Phe-restricted diet, which is supplemented by nutritional replacement products, like formulas and specially-manufactured foods; however, the strict diet is difficult for most patients to adhere to the extent needed for achieving adequate control of blood Phe levels. To learn more about PKU, please visit www.PKU.com. Information on this website is not incorporated by reference into this press release.

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 Vimizim (N-acetylgalactosamine 6-sulfatase), formally referred to as GALNS, which successfully completed Phase III clinical development for the treatment of MPS IVA, PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase III 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-natriuretic 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.

Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: the expectations related to the recently initiated Phase 3 trial for PEG-PAL, the expected future Phase 3 trial of PEG-PAL and the PEG-PAL development program generally. 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 PEG-PAL; our ability to successfully manufacture PEG-PAL; the content and timing of decisions by the U.S. Food and Drug Administration and other regulatory authorities concerning PEG-PAL; and those risks that are discussed in BioMarin's filings with the Securities and Exchange Commission, including, without limitation, BioMarin's 2012 Annual Report on Form 10-K, and our periodic reports on Form 10-Q and Form 8-K. 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.

CONTACT: Investors:

Eugenia Shen

BioMarin Pharmaceutical Inc.

(415) 506-6570

Media:

Debra Charlesworth

BioMarin Pharmaceutical Inc.

(415) 455-7451
