BioMarin Doses First Patient in Phase 1/2 Trial With BMN 190 for the Treatment of Neuronal Ceroid Lipofuscinosis Type 2, a Form of Batten Disease

SAN RAFAEL, Calif., Sept. 23, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that it has dosed the first patient in the Phase 1/2 trial for BMN 190, a recombinant human tripeptidyl peptidase 1 (rhTPP1) for the treatment of patients with neuronal ceroid lipofuscinosis type 2 (NCL-2), a form of Batten disease. This is the first time that a patient with Batten Disease has been treated with an enzyme replacement therapy in a clinical trial setting.

"This program is representative of the company's core competency of developing life-altering enzyme replacement therapies for serious unmet medical needs," said Hank Fuchs, M.D., Executive Vice President and Chief Medical Officer of BioMarin. "We are inspired and motivated by the patient and physician community and encouraged by the pharmacological activity demonstrated in preclinical models. We hope to leverage our expertise in enzyme replacement therapy development to deliver a viable treatment option to patients with this form of Batten Disease."

"The completion of dosing of the first patient in the trial of BMN190 marks the beginning of an important journey. This neurodegenerative disease of childhood is devastating for patients and families. While we hope to make a large difference in their outcome, we are moved and grateful for the support of affected families worldwide," said Angela Schulz, M.D. Ph.D., Children's Hospital, University Medical Center Hamburg-Eppendorf. "Clinical trials are the only means to ascertain whether the promise of this new therapeutic approach will be fulfilled. Without the families' selfless dedication to the important principles of science, this would not be possible."

"We are encouraged by this important milestone, and support the clinical trial process as the best way to bring much needed therapies to underserved patient populations," said Tracy VanHoutan, founder of the Noah's Hope Batten Disease research fund, and 2nd Vice President of the Batten Disease Support and Research Association, the largest organization in the world dedicated to family support and medical research in Batten Disease.

The Phase 1/2 study is an open-label, dose-escalation study in patients with NCL-2. The primary objectives are to evaluate the safety and tolerability of BMN 190 and to evaluate effectiveness using an NCL-2-specific rating scale score in comparison with natural history data after 48 weeks of treatment. Secondary objectives are to evaluate the impact of treatment on brain atrophy in comparison with NCL-2 natural history after 48 weeks of treatment and to characterize pharmacokinetics and immunogenicity. The study will enroll approximately 22 subjects at up to ten clinical sites for a treatment duration of 48 weeks.

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 BMN 110 (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, BMN 111, a modified C-natriuretic peptide, which is currently in Phase I clinical development for the treatment of achondroplasia, and BMN 190, a recombinant human tripeptidyl peptidase 1 (rhTPP1), which is currently in Phase 1/2 development for the treatment of neuronal ceroid lipofuscinosis Type 2 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 190 program generally and the timing and results of the planned Phase 1/2 trial of BMN 190. 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 190; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities; 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 Annual Report on Form 10-K for the Year ended December 31, 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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