BioMarin Submits CTA for BMN-190 for Batten Disease

SAN RAFAEL, Calif., April 1, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that it has submitted a Clinical Trial Application (CTA) with the Medicines and Healthcare Products Regulatory Agency (MHRA) in the U.K. 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. The company expects to start enrolling patients in a Phase 1/2 trial in mid-2013.

"The Batten disease program embodies our core mission at BioMarin -- to develop life-altering therapies for ultra-orphan diseases with a significant and serious unmet medical need," said Hank Fuchs, M.D., Executive Vice President and Chief Medical Officer of BioMarin. "We hope to leverage our expertise in enzyme replacement therapy development to advance this program and bring a therapeutic option to patients suffering from NCL-2. We have demonstrated encouraging pharmacological activity in preclinical models and hope to see this benefit translated into human subjects."

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. Secondary objectives are to evaluate the impact of treatment on brain atrophy in comparison with NCL-2 natural history, and to characterize the pharmacokinetics and immunogenicity. The study will enroll approximately 22 subjects worldwide 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 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, 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.

Aldurazyme® is a registered trademark of BioMarin/Genzyme LLC.

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