BioMarin Enrolls First Patient in Phase 1/2 Trial of NAGLU Fusion Protein BMN 250 for Treatment of MPS IIIB (Sanfilippo B Syndrome)

European Commission Grants BMN 250 Orphan Drug Designation to Treat Ultra-Rare Condition

SAN RAFAEL, Calif., April 21, 2016 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (NASDAQ:BMRN) announced today that it has enrolled the first patient in a Phase 1/2 trial for BMN 250, an investigational enzyme replacement therapy using a novel fusion of recombinant human alpha-N-acetyglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo B syndrome or mucopolysaccharidosis IIIB (MPS IIIB). Discovered by BioMarin, BMN 250 is being studied in a multicenter, international clinical trial evaluating safety and tolerability, as well as cognitive function of patients with MPS IIIB receiving BMN 250. Designed to restore functional NAGLU activity in the brain, BMN 250 is administered via intracerebroventricular (ICV) infusion.

"Sanfilippo B has long been a difficult condition to treat due to challenges in effectively delivering a therapy that bypasses the blood brain barrier to address the underlying cause of the disease. BMN 250 is designed to overcome these challenges by leveraging our proprietary technology to deliver enzyme replacement therapies directly to the brain," said Hank Fuchs, M.D., Chief Medical Officer at BioMarin. "BMN 250 is the result of building upon our almost two decades of experience in developing treatments for MPS and our most recent experience with an intracerebroventricular delivery approach, which we have used with another experimental enzyme replacement therapy delivered directly to the brain to treat a form of Batten disease."

The company also announced that BMN 250 has been granted orphan drug designation by the European Commission. In late 2014, BMN 250 also received orphan drug designation from the U.S. Food and Drug Administration. BioMarin has three approved therapies to treat different forms of MPS. BMN 250 is a potential fourth therapy in development for the treatment of an MPS disorder.

"Children with Sanfilippo syndrome need therapies that target the root cause of this fatal genetic disease that currently has no approved treatment options," said Jill Wood, Co-Founder and Treasurer, Jonah's Just Begun Foundation to Cure Sanfilippo. "We applaud companies like BioMarin who are bringing scientific research into the clinic. This is a critical first step to finding potential treatments."

Study Design
The BMN 250 development program consists of two independent and complementary, multicenter, international studies. BMN 250-901 is an observational study of the progression of MPS IIIB over time in children. BMN 250-201 is a Phase 1/2 treatment study conducted in two parts, with Part 1 focused on safety and dose escalation. Part 2 consists of eligible patients rolling over from the BMN 250-901 observational study in addition to continued treatment of the patients from Part 1 of the study. Efficacy will be assessed by comparing changes in disease progression in the observational BMN 250-901 study vs. changes observed in Part 2 of the BMN 250-201 Phase 1/2 treatment study.

About Sanfilippo B Syndrome
Mucopolysaccharidosis IIIB (MPS IIIB) or Sanfilippo B syndrome a lysosomal storage disease is caused by deficiency in the enzyme alpha-N-acetyglucosaminidase (NAGLU), one of the four enzymes required for heparan sulfate (HS) degradation. There are an estimated 2,000-3,000 patients in existing BioMarin territories who are living with Sanfilippo B syndrome.

The first symptoms generally appear between the ages of two and six years old, with behavior disorders, intellectual deterioration, sleep disorders, and in some cases, very mild dysmorphism. The neurological involvement becomes more prominent with progressive loss of motor milestones and communication problems. The prognosis is poor with death occurring in most cases in the late teens or early 20s.

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare disorders. The company's portfolio consists of five commercialized products and multiple clinical and pre-clinical product candidates.

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 plans for BMN 250 and expectations regarding the clinical trials for this product candidate. 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: the results and timing of current and planned preclinical and clinical studies; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities; our ability to successfully manufacture the product candidate for the preclinical and clinical 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 2015 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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