BioMarin Announces Selection of NAGLU Fusion Protein Drug Development Candidate BMN 250 for the Treatment of Sanfilippo B (MPS IIIB)

Potential to Add Fourth MPS Treatment to BioMarin Franchise
Two presentations at Lysosomal Disease Network's 10th Annual WORLDSymposium™

SAN RAFAEL, Calif., Feb. 11, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that it has selected a new drug development candidate, BMN 250, a novel fusion of alpha-N-acetyglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo B syndrome or Mucopolysaccharidosis type IIIB (MPS IIIB). BioMarin has initiated IND-enabling studies and expects to initiate clinical studies with BMN 250 in mid-2015.

Discovered by BioMarin, BMN 250 is an enzyme replacement therapy using recombinant human NAGLU with an IGF2, or Glycosylation Independent Lysosomal Targeting (GILT) tag. BMRN 250 is delivered directly to the brain using BioMarin's patented technology. BioMarin has issued patents which broadly cover delivery of lysosomal enzymes directly into the cerebrospinal fluid to treat lysosomal storage diseases.

"We are pleased to add an exciting new candidate to our pipeline that could be a potentially first-in-class therapy for Sanfilippo B patients who currently have no drug treatment options available," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "Developing BMN 250 for Sanfilippo B or MPS IIIB brings together the best of BioMarin's scientific and clinical expertise. We are building upon a deep knowledge of MPS diseases, and experience developing fusion proteins and enzyme replacement therapies overall. Adding a fourth treatment for MPS complements our current franchise of two approved therapies for the treatment of MPS I and MPS VI and a third expected for MPS IVA."

Data on the NAGLU fusion protein will be presented at the Lysosomal Disease Network's (LDN) 10th Annual WORLDSymposium™ being held February 11-13 in San Diego, California.

"We are encouraged by the results in the preclinical data where we have seen excellent cellular uptake of the enzyme throughout the brain," said Elizabeth Neufeld, Ph.D., Emerita Member, Brain Research Institute and Professor Emerita, Biological Chemistry, David Geffen School of Medicine at the University of California Los Angeles. "The animal studies show intracellular storage is cleared with NAGLU-IGF2 treatment which we hope will translate well in the clinic."

NAGLU Fusion Protein Presentations at LDN's 10th Annual WORLDSymposium

1. Poster presentation by BioMarin on February 11 and 12 at 4:30 - 6:30 p.m PT

Engineering of a recombinant NAGLU fusion protein with insulin-like growth factor 2 leads to improved cellular uptake via a glycosylation-independent lysosomal targeting pathway

Mika Aoyagi-Scharber, et al. BioMarin Pharmaceutical Inc.; Department of Pediatrics, Los Angeles Biomedical Research Institute at Harbor-UCLA Medical Center.

2. Oral presentation by Academic Collaborators on February 12 at 2:30 p.m PT

Intracerebroventricular enzyme replacement therapy with glycosylation-independent lysosomal targeted NAGLU leads to widespread enzymatic activity, reduction of lysosomal storage and of secondary defects in brain of Sanfilippo B mice

Shih-hsin Kan, et al. Department of Pediatrics, Los Angeles Biomedical Research Institute at Harbor-UCLA Medical Center; BioMarin Pharmaceutical Inc.; Department of Biological Chemistry, University of California Los Angeles.

About Sanfilippo B syndrome

Mucopolysaccharidosis type IIIB (MPS IIIB) or Sanfilippo B syndrome is a lysosomal storage disease belonging to the group of mucopolysaccharidosis and characterized by severe and rapid intellectual deterioration. MPS IIIB is caused by deficiency in the enzyme alpha-N-acetylglucosaminidase (NAGLU), one of the four enzymes required for heparan sulfate (HS) degradation. There are an estimated 1,000 - 2,000 patients in the developed world with Sanfilippo B syndrome. The first symptoms appear between the ages of two and six years old, with

behavior disorders, intellectual deterioration, sleep disorders and very mild dysmorphism. The neurological involvement becomes more prominent around the age of ten with loss of motor milestones and communication problems. Seizures often occur after the age of ten. The prognosis is poor with death occurring in most cases of type IIIB between 30-40 years of age.

Source: Orphanet

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 MPS VI, a product wholly developed and commercialized by BioMarin; Aldurazyme® (laronidase) for 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 3 clinical development for the treatment of MPS IVA, PEG PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase 3 clinical development for the treatment of PKU, BMN 673, a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase 3 clinical development for the treatment of germline BRCA breast cancer, BMN 111, a modified C-natriuretic peptide, which is currently in Phase 2 clinical development for the treatment of achondroplasia, BMN 701, a novel fusion of acid alpha glucosidase (GAA) with a peptide derived from insulin like growth factor 2, which is currently in Phase 1/2 clinical development for the treatment of Pompe disease, BMN 190, a recombinant human tripeptidyl peptidase-1 (rhTPP1) for the treatment of late-infantile neuronal ceroid lipofuscinosis (CLN2), a form of Batten Disease, which is currently in Phase 1, BMN 270, an AAV-factor VIII vector, for the treatment of hemophilia A and BMN 250, a novel fusion of alpha-N-acetyglucosaminidase(NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of MPS IIIB.

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 BMN 250, including the expected timing of the pre-clinical trials and initiation of clinical trials of the 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 of current and ongoing preclinical trials, particularly the IND-enabling toxicology; 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 2012 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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