# FDA Grants BioMarin Orphan Drug Designation for NAGLU Fusion Protein, BMN 250, for the Treatment of MPS IIIB (Sanfilippo Syndrome Type B)

**Clinical Studies Expected to Begin in Mid-2015**

SAN RAFAEL, Calif., Dec. 10, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that the Food and Drug Administration (FDA) has granted orphan drug designation for 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 Syndrome Type B or Mucopolysaccharidosis IIIB (MPS IIIB). BioMarin 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 pioneered a proprietary approach to deliver large proteins directly to the brain, which bypasses the blood brain barrier and has typically proved difficult. This BioMarin technology to be used for the treatment of Sanfilippo patients builds on the experience we have gained using this approach to treat CLN2 disorder, a form of Batten disease," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "We look forward to meeting with the FDA to determine the regulatory path for BMN 250 for MPS IIIB or Sanfilippo Type B and applying our experience in developing three first-in-class therapies for different varieties of MPS diseases."

"We are pleased that BioMarin is focusing on developing a therapy for Sanfilippo B patients," said Kathleen Buckley, President of the Team Sanfilippo Foundation. "Children affected by MPS IIIB or Sanfilippo B have no approved drug treatment options, and the families affected by this terminal disease are hopeful that BioMarin will quickly advance its experimental therapy, which has the possibility of making a difference."

"BioMarin has been committed to developing therapies for many MPS disorders. We are encouraged that this biotechnology company is now turning its focus to MPS IIIB or Sanfilippo B Syndrome," said Barbara Wedehase, MSW, CGC, Executive Director of the National MPS Society. "BioMarin has expertise in enzyme replacement therapies and MPS disorders, and we appreciate their continued efforts to deliver a therapy for this unmet medical need."

**About Sanfilippo B syndrome**

Mucopolysaccharidosis IIIB (MPS IIIB) or Sanfilippo Syndrome Type B is a lysosomal storage disease belonging to the group of mucopolysaccharidosis. MPS IIIB 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 1,000 - 2,000 patients in the developed world with Sanfilippo Syndrome Type B. MPS IIIB is unique among the MPS disorders as it is predominantly a neurological disease. The first symptoms 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 of type IIIB in the late teens or early 20s.

*Source:  Orphanet and American Journal of Genetics*

**About BioMarin**

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises five approved products and multiple clinical and pre-clinical product candidates. Approved products include VIMIZIM® (elosulfase alfa) for MPS IVA, a product wholly developed and commercialized by BioMarin; 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) Powder for Oral Solution and 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 165 (PEGylated recombinant phenylalanine ammonia lyase), also referred to as PEG PAL, which is currently in Phase 3 clinical development for the treatment of PKU, talazoparib (formerly referred to as 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 701, a novel fusion protein of insulin-like growth factor 2 and acid alpha glucosidase (IGF2-GAA), which is currently in Phase 3 clinical development for the treatment of Pompe disease, BMN 111, a modified C-natriuretic peptide, which is currently in Phase 2 clinical development for the treatment of achondroplasia, BMN 190, a recombinant human tripeptidyl peptidase-1 (rhTPP1) for the treatment of CLN2 disorder, 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 the development plans for BMN 250 and 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 2013 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.



CONTACT: Investors:

Traci McCarty

BioMarin Pharmaceutical Inc.

(415) 455-7558

Media:

Debra Charlesworth

BioMarin Pharmaceutical Inc.

(415) 455-7451

---