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BioMarin Doses First Patient in Phase 3 INSPIRE Trial With BMN 701 for the Treatment of Pompe Disease

Proprietary Glycosylation Independent Lysosomal Targeting (GILT) Tagging Technology Has Been Shown to Improve Respiratory Muscle Strength in Patients With Rare Genetic Disorder

SAN RAFAEL, Calif., May 27, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that it has dosed the first patient with BMN 701 (GILT-tagged Recombinant Human GAA) in the Phase 3 INSPIRE trial for Pompe disease. BMN 701 is a novel fusion protein of insulin-like growth factor 2 and acid alpha glucosidase (IGF2-GAA), designed to target delivery to the lysosomes where the enzyme is most needed. The INSPIRE trial focuses on late onset Pompe disease - an inherited condition caused by a deficiency in the lysosomal enzyme acid alpha glucosidase, which can lead to progressive weakening of the muscles of the body, including the diaphragm, a crucial respiratory muscle. Respiratory impairment is the leading cause of morbidity and mortality in late onset Pompe disease.

"Most patients with Pompe disease suffer breathing problems related to respiratory weakness, and many will eventually require respiratory support, such as a ventilator," stated Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "In the Phase 3 INSPIRE trial, we hope to see improvements in respiratory muscle weakness, which is one of the hallmarks of Pompe disease. We hope that treatment with BMN 701 will be well-tolerated and that it could benefit patients by potentially helping them breathe easier and reducing the need for mechanical breathing assistance and increasing endurance."

Research has demonstrated that over time, Pompe patients experience a progressive decrease in clinical measures of respiratory function, maximal inspiratory pressure (MIP) and maximal expiratory pressure (MEP). The BMN 701 Phase 3 INSPIRE trial is a single-arm, open-label, switchover study designed to assess if patients who have been taking rhGAA (recombinant human acid alglucosidase alfa) can realize further improvement in measures of respiratory muscle strength and endurance, and to better understand the safety effects when switching therapies from rhGAA to BMN 701.

"We have a strong track record of quickly developing enzyme replacement therapies for unmet medical needs. The development of BMN 701 is another opportunity for us to leverage our clinical and regulatory experience and manufacturing know-how," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "There is a significant amount of interest in the medical and Pompe patient communities for more treatment options for late-onset Pompe disease. We believe, based on our studies to date, that BMN 701 has the potential to deliver more of the enzyme to muscle lysosomes compared to the currently available option."

"Research and treatment options for Pompe disease have come a long way in the last eighteen years," said Tiffany House, President of the Acid Maltase Deficiency Association (AMDA), an organization founded in 1995 that is dedicated to research, patient support and raising awareness of the disorder. "We welcome BioMarin into the Pompe research field and applaud their commitment to the Pompe community. The Pompe community is looking forward to the results of the upcoming Phase III program with BMN 701."

The safety and efficacy of BMN 701 was initially assessed in a 24-week study of 22 treatment naïve, late-onset Pompe patients 13 years of age and older. The Phase 1/2 trial was an open-label study to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamic and clinical activity of BMN 701 administered as an intravenous infusion every two weeks at doses of 5 mg/kg, 10 mg/kg and 20 mg/kg. In the trial, BMN 701 demonstrated improvements in clinical measures of respiratory muscle strength and endurance. 15 out of 16 patients demonstrated an improvement in MIP on a 20 mg/kg infusion regimen over 24 weeks; a 27.0 percent relative mean improvement from pre-treatment baseline to week 24 was observed. Three out of 16 patients on a 20 mg/kg infusion regimen, or 18.8%, demonstrated a > 75 m increase in the 6 minute walk test (6MWT) over baseline.

About Pompe Disease

Pompe disease is a lysosomal storage disorder where glycogen accumulates in muscles due to deficiency in the enzyme acid alpha glucosidase (GAA). There are two main forms of Pompe disease: infantile onset, characterized by cardiomyopathy, and late onset, which can, in fact, occur at any age. Patients with late onset Pompe disease suffer from muscle weakness and respiratory difficulties. Typically, late onset Pompe disease progresses slowly over many years, frequently resulting in respiratory failure.

In the general population, Pompe disease occurs at a rate of about one in 40,000 people. The late onset form of the disease occurs in about one in 57,000 births, and the infantile onset form occurs in about one in 140,000 births. Pompe disease affects males and females equally, and in most cases, both parents of an affected child are asymptomatic carriers of the disease.

About BMN 701

BMN 701 is a GILT-tagged Recombinant Human GAA, a novel fusion protein of insulin-like growth factor 2 and acid alpha glucosidase (IGF2-GAA), for the treatment of acid maltase deficiency or Pompe disease. BMN 701 works differently than current Pompe treatments. BMN 701 has a small protein attached to it (IGF-2) which allows it to attach to the surface of the muscle cell more tightly than the normal enzyme that is absent or low in Pompe patients, acid alpha glucosidase. Animal research has shown that BMN 701 is able to get into the cell and clear much of the excess glycogen buildup that creates the problems in Pompe disease.

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; Naglazyme® (galsulfase) for MPS VI; Aldurazyme® (laronidase) for MPS I, a product which BioMarin developed through a 50/50 joint venture with Genzyme, a Sanofi Company; Kuvan® (sapropterin dihydrochloride) Tablets or Powder for Oral Solution, 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 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 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 1 clinical development for the treatment of achondroplasia, 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 the development of BioMarin's BMN 701 program generally and the timing and results of the planned Phase 3 INSPIRE trial of BMN 701. 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 701; 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.

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