FDA Accepts Vimizim BLA and Grants Priority Review Designation

PDUFA Action Date Extended to February 28, 2014

SAN RAFAEL, Calif., May 30, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that the U.S. Food and Drug Administration (FDA) has accepted for review the Biologics License Application (BLA) for Vimizim (BMN-110, elosulfase alfa), an enzyme replacement therapy under evaluation for the treatment of patients with the rare lysosomal storage disorder Mucopolysaccharidosis Type IVA (MPS IVA), also called Morquio A Syndrome.

The FDA has granted priority review designation to Vimizim, which is granted to drugs that offer major advances in treatment, or provide a treatment where no adequate therapy exists. During the initial review of the application, the FDA requested additional Chemistry, Manufacturing and Controls (CMC) information. The company provided the information as requested, and the FDA designated it as a major amendment to the application thus extending the PDUFA action date by three months. The extended PDUFA action date is February 28, 2014.

In the FDA's filing communication, the Agency informed the company that it is currently planning to hold an advisory committee meeting to discuss the application. No date has been set for this meeting.

"We are pleased that the FDA has granted priority review to the Vimizim BLA, and we look forward to a productive dialog with the FDA as they review the application," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "With our expertise in developing enzyme replacement therapies to treat serious unmet medical needs, we plan to work closely with the FDA and other regulatory authorities to bring this much needed therapy to MPS IVA patients worldwide."

About MPS IVA

Mucopolysaccharidosis IVA (MPS IVA, also known as Morquio A Syndrome) is a disease characterized by deficient activity of N-acetylgalactosamine-6-sulfatase (GALNS) causing excessive lysosomal storage of glycosaminoglycans such as keratan sulfate and chondroitin sulfate. This excessive storage causes a systemic skeletal dysplasia, short stature, and joint abnormalities, which limit mobility and endurance. Malformation of the chest impairs respiratory function, and looseness of joints in the neck cause spinal instability and potentially spinal cord compression. Other symptoms may include hearing loss, corneal clouding, and heart disease. Initial symptoms often become evident in the first five years of life. The disease substantially limits both the quality and length of life of those affected.

The rate of incidence of MPS IVA is as yet unconfirmed and varies among different populations but estimates vary between 1 in 200,000 live births and 1 in 250,000 live births. The estimated prevalence is approximately 3,000 patients in the developed world. Based on knowledge of the worldwide distribution of the MPS VI market and the more than 1,300 identified MPS IVA patients worldwide, the company estimates that approximately 20 percent of patients are in North America (15 percent in the U.S.) and approximately 50 percent of patients are in EUMEA.

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 (elosulfase alfa), 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, and BMN-111, a modified C-natriuretic peptide, which is currently in

Phase I clinical development for the treatment of achondroplasia. 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: expectations regarding the regulatory process for the BLA filing for Vimizim with the FDA; the potential outcome of the review of such filing; and the possible approval of such 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: results and timing of current and planned clinical trials of its product candidates; the nature of the FDA's questions associated with the BLA and BioMarin's ability to timely respond to those questions; the FDA's compliance with it internal review guidelines; the outcome of the advisory committee meeting related to the BLA; the content and timing of decisions by the U.S. Food and Drug Administration; 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, as amended, and the factors contained in BioMarin's reports on Form 8-K. 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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