Vimizim MAA Validated by the EMA

Vimizim Designated for Accelerated Assessment by the EMA

SAN RAFAEL, Calif., May 30, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that the European Medicines Agency (EMA) has validated the Marketing Authorization Application (MAA) for Vimizim. Validation of the MAA confirms that the submission is complete and starts the EMA's formal review process. Earlier this year, the EMA accepted BioMarin's request for accelerated assessment for this MAA on the grounds that Vimizim could satisfy an unmet medical need and is of major interest from the point of view of therapeutic innovation and public health. Accelerated assessment has the potential to shorten EMA's review procedure. However, at any time during the MAA assessment, the EMA may decide to continue the assessment under standard assessment timelines. Assuming the Vimizim application remains on the accelerated assessment timeline, a CHMP opinion is anticipated in December 2013, and, if positive, a decision from the European Commission could be received in the first quarter of 2014.

"With approximately half of all MPS IVA patients living in Europe, the Middle East and Africa, this is an important milestone in our efforts to bring the first therapeutic option to patients with Morquio A Syndrome," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "Along with the acceptance of the BLA by the FDA, we are pleased to enter the review phase on a global basis. MPS IVA represents a significant unmet medical need for those affected, and our hope is to offer a life-altering treatment option to 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 MAA for

Vimizim with the EMA; 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 EMA's questions associated with the MAA and BioMarin's ability to timely respond to those questions; BioMarin's ability to continue to meet the requirements for accelerated assessment; the EMA's compliance with it internal review guidelines; the content and timing of decisions by the EMA; 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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