FDA Advisory Committee Recommends Approval for BioMarin's Vimizim(TM) for the Treatment of Patients With Morquio A Syndrome

SAN RAFAEL, Calif., Nov. 19, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that the Endocrinologic and Metabolic Drugs Advisory Committee (EMDAC) of the U.S. Food and Drug Administration (FDA) voted in favor of approval of Vimizim for the treatment of Morquio A syndrome, also called Mucopolysaccharidosis Type IVA (MPS IVA). Of the 21 panel members, 19 voted in favor of approval of Vimizim for use in all MPS IVA patients, 1 voted in favor of approval for a subgroup of MPS IVA patients, and one panel member voted to not recommend approval.

Vimizim is an investigational enzyme replacement therapy for the treatment of patients with the lysosomal storage disorder Morquio A syndrome. Morquio A syndrome is an ultra-rare, severely debilitating disease that affects an estimated 3,000 patients in the developed world.

"We are thrilled to have achieved this important milestone in our mission to bring the first approved therapy to treat Morquio A patients," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "I am very pleased with the outcome of today's panel vote and look forward to continuing to work with the FDA to bring this much-needed therapy to these patients."

The FDA has assigned a Prescription Drug User Fee Act (PDUFA) action date of February 28, 2014, for completion of its review of the Biologics License Application (BLA) for Vimizim. The EMDAC provides the FDA with independent expert advice and recommendations. The FDA is not bound by the EMDAC's recommendation, but will consider the committee's recommendation as the FDA completes its review of the Vimizim BLA.

About VIMIZIM

VIMIZIM (elosufase alfa) is an investigational treatment for patients with Morquio A syndrome, or mucopolysaccharidosis IVA (MPS IVA), which currently is under review by the FDA. VIMIZIM is the first and only enzyme replacement therapy (ERT) designed to target the underlying cause of Morquio A Syndrome — a deficiency in the enzyme N-acetylgalactosamine-6 sulfatase (GALNS). Infused enzyme replacement therapy with VIMIZIM replaces deficient GALNS activity to minimize progressive multi-systemic manifestations. Morquio A syndrome is a rare, severely debilitating and progressive disease that currently has no standard accepted treatment other than supportive care.

About Morquio A Syndrome

Morquio A syndrome, or mucopolysaccharidosis IVA (MPS IVA) is a disease in which people are missing an enzyme that is essential in the breakdown and removal of the glycosaminoglycans (GAGs) called keratan sulfate (KS) and chondroitin-6-sulfate (C6S). The incompletely broken down GAGs remain stored in cells in the body causing progressive damage. This excessive storage causes 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 Morquio A syndrome is as yet unconfirmed and varies among different populations, and estimates vary between 1 in 200,000 live births and 1 in 450,000 live births. The estimated prevalence is approximately 3,000 patients in the developed world.

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 VIMIZIM (N-acetylgalactosamine 6-sulfatase),

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 III clinical development for the treatment of PKU, BMN 673, a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase III 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 I/II clinical development for the treatment of Pompe disease, BMN-111, a modified C-natriuretic peptide, which is currently in Phase I clinical development for the treatment of achondroplasia and BMN 190, a recombinant human tripeptidyl peptidase-1 (rhTPP1) for the treatment of late-infantile neuronal ceroid lipofuscinosis (CLN2), a form of Batten Disease. 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 future regulatory actions by the FDA, including the PDUFA date for Vimizim; 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 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.

CONTACT: Investors

Traci McCarty

BioMarin Pharmaceutical Inc.

(415) 455-7558

Media

Debra Charlesworth

BioMarin Pharmaceutical Inc.

(415) 455-7451
