BioMarin Receives Positive Opinion From the CHMP in the European Union for VIMIZIM(TM) (elosulfase alfa) for Morquio A Syndrome

SAN RAFAEL, Calif., Feb. 20, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion for the company's Marketing Authorization Application (MAA) for VIMIZIM™ (elosulfase alfa) for the treatment of Morquio A syndrome, also called Mucopolysaccharidosis Type IVA (MPS IVA). The CHMP's recommendation is now referred to the European Commission (EC). If approved by the EC, BioMarin would receive marketing authorization for VIMIZIM in all EU Member States. The EC is expected to render a final decision for VIMIZIM in the second quarter of 2014.

"This positive CHMP opinion is an important milestone in our mission to provide the first approved therapy to treat Morquio A patients across Europe. We will leverage our existing European infrastructure to ensure that these patients gain access to VIMIZIM as quickly as possible," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "We are grateful for the continuous support we have received from the Morquio A community, and in particular, those patients who participated in the clinical development of VIMIZIM and their families."

"As a treating physician, the CHMP opinion is meaningful news for Morquio A patients who currently have no specific drug treatment option beyond supportive care," said Christian J. Hendriksz, Salford Royal NHS Foundation Trust. "VIMIZIM improved endurance in clinical trials, which may change the course of this devastating disease."

The U.S. Food and Drug Administration (FDA) approved VIMIZIM for patients with Mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome) on February 14, 2014.

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

The disease occurs as a result of a deficiency of activity in an enzyme involved in glycosaminoglycan (GAG) metabolism. The pervasive and progressive accumulation of GAGs leads to significant morbidities and multisystemic clinical impairments resulting in diminished functional capacity, impaired quality of life, and early mortality. The most common features of the disease are progressive skeletal dysplasia, the need for frequent surgical procedures related primarily to musculoskeletal or respiratory dysfunction, and significant limitations in mobility, endurance, and breathing.

The CHMP is a scientific committee composed of representatives from the 28-member states of the EU, and Iceland and Norway. The committee reviews medical product applications on their scientific and clinical merit and provides advice to the EC, which has the authority to approve medicines for the EU. The EU, which generally follows the recommendation of the CHMP, is expected to make its final decision in about 60 days.

About VIMIZIM™

VIMIZIM (elosulfase alfa) is a treatment for patients with Morquio A syndrome, or mucopolysaccharidosis IVA (MPS IVA). VIMIZIM is the first enzyme replacement therapy (ERT) designed to target the underlying cause of Morquio A Syndrome - a deficiency in the enzyme N-acetylgalactosamine-6 sulfatase (GALNS). VIMIZIM is intended to provide the exogenous enzyme GALNS that will be taken up into the lysosomes and increase the catabolism of GAGs. Morquio A syndrome is a rare, severely debilitating and progressive disease that previously had 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.

Important Safety Information

Life-threatening allergic reactions, known as anaphylaxis, can occur during VIMIZIM™ (elosulfase alfa) infusions. Due to the potential for anaphylaxis, appropriate medical support should be readily available when VIMIZIM is administered and for an appropriate period of time following administration.

Hypersensitivity reactions have been observed as early as 30 minutes after the start of infusion but as late as six days after infusion. Frequent symptoms of hypersensitivity reactions included anaphylactic reactions, urticaria, peripheral edema, cough, dyspnea, and flushing.

Because of the potential for hypersensitivity reactions, administer antihistamines with or without antipyretics prior to infusion. If severe hypersensitivity reactions occur, immediately stop the infusion of VIMIZIM and initiate appropriate treatment. Patients with acute febrile or respiratory illness at the time of VIMIZIM infusion may be at higher risk of life-threatening complications from hypersensitivity reactions.

Sleep apnea is common in MPS IVA patients. Evaluation of airway patency should be considered prior to initiation of treatment with VIMIZIM. Patients using supplemental oxygen or continuous positive airway pressure (CPAP) during sleep should have these treatments readily available during infusion in the event of an acute reaction, or extreme drowsiness/sleep induced by antihistamine use.

Spinal or cervical cord compression (SCC) is a known and serious complication of MPS IVA and may occur as part of the natural history of the disease. In clinical trials, SCC was observed both in patients receiving VIMIZIM and patients receiving placebo. Patients with MPS IVA should be monitored for signs and symptoms of SCC (including back pain, paralysis of limbs below the level of compression, urinary and fecal incontinence) and given appropriate clinical care.

All patients treated with VIMIZIM 2 mg/kg once per week in the placebo-controlled trial developed anti-drug antibodies.

VIMIZIM should be used during pregnancy only if the potential benefit justifies the potential risk to the fetus. It is not known if VIMIZIM is present in human milk.

Safety and effectiveness in pediatric patients below 5 years of age have not been established.

In clinical trials, the most common adverse reactions (≥10%) occurring during infusion included pyrexia, vomiting, headache, nausea, abdominal pain, chills, and fatigue. The acute reactions requiring intervention were managed by either temporarily interrupting or discontinuing infusion, and administering additional antihistamine, antipyretics, or corticosteroids.

Please see full Prescribing Information, including boxed warning, or visit www.VIMIZIM.com.

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, 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 1/2 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: expectations regarding the approval of VIMIZIM by the FDA and the EMA, BioMarin's ability to support the launch of a new product and ship to specialty pharmacies, the company's ability to conduct additional clinical trials. 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 and timing of actions by the FDA and the EMA, results and timing of current and planned clinical trials of its products; the timing of pricing approval in several countries in Europe; the availability and launch of VIMIZIM in Europe and the market potential for VIMIZIM as a treatment for Morquio A, 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, 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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