BioMarin Announces Approval of VIMIZIM(R) (elosulfase alfa) in Japan for Treatment of Morquio A Syndrome

First and Only Treatment in Japan Approved for Patients With This Ultra-Rare Genetic Condition

SAN RAFAEL, Calif., Dec. 29, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced the Ministry of Health, Labor and Welfare granted approval of the registration of VIMIZIM® (elosulfase alfa) for the treatment of patients with mucopolysaccharidosis type IVA (MPS IVA), also known as Morquio A syndrome. VIMIZIM is the first treatment in Japan approved for this condition. VIMIZIM was reviewed under the Orphan Drug program.

Professor Torayuki Okuyama from the National Center for Child Health and Development, who is currently using VIMIZIM on patients in a clinical trial said, "Morquio A syndrome is an ultra-rare and difficult condition to treat. VIMIZIM is the only specific treatment available and offers improved endurance to these patients."

"We are pleased to be able to deliver the first drug therapy for Morquio A to patients in Japan. VIMIZIM will be the first product approval that BioMarin will market in Japan," said Jean-Jacques Bienaimé, CEO of BioMarin. "Now that BioMarin has established its presence in Japan, we look forward to delivering future therapies to treat patients who have rare genetic diseases with unmet medical needs."

Morquio A syndrome is an ultra-rare, severely debilitating disease affecting an estimated 3,000 patients in the developed world. 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 U.S. Food and Drug Administration (FDA) approved the VIMIZIM license application for the treatment of patients with Morquio A syndrome on February 14, 2014. The therapy is also approved in Australia, Canada, Brazil and the European Union. Marketing applications have been submitted in several other countries.

"The approval of VIMIZIM in Japan underscores our commitment to providing this much needed therapy to patients with Morquio A syndrome across the globe," said Hank Fuchs, M.D., chief medical officer of BioMarin. "We will continue to seek approvals in other countries so that more patients within the MPS community have access to the treatments they deserve."

About VIMIZIM

VIMIZIM® (elosulfase alfa) is a treatment for patients with Morquio A syndrome, or mucopolysaccharidosis IVA (MPS IVA). VIMIZIM is the first approved 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 approved, standard-of-care treatment other than supportive care.

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 from 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 Morquio A Syndrome

Morquio A syndrome, or Mucopolysaccharidosis IVA (MPS IVA) is a disease in which people are missing an enzyme 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, limiting mobility and endurance. Malformation of the chest impairs respiratory function, and looseness of joints in the neck causing 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.

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, a product wholly developed and commercialized by BioMarin; Naglazyme® (galsulfase) for MPS VI, a product wholly developed and commercialized by BioMarin; Aldurazyme® (laronidase) for MPS I, a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; KUVAN® (sapropterin dihydrochloride) Powder for Oral Solution and 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 165 (PEGylated recombinant phenylalanine ammonia lyase), also referred to as PEG PAL, which is currently in Phase 3 clinical development for the treatment of PKU, talazoparib (formerly referred to as 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 2 clinical development for the treatment of achondroplasia, BMN 190, a recombinant human tripeptidyl peptidase-1 (rhTPP1) for the treatment of CLN2 disorder, 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 marketing application filing for VIMIZIM with the Pharmaceuticals and Medical Devices Agency; and the marketing and commercialization of VIMIZIM in Japan. 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; any further actions by the Pharmaceuticals and Medical Devices Agency; the outcome of pricing and reimbursement negotiations with relevant authorities in Japan; 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, 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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