BioMarin Announces European Commission Approval for VIMIZIM(R) (elosulfase alfa) for the Treatment of Morquio A Syndrome in Patients of All Ages

VIMIZIM is the First and Only Specific Treatment for Patients With This Ultra-Rare Genetic Condition

SAN RAFAEL, Calif., April 28, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced the European Commission has granted marketing authorization for VIMIZIM® (elosulfase alfa), the first specific treatment approved in the European Union for Mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome) in patients of all ages. As the first drug ever approved for Morquio A syndrome, VIMIZIM has been granted orphan drug status in the European Union, which confers ten years of market exclusivity.

"This approval of VIMIZIM in Europe is a key milestone because we estimate that 85% of Morquio A or MPS IVA patients live outside of the United States. BioMarin is a leader in advancing therapies to treat MPS diseases with three therapies to treat three different MPS diseases. We continue to build on our extensive scientific and clinical knowledge of lysosomal storage disorders to develop therapies for other rare genetic diseases," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "We appreciate the support from our employees, physicians and patients to develop the first approved drug therapy specifically for patients with Morquio A syndrome."

"VIMIZIM addresses the condition at the cellular level, fulfilling a large unmet medical need and represents an advance for Morquio A patients and their families," said Christian J. Hendriksz, Salford Royal NHS Foundation Trust and lead investigator for the Phase 3 clinical trial. "As a treating physician, I am encouraged that the therapy shifts the treatment beyond supportive care to treating the underlying cause of the disease, potentially changing the course of this devastating disease."

"This is a momentous occasion for patients with Morquio A disease in Europe because this is the first step to gain access to a potentially life-changing therapy. We appreciate BioMarin's commitment to the MPS community and its determination to develop targeted therapies to treat the many forms of MPS," said Christine Lavery, Chief Executive of the MPS Society, (United Kingdom). "For patients with Morquio A disease now having a specific drug treatment option provides real hope after decades of sadness and loss for so many families."

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. BioMarin has also submitted marketing applications for VIMIZIM in Brazil, Australia, Canada, Mexico, and Japan.

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.

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.

Clinical Trial Results

The safety and efficacy of VIMIZIM were assessed in a 24-week, randomized, double-blind, placebo-controlled clinical trial of 176 patients with MPS IVA ages 5 to 57 years old. The primary endpoint of the trial, change in six-minute walk distance at 24 weeks, was statistically significant in patients receiving weekly infusions of VIMIZIM at the dose of 2 mg/kg with a mean increase of 22.5 meters ($p=0.0174$) over placebo.

In patients who continued to receive VIMIZIM 2 mg/kg once per week for another 48 weeks (for a total of 72-week exposure), walking ability was sustained to a similar level that was achieved during the first 24 weeks of treatment in the placebo-controlled trial, MOR-004.

Overall, sustained improvements across multiple efficacy measurements and across multiple clinical trials provided evidence of clinical benefit to patients with MPS IVA, a chronic, progressive disease in which clinical deterioration is the expected course.

The adverse events observed in clinical trials were similar to those seen in other enzyme replacement therapies. In the Phase 3 trial, the most common adverse reactions (≥10%) were vomiting, pyrexia, headache, nausea, abdominal pain, diarrhea, oropharyngeal pain, dizziness, dyspnoea and chills. No new types of adverse reactions were reported in the Phase 3 extension trial. The most common adverse reactions (≥10%) observed across pre-marketing clinical trials were similar in type and frequency as those observed in the placebo-controlled trial. Acute reactions requiring intervention were managed by either temporarily interrupting or discontinuing infusion, and administering additional antihistamine, antipyretics, or corticosteroids.

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.

EU Important Product Information about VIMIZIM

Anaphylaxis and severe allergic reactions 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. Frequent symptoms of hypersensitivity reactions included anaphylactic reactions, urticaria, peripheral edema, cough, dyspnea, and flushing. If these reactions occur, immediately stop the infusion of VIMIZIM and initiate appropriate medical treatment.

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 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.

It is important to initiate treatment as early as possible. Treatment of young children < age 5 years might be started although this population was not included in the pivotal study.

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

For additional information, please 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 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.

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, 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) 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 2 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 in countries other than the United States and European Union, BioMarin's ability to support the launch and obtain pricing approval for VIMIZIM in Europe, and the actual clinical efficacy of the product when used commercially. These forward-looking statements and predictions involve risks and uncertainties such that actual results may differ materially from these statements. These risks and uncertainties include, among others: actions by regulatory agencies other than the FDA and European Commission, results and timing of current and planned clinical trials of its products and the clinical experience of using VIMIZIM commercially; the ability to, and timing of obtaining reimbursement approval in each of the countries in the European Union, 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, 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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