BioMarin Announces FDA Approval for VIMIZIM(TM) (elosulfase alfa) for the Treatment of Patients With Morquio A Syndrome

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

SAN RAFAEL, Calif., Feb. 14, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that the U.S. Food and Drug Administration (FDA) has approved VIMIZIM™ (elosulfase alfa) for patients with Mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome).

"The FDA approval of VIMIZIM is an important milestone for BioMarin and for patients with Morquio A syndrome. VIMIZIM is the first and only therapy designed to address the condition at the cellular level, fulfilling a large unmet medical need for patients and their families," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "With the approval of VIMIZIM, BioMarin firmly establishes its leadership in advancing therapies to treat MPS diseases. We have developed three therapies to treat three different MPS diseases and continue to build on our extensive scientific and clinical knowledge of lysosomal storage disorders to develop therapies for other rare genetic diseases."

VIMIZIM is an enzyme replacement treatment for Morquio A syndrome, which 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.

"In clinical trials, VIMIZIM was shown to significantly improve endurance, which possibly could change the course of the disease. As a treating physician, I am encouraged that the therapy has proven to provide clinical benefit, which is not always possible to demonstrate with ultra-rare diseases," said Paul Harmatz, M.D., Associate in Gastroenterology and Nutrition at the Children's Hospital and Research Center in *Oakland*, California and clinical investigator in the VIMIZIM Phase 3 trial. "The approval of VIMIZIM is an important advance for Morquio A patients and their families and moves treatment beyond supportive care to treating the underlying cause of the disease."

"We are thrilled that patients with Morquio A syndrome will have access to this potentially life-changing therapy and appreciate BioMarin's commitment to the MPS community and the individuals and their families who are affected by these devastating conditions," said Barbara Wedehase, MSW, CGC, Executive Director of the National MPS Society. "Until now, patients with Morquio A syndrome didn't have a drug treatment option. This approval provides the community with a therapy and with hope."

Shipments of VIMIZIM to the distribution channels will commence immediately, and BioMarin will begin promotion of VIMIZIM in the U.S. immediately. BioMarin has also submitted marketing applications for VIMIZIM in the European Union, Brazil, Australia, Canada, and Mexico.

BioMarin will offer support to patients through its BioMarin Patient & Physician Support (BPPS) team. Through BPPS, patients receive live, personalized support by a specialized case manager who will research insurance coverage and alternative benefit options. BPPS will help patients obtain coverage and minimize out-of-pocket expenses and find alternative financial assistance for treatment. To reach a BPPS case manager, please call, toll-free, 1-866-906-6100 or e-mail bpps@bmrn.com. For more information about VIMIZIM, please visit www.VIMIZIM.com.

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 (MOR-004). 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% and a higher incidence than placebo) that occurred were pyrexia, vomiting, headache, nausea, abdominal pain, chills, and fatigue. 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.

Note to Investors

BioMarin will host a webcast to discuss the VIMIZIM approval Tuesday, February 18, 2014 at 5:00 a.m. PT. Dial-in information for the conference call will be distributed prior to the call.

Interested parties may access a live audio webcast of the conference call via the investor section of the BioMarin website, www.BMRN.com. A replay of the call will be archived on the site for one week following the call.

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.

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 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 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 in countries other than the United States, 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 and predictions and 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, results and timing of current and planned clinical trials of its products; the availability and launch of VIMIZIM in the United States 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.

CONTACT: Investors

Traci McCarty

BioMarin Pharmaceutical Inc.

(415) 455-7558

Media

Debra Charlesworth

BioMarin Pharmaceutical Inc.

(415) 455-7451
