BioMarin Announces 11 Presentations at 13th Annual WORLDSymposium™ 2017

Oral Presentations to Include Research and Data in Ongoing Studies for the Treatment of Morquio A Syndrome and CLN2 Disease, a Form of Batten Disease

SAN RAFAEL, Calif., Feb. 15, 2017 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today that the company will present data in four oral and seven poster presentations at the 13th Annual WORLD*Symposium*™ being held February 13-17, 2017 in San Diego, California.

"The annual WORLD Symposium is a great forum for the rare disease community as it brings together leading industry experts, each committed to developing and advancing treatment options for patients," said Hank Fuchs, M.D., President Worldwide Research and Development at BioMarin. "We look forward to sharing our latest research through eleven presentations that explore both our existing therapies, as well as potential new therapies for conditions with unmet medical needs."

Listing of Posters and Presentations Related to BioMarin Products and Programs at the 13th Annual WORLD*Symposium*™

Oral Presentations

Title	**Authors**
Preliminary findings of a twenty-six week or longer intracerebroventricular infusion study of BMN 250 administered once every 2 weeks in a canine model of Mucopolysaccharidosis type IIIB **Presentation:** February 15 at 4:15-4:30 PM	Ellinwood NM, DVM, PhD
Long-term safety and efficacy of intracerebroventricular enzyme replacement therapy with cerliponase alfa in children with CLN2 disease: interim results from an ongoing multicenter, multinational extension study **Presentation:** February 16 at 10:15-10:30 AM	Schulz A, MD
Elosulfase alfa treatment and changes in physical functioning and disability in Morquio syndrome type A **Presentation:** February 16 at 11:30-11:45 AM	Hendriksz CJ, MD
Sub-analysis of long-term elosulfase alfa treatment outcomes in adults with Morquio syndrome type A **Presentation:** February 16 at 1:00-1:15 PM	Parini R, MD

Poster Presentations

CLN2	
Title	**Authors**
Immunogenicity to cerliponase alfa, an enzyme replacement therapy for patients with CLN2 disease: results from a Phase 1 / 2 study **Presentation:** February 14 at 4:30-6:30 PM	Cherukuri A, Cahan H, Van Tuyl A, de Hart G, Slasor P, Bray L, Henshaw J, Ajayi T, Jacoby D, O'Neill C, Schweighardt B.

Poster/Presentation: #55	

MPS	
Title	**Authors**
Long-term galsulfase treatment improves survival of patients with mucopolysaccharidosis VI (MPS VI, Maroteaux-Lamy Syndrome): 15 year follow up from the survey study **Presentation:** February 14 at 4:30-6:30 PM **Poster/Presentation:** #113	Giugliani R, Lampe C, Guffon N, Ketteridge D, Leao-Teles E, Jones SA, Quartel A, Harmatz P.
Elosulfase alfa treatment and changes in physical functioning and disability in Morquio A **Presentation:** February 14 at 4:30-6:30 PM **Poster/Presentation:** #140	Hendriksz CJ, Parini R, AlSayed MD, Raiman J, Giugliani R, Mitchell JJ, Burton BK, Guelbert N, Stewart F, Hughes DA, Matousek R, Hawley SM, Decker C, Harmatz P.
Clinical outcomes from a sub-analysis of adults with Morquio A in a long-term extension study of elosulfase alfa treatment **Presentation:** February 14 at 4:30-6:30 PM **Poster/Presentation:** #148	Hughes D, Giugliani R, Guffon N, Jones SA, Mengel KE, Parini R, Matousek R, Jurecki E, Quartel A.
Design and rationale of ongoing observational and treatment studies for BMN 250, a novel enzyme replacement therapy for Sanfilippo B syndrome **Presentation:** February 15 at 4:30-6:30 PM **Poster/Presentation:** #308	Shaywitz A, Maricich SM, Yu H, Kent S.
Preliminary safety and pharmacodynamic response from a Phase 1/2 study of ICV BMN 250, a novel enzyme replacement therapy for the treatment of Sanfilippo B syndrome (MPS IIIB) **Presentation:** February 15 at 4:30-6:30 PM **Poster/Presentation:** #LB-12	de Castro Lopez MJ, Muschol N, Clearly M, Shaywitz AJ, Cahan H, Grover A, Maricich S, Melton A, Pinkstaff J, Smith L, Couce ML.
Development of an assay to measure LAMP2 in canine brain and cerebrospinal fluid samples **Presentation:** February 15 at 4:30-6:30 PM **Poster/Presentation:** #320	Soon RK, Tripp M, Chandriani S, Wait J, Pinkstaff J, Russell C, Prill H, Lawrence R, Crawford B, Ellinwood NM, Pryer N, Jesaitis L, Melton A.

Vimizim Indication

Vimizim (elosulfase alfa) is indicated for patients with Mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome).

Vimizim Important Safety Information

Life-threatening allergic reactions, known as anaphylaxis, can occur during Vimizim (elosulfase alfa) infusions. Typical signs of anaphylaxis include cough, rash, throat tightness, hives, flushing, changes in skin color, low blood pressure, shortness of breath, chest pain, and gastrointestinal symptoms such as nausea, abdominal pain, retching, and vomiting. Contact your doctor or get medical help right away if these symptoms occur during or after Vimizim infusions. If you have a respiratory illness, you may be at risk for a sudden worsening of your condition, and you may require additional monitoring.

Vimizim is a prescription medicine. Before treatment with Vimizim, it is important to discuss your medical history with your doctor. Tell your doctor if you are sick or taking any medication and if you are allergic to any medicines. Also tell your doctor if you are pregnant, planning to become pregnant, or are a nursing mother. Your doctor will decide if Vimizim is right for you. If you have questions or would like more information about Vimizim, contact your doctor.

Anaphylaxis can occur during any Vimizin infusion and up to three hours after any infusion, and hypersensitivity reactions have been observed as early as 30 minutes from the start of infusion but as late as six days after infusion.

Serious and severe reactions can happen with Vimizim treatment, including life-threatening allergic reactions (anaphylaxis), hives, swelling, cough, shortness of breath, and flushing. You should receive medication such as antihistamines before Vimizim infusions to reduce the risk of reactions. If a reaction occurs, the infusion should be slowed or stopped and you may be given additional medication. If a severe reaction occurs, the infusion should be stopped immediately and you will receive appropriate medical treatment.

If you have acute febrile or respiratory illness at the time of Vimizim infusion you may be at higher risk of life-threatening complications from hypersensitivity reactions. If you use supplemental oxygen or continuous positive airway pressure (CPAP) you should have it available during your infusion in the event of a sudden reaction, or extreme drowsiness/sleep from antihistamines.

Spinal cord damage may occur due to the natural MPS IVA disease process. Signs of spinal cord injury include back pain, numbness and paralysis, and loss of bladder and bowel control. Contact your doctor immediately if you develop any of these symptoms.

The most common side effects reported during Vimizim infusions included fever, vomiting, headache, nausea, abdominal pain, chills, and fatigue. These are not all of the possible side effects with Vimizim. Talk to your doctor if you have any symptoms that bother you or that do not go away.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

For more information, call BioMarin Patient and Physician Support (BPPS) at 1-855-MORQUIO (1-855-667-7846).

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

Indication

NAGLAZYME® (galsulfase) is indicated for patients with Mucopolysaccharidosis VI (MPS VI; Maroteaux-Lamy syndrome). NAGLAZYME has been shown to improve walking and stair-climbing capacity.

Important Safety Information

Life-threatening anaphylactic reactions and severe allergic reactions have been observed in some patients during NAGLAZYME (galsulfase) infusions and up to 24 hours after infusion. If these reactions occur, immediate discontinuation of NAGLAZYME is recommended and appropriate medical treatment should be initiated, which may include resuscitation, epinephrine, administering additional antihistamines, antipyretics or corticosteroids. In patients who have experienced anaphylaxis or other severe allergic reactions during infusion with NAGLAZYME, caution should be exercised upon rechallenge; appropriately trained personnel and equipment for emergency resuscitation (including epinephrine) should be available during infusions.

As with other enzyme replacement therapies, immune-mediated reactions, including membranous glomerulonephritis have been observed. In clinical trials, nearly all patients developed antibodies as a result of treatment with NAGLAZYME; however, the analysis revealed no consistent predictive relationship between total antibody titer, neutralizing or IgE antibodies, and infusion-associated reactions, urinary glycosaminoglycan (GAG) levels, or endurance measures.

Caution should be exercised when administering NAGLAZYME to patients susceptible to fluid volume overload because congestive heart failure may result. Consider a decreased total infusion volume and infusion rate when administering NAGLAZYME to these patients.

Consideration to delay NAGLAZYME infusion should be given when treating patients who present with an acute febrile or respiratory illness. Sleep apnea is common in MPS VI patients and antihistamine pretreatment may increase the risk of apneic episodes. Evaluation of airway patency should be considered prior to the initiation of treatment. 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 infusion reaction, or extreme drowsiness/sleep induced by antihistamine use.

Pretreatment with antihistamines with or without antipyretics is recommended prior to the start of infusion to reduce the risk of infusion reactions. If infusion reactions occur, decreasing the infusion rate, temporarily stopping the infusion, or administering additional antihistamines and/or antipyretics is recommended.

During infusion, serious adverse reactions included laryngeal edema, apnea, pyrexia, urticaria, respiratory distress, angioedema, and anaphylactoid reaction; severe adverse reactions included urticaria, chest pain, rash, abdominal pain, dyspnea, apnea, laryngeal edema, and conjunctivitis. The most common adverse events (≥ 10%) observed in clinical trials in patients treated with NAGLAZYME were rash, pain, urticaria, pyrexia, pruritus, chills, headache, nausea, vomiting, abdominal pain and dyspnea. The most common adverse reactions requiring interventions are infusion-related reactions.

Spinal/cervical cord compression is a known and serious complication that is expected to occur during the natural course of MPS VI. Signs and symptoms of spinal/cervical cord compression include back pain, paralysis of limbs below the level of compression, and urinary or fecal incontinence. Patients should be evaluated for spinal/cervical cord compression prior to initiation of NAGLAZYME to establish a baseline and risk profile. Patients treated with NAGLAZYME should be regularly monitored for the development or progression of spinal/cervical cord compression and be given appropriate clinical care.

To report SUSPECTED ADVERSE REACTIONS contact BioMarin Pharmaceutical Inc. at 1-866-906-6100, or FDA at 1-800-FDA-1088 or go to www.fda.gov/medwatch.

Please see full Prescribing Information.

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for patients with serious and life-threatening rare and ultra-rare genetic diseases. The company's portfolio consists of five commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit www.biomarin.com.

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