BioMarin Announces 20 Presentations at 14th Annual WORLDSymposium™ 2018

Oral Presentations to Highlight Results from Studies for the Treatment of Sanfilippo Syndrome Type B, Morquio A Syndrome and CLN2 Disease, a Form of Batten Disease

SAN RAFAEL, Calif., Feb. 5, 2018 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today that the company will present data in five oral and 15 poster presentations at the 14th Annual WORLD*Symposium*™ being held February 5-9, 2018 in San Diego, California.

"We are excited to share our latest research across several medicines and treatment approaches at the annual WORLD*Symposium*," said Hank Fuchs, M.D., President of Worldwide Research and Development at BioMarin. "We are making significant advances in our rare disease portfolio, and the data underscore our ongoing commitment to advancing the care of patients with rare genetic diseases."

Listing of BioMarin Posters and Presentations at the 14th Annual WORLD*Symposium*™

Oral Presentations

Title	Authors
Translational dose-response and frequency scaling for BMN 250 Administered via the intracerebroventricular route: predicting a clinically effective dosing regimen from animal	Grover A, McCullagh E, Aoyagi-Scharber M, Maricich S, Wait J, Melton A, Zhou H, Lawrence R,

models of disease for the treatment of Sanfilippo syndrome type B **Presentation:** February 7 at 2:00-2:15 PM	Prill H, Crawford B, Henshaw J.
Intravitreal enzyme replacement therapy attenuates retinal disease progression in a canine model of neuronal ceroid lipofuscinosis type 2 (CLN2) **Presentation:** February 7 at 4:15-4:30 PM	Sinclair JR, Whiting REH, Robinson GO, Bibi K, Nguyen A, Cherukuri A, Henshaw J, de Hart G, Chandra SA, O'Neill CA, Katz ML.
ICV-administered BMN 250 (NAGLU-IGF2) is well tolerated and reduces heparan sulfate accumulation in the CNS of subjects with Sanfilippo syndrome type B (MPS IIIB) **Presentation:** February 8 at 9:15-9:30 AM	Muschol N, Cleary M, Couce ML, Shaywitz AJ, Cahan H, Grover A, Maricich SM, Melton A, Smith L, de Castro Lopez MJ.
Long-term safety and efficacy of intracerebroventricular enzyme replacement therapy with cerliponase alfa in children with CLN2 disease: two year results from an ongoing multicenter extension study **Presentation:** February 8 at 9:30-9:45 AM	Schulz A, Specchio N, Gissen P, de los Reyes E, Cahan H, Slasor P, Ajayi T, Jacoby D.
Clinical outcomes in Morquio syndrome type A treated with elosulfase-alfa: results from the managed access agreement in England	Vijay S, Cleary M, Geberhiwot T, Hendriksz CJ, Hughes D, Jones SA, Jovanovic A, Lavery C,

Presentation: February 8 at 2:15-2:30 PM	Murphy E.

Poster Presentations

Poster sessions will be held in the Harbor Ballroom.

CLN2	
Title	**Authors**
Use of epilepsy gene panels for early diagnosis of epilepsy in children 2-4 years of age: expert considerations on current and future practices in Europe **Poster/Presentation:** #162	Izzo E, Barroso E, Bailey M, Griesbach S, Lerche H, Jenkins L, Guern E, Mei D, Mikhaylova S, Santorelli F, Miller N.
Molecular basis of CLN2 disease: a review and classification of TPP1 gene variants reported world wide **Poster/Presentation:** #246	Mole S, Gardner E, Schulz A, Xin W.
CLN2 Disease (neuronal ceroid lipofuscinosis type 2): experience in the real world with cerliponase alfa intracerebroventricular enzyme replacement therapy in a public hospital in Cordoba, Argentina **Poster/Presentation:** #340	Seratti G, Muñoz V, Guelbert N, Jalil F, Velazquez D, Pueyrredón F, Guelbert G, Caraballo R, Rodrigo T.

MPS	
Title	**Authors**
Mucopolysaccharidosis type VI (MPS VI) and molecular analysis: a review of published classified variants in the *ARSB* gene **Poster/Presentation:** #20	Bailey M, Tomanin R, Karageorgos L, AlSayed M, Izzo E, Miller N, Sakuraba H, Zanetti A, Hopwood JJ.
Enzyme replacement therapy attenuates disease progression in two Japanese siblings with mucopolysaccharidosis type VI: 10-year follow up (case report) **Poster/Presentation:** #102	Furujo M, Kosuga M, Okuyama T.
Mucopolysaccharidosis VI enzyme replacement therapy initiated in adulthood: findings from the MPS VI clinical surveillance program **Poster/Presentation:** #132	Harmatz P, Lampe C, Parini R, Sharma R, Teles EL, Hawley S, Johnson J, Sivam D, Sisic Z.

Mucopolysaccharidosis VI enzyme replacement therapy outcomes across the disease spectrum: findings from the MPS VI Clinical Surveillance Program **Poster/Presentation:** #133	Harmatz P, Lampe C, Parini R, Sharma R, Teles EL, Hawley S, Johnson J, Sivam D, Sisic Z.
Safety, efficacy, and immunogenicity of elosulfase alfa in patients with Morquio A syndrome participating in 2 sequential open-label studies (MOR-002/MOR-100), representing 5 years of treatment **Poster/Presentation:** #139	Hendriksz C, Santra S, Jones SA, Geberhiwot T, Jesaitis L, Long B, Qi Y, Hawley S, Decker C.
Presenting signs and symptoms of MPS: results of an international physician survey **Poster/Presentation:** #244	Mitchell J, Clarke L, Ellaway C, Foster H, Giugliani R, Goizet C, Goring S, Hawley S, Jurecki E, Khan Z, Lampe C, Martin K, McMullen S, Mubarack F, Muenzer J, Sivri S, Stewart F, Tylki-Szymanska A, White K, Wijburg F.
Presenting signs and symptoms of MPS: results of	Mitchell J, Clarke L, Ellaway C, Foster H, Giugliani R, Goizet C, Goring S, Hawley S, Jurecki E, Khan Z, Lampe C, Martin K,

systematic literature analysis **Poster/Presentation:** #245	McMullen S, Mubarack F, Muenzer J, Sivri S, Stewart FJ, Tylki-Szymanska A, White K, Wijburg F.
Natural history data for young subjects with Sanfilippo Syndrome type B (MPS IIIB) **Poster/Presentation:** #279	Okur I, Cleary M, de Castro Lopez MJ, Harmatz P, Lee J, Lin S-P, Couce ML, Muschol N, Peters H, Solano Villarreal M, Shaywitz AJ, Cahan H, Grover A, Maricich SM, Melton A, Smith L, Ezgu F.
Pharmacology of BMN 250 administered via intracerebroventricular infusion once every 2 weeks for twenty-six weeks or longer in a canine model of mucopolysaccharidosis type IIIB **Poster/Presentation:** #82	Ellinwood NM, Valentine B, Hess AS, Jens JK, Snella EM, Ware W, Hostetter S, Ben Shlomo G, Jeffery N, Safayi S, Smith JD, Millman S, Parsons B, Butt M, Cooper J, Nestrasil I, Prill H, Liu X, Zhou H, Lawrence R, Grover A, Melton A, Cherukuri A, Wait JCM, Pinkstaff J, McCullagh E.
Biochemical evaluation of from-birth intracerebroventricular rhNAGLU-IGF2 enzyme replacement therapy treatment in mice with Sanfilippo syndrome type B **Poster/Presentation:** #LB-28	Kan S, Le SQ, Cooper JD, Sands MS, Dickson PI.

MPS/CLN2	
Title	**Authors**
Immunogenicity bioanalytical methods and assessment of anti-drug antibody impact **Poster/Presentation:** #LB-27	Jesaitis L, Melton A, de Hart G, Cherukuri A, Long B, Steinhauer S, Zoog S, Pryer N, Schweighardt B, Hock B.

Other	
Title	**Authors**
Exploration of disease biomarkers in a canine model of Krabbe disease **Poster/Presentation:** #LB-10	Corado C, Chandriani S, Scholler O, Russell C, Ru K, Jiang X, Pinkstaff J, Vite C, Bradbury A.

About Brineura®

Brineura is a recombinant form of human tripeptidyl peptidase 1 (TPP1), the enzyme deficient in patients with CLN2 disease. It is an enzyme replacement therapy designed to restore TPP1 enzyme activity and break down the storage materials that cause CLN2 disease. In order to reach the cells of the brain and central nervous system, the treatment is delivered directly into the fluid surrounding the brain (cerebrospinal fluid) using BioMarin's patented

technology.

For additional information regarding this product, please contact BioMarin Medical Information at medinfo@bmrn.com.

Indication

Brineura® (cerliponase alfa) is a prescription medication used to slow loss of ability to walk or crawl (ambulation) in symptomatic pediatric patients 3 years of age and older with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), also known as tripeptidyl peptidase 1 (TPP1) deficiency.

Important Safety Information

Brineura is a prescription medicine. Before treatment with Brineura, it is important to discuss your child's medical history with their doctor. Tell the doctor if they are sick or taking any medication and if they are allergic to any medicines. Your child's doctor will decide if Brineura is right for them. If you have questions or would like more information about Brineura, contact your child's doctor.

Brineura is only given by infusion into the fluid of the brain (known as an intraventricular injection) and using sterile technique to reduce the risk of infection. An intraventricular access device or port must be in place at least 5 to 7 days prior to the first infusion. Intraventricular access device-related infections were observed with Brineura treatment. If any signs of infection occur, contact your child's doctor immediately. Your child's intraventricular access device may need to be replaced over time.

Brineura should not be used in patients with active intraventricular access

device-related complications (e.g., leakage, device failure, or device-related infection) and with shunts used to drain extra fluid around the brain.

Low blood pressure and/or slow heart rate may occur during and following the Brineura infusion. Contact your child's doctor immediately if these reactions occur.

Undesirable or hypersensitivity reactions related to Brineura treatment, including fever, vomiting, and irritability, may occur during treatment and as late as 24 hours after infusion. Your child may receive medication such as antihistamines before Brineura infusions to reduce the risk of reactions. Serious and severe allergic reactions (anaphylaxis) may occur. If a reaction occurs, the infusion will be stopped and your child may be given additional medication. If a severe reaction occurs, the infusion will be stopped and your child will receive appropriate medical treatment. If any signs of anaphylaxis occur, immediately seek medical care.

Safety and effectiveness in pediatric patients below 3 years of age have not been established.

The most common side effects reported during Brineura infusions included fever, problems with the electrical activity of the heart, decreased or increased protein in the fluid of the brain, vomiting, seizures, hypersensitivity, collection of blood outside of blood vessels (hematoma), headache, irritability, and increased white blood cell count in the fluid of the brain, device-related infection, slow heart rate, feeling jittery, and low blood pressure. Intraventricular device-related side effects included infection, delivery system-related complications, and increased white blood cell count in fluid of the brain.

These are not all of the possible side effects with Brineura. Talk to your child's doctor if they have any symptoms that bother them 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.

Please see accompanying full Prescribing Information, or visit www.Brineura.com.

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

Severe and life-threatening allergic reactions can occur during NAGLAZYME (galsulfase) infusions and up to 24 hours after infusion. Typical signs of an allergic reaction include shock, difficulty breathing, wheezing, swelling of the throat, and low blood pressure. If a severe allergic reaction occurs during infusion, the infusion should be stopped immediately and you should receive medical attention. Contact your doctor or get medical help right away if you develop any severe symptoms after infusion.

In clinical trials, most patients developed antibodies to NAGLAZYME treatment. There was no clear relationship between antibody formation and the safety or effectiveness of NAGLAZYME.

Serious and severe infusion reactions are associated with NAGLAZYME, including hives, chest pain, rash, abdominal pain, difficulty breathing, swelling, fever, and eye irritation. You should receive medication such as antihistamines before NAGLAZYME infusions to reduce the risk of infusion reactions. If an infusion reaction occurs, the infusion should be slowed or stopped and you may be given additional medication.

The most common side effects of NAGLAZYME seen in clinical trials were rash, pain, hives, fever, itching, chills, headache, nausea, vomiting, abdominal pain and difficulty breathing. The most common side effects requiring medical attention are infusion-related effects.

These are not all of the possible side effects with NAGLAZYME. Talk to your doctor if you have any symptoms that bother you or that do not go away.

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

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

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.BMRN.com.

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