

BioMarin Announces 21 Presentations at 12th Annual WORLDSymposium™ 2016 February 29-March 4 in San Diego, California: 6 Oral and 15 Poster

Oral Presentations Include Results From Phase 1/2 Study on Cerliponase Alfa in Children With CLN2 Disease, a Form of Batten Disease

SAN RAFAEL, Calif., Feb. 29, 2016 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (NASDAQ:BMRN) announced today that the company will present data in six oral and 15 poster presentations at the 12th Annual WORLDSymposium™ being held February 29-March 4, 2016 in San Diego, California.

Results will be presented from a phase 1/2, open-label, dose-escalation study on cerliponase alfa (BMN 190) in children with late-infantile neuronal ceroid lipofuscinosis type 2, or CLN2 disease. Cerliponase alfa is an investigational enzyme replacement therapy designed to treat CLN2 disease, a form of Batten disease that causes progressive neurodegeneration and loss of cognitive, motor and visual functions, and early mortality. Additionally, poster presentations will discuss expert opinions on the diagnosis, and management of the disease.

"We are committed to developing first-in-class and best-in-class therapeutics for children and adults who live with serious and life-threatening rare genetic diseases. We continue to evaluate both existing and emerging therapies to better understand their impact on patients, as well as the challenges of diagnosing and managing these diseases," said Hank Fuchs, MD, Executive Vice President and Chief Medical Officer at BioMarin. "We have more than 20 presentations across multiple conditions showing many promising results, and we are delighted to be moving continually closer to better addressing the needs of those with rare conditions."

An additional five oral sessions will highlight research on the management and treatment of mucopolysaccharidosis (MPS), a group of metabolic disorders caused by the deficiency of specific lysosomal enzymes. Two of the sessions will evaluate the impact of Vimizim® (elosulfase alfa) in patients with Morquio A syndrome, a rare inherited disease that affects major organ systems in the body and can cause heart disease, skeletal abnormalities, vision and hearing loss, difficulty breathing, and early death. Other studies on MPS disorders provide an understanding into the challenges associated with these diseases, such as the management of fertility and pregnancy.

Data will also be presented on BMN 250, an investigational enzyme replacement therapy being developed to treat Sanfilippo B Syndrome.

Listing of BioMarin Posters and Presentations at the 12th Annual WORLDSymposium™

Oral Presentations

Title

Authors

Intracerebroventricular cerliponase alfa (BMN 190) in children with CLN2 disease: Results from a Phase 1/2, open-label, dose-escalation study

Schulz A, Specchio N, Gissen P, de los Reyes E, Williams R, Cahan H, Genter F, Jacoby D

Presentation: March 2, 4:00 — 4:15 PM

Poster/Presentation: #LB-21

Intracerebroventricular (ICV) administration of BMN 250 to cynomolgus monkeys results in elevated tissue levels and superior biodistribution in the central nervous system (CNS) in comparison to intravenous (IV) delivery

Grover A, Shaywitz A, Wait JCM, Melton A, O'Neill C, Pinkstaff J

Presentation: March 2, 3:00 — 3:15 PM

Poster/Presentation: #119

Time- and dose-dependent normalization of pathological lysosomal storage and biochemistry in the Mucopolysaccharidosis IIIB (MPS IIIB, Sanfilippo B) mouse model by intracerebroventricular enzyme replacement therapy with BMN 250, a NAGLU-IGF2

Aoyagi-Scharber M, Vincelette J, Lawrence R, Crippen-Harmon D, Yip BK, Baridon B, Prill H, Minto WC, Van Vleet JL, Vitelli C, Adintori EG, Christianson T, Tiger PMN, Lo MJ,

fusion protein

Holtzinger J, Chen E, Webster KA, Brown JR, Fitzpatrick PA, LeBowitz JH, Bagri A, Bullens S, Crawford BE, Bunting S

Presentation: March 2, 2:00 — 2:15 PM

Poster/Presentation: #22

Utilizing activity assays and population-wide allele frequencies to assess the contribution of novel mutations in NAGLU to MPS IIIB incidence

LeBowitz JH, Clark WT, Yu GK, Aoyagi-Scharber M

Presentation: March 1, 2:30 — 2:45 PM

Poster/Presentation: #176

Impact of long-term elosulfase alfa treatment on pulmonary function in patients with Morquio syndrome type A

Hendriksz CJ, Burton BK, Al-Sayed MD, Giugliani R, Guelbert N, Hughes D, Mealiffe M, Mitchell JJ, Parini R, Raiman J, Shaywitz AJ, Slasor P, Solano Villarreal ML, Stewart F, Berger KI, Harmatz PR

Presentation: March 3, 9:15 — 9:30 AM

Poster/Presentation: #129

Impact of elosulfase alfa in patients with Morquio syndrome type A who have limited ambulation: an open-label, phase 2 study

Harmatz PR, Jester A, Mengel E, Treadwell M, Burton BK, Berger KI, Hendriksz CJ, Geberhiwot T, Sisic Z, Decker C

Presentation: March 3, 9:30 — 9:45

Poster/Presentation: #126

Poster Presentations

CLN2

Title

Expert opinion on the management of Intracerebroventricular (ICV) drug delivery

Authors

Slavc I, Cohen-Pfeffer JL, Gururangan S, Jurecki E, Krauser J, Lester T, Lim D, Maldaun M, Schwering C, Shaywitz AJ, Westphal M

Poster/Presentation: #LB-03

Expert opinion on the management of CLN2 disease

Williams R, Adams HR, Blohm M, Cohen-Pfeffer JL, de los Reyes E, Denecke J, Drago K, Fairhurst C, Frazier M, Guelbert N, Kiss S, Kofler A, Lawson J, Lehwald L, Leung M, Mikhailova S, Mink JW, Nickel M, Shediak R, Sims K, Specchio N, Topcu M, von Lobbecke

Poster/Presentation: #274

Expert recommendations for the laboratory diagnosis of neuronal ceroid lipofuscinosis type 2 (CLN2 disease): diagnostic algorithm and best practice guidelines for a timely diagnosis

Fietz M, Giugliani R, Al-Sayed M, Burke D, Cohen-Pfeffer J, Cooper J, Noher de Halac I, Dvoráková L, Izzo E, Jahnová H, Lukacs Z, Mole S, Pearce D, Schulz A, Specchio N, Xin W, Miller N

Poster/Presentation: #143

Natural history of CLN2 disease: Quantitative

assessment of disease characteristics and rate of progression

Nickel M, Jacoby D, Lezius S, Down M, Genter F, Wittes J, Kohlschütter A, Schulz A

Poster/Presentation: #LB-20

Mucopolysaccharidosis (MPS)

Title

Management of fertility and pregnancy in individuals with mucopolysaccharidosis (MPS)

Authors

Stewart F, Harmatz PR, Bentley A, Burton BK, Guffon N, Hale SL, Johnston TA, Kircher SG, Kochhar PK, Mitchell J, Plöckinger U, Semotok J, Sisic Z

Poster/Presentation: #293

Pregnancy in individuals with mucopolysaccharidosis (MPS): a case series

Stewart F, Harmatz PR, Braunlin EA, Bentley A, Burton BK, Guffon N, Hale SL, Johnston TA, Kircher SG, Kochhar P, Mitchell J, Plöckinger U, Sisic Z

Poster/Presentation: #292

Histologic characterization of the progression of CNS pathology in the Mucopolysaccharidosis IIIB (MPS IIIB, Sanfilippo B) mouse model and bio-distribution and efficacy of the intracerebroventricular enzyme replacement therapy, BMN-250, a NAGLU-IGF2 fusion protein

Bagri A, Crippen-Harmon D, Vincelette J, Vitelli C, Minto WC, Yates B, Rigney S, Santiago P, Baridon B, Xie L, Gorostiza O, Christianson T, Tiger PMN, Yip BK, Lo MJ, Holtzinger J, Chen E, Aoyagi-Scharber M, Fitzpatrick PA, LeBowitz JH, Bullens S, Bunting S

Poster/Presentation: #29

Glycosylation independent lysosomal targeting of alpha-N-acetylglucosaminidase confers highly efficient enzyme uptake into critical cellular targets of disease pathogenesis in Mucopolysaccharidosis Type IIIB

Yogalingam G, Prill H, Lee A, Christianson T, Tiger PMN, Lo MJ, Holtzinger J, Berguig G, Yip BK, Aoyagi-Scharber M, Fitzpatrick PA, Lawrence R, Crawford BE, LeBowitz JH

Poster/Presentation: #333

Immunohistochemical analysis of mannose 6-phosphate/insulin-like growth factor 2 receptor (M6PR/IGF2R) in wild-type and MPS IIIB CNS vasculature and implications for trans-blood brain barrier (BBB) transport

Bagri A, Crippen-Harmon D, Rigney S, Nave L, Minto WC, Vitelli C, Yates B, Vincelette J, Santiago P, Baridon B, Xie L, Gorostiza O, Bullens S, LeBowitz JH, Bunting S

Poster/Presentation: #30

New chondroitin sulfate derived non-reducing end (NRE) biomarker for the diagnosis and measurement of therapeutic response in Morquio syndrome type A (MPS IVA)

Lawrence R, Prill H, Wang RY, Adintori EG, Pollock T, Wang E, Pungor E, Pasquali M, Crawford BE

Poster/Presentation: #LB-14

Use of a Radial Arm Maze to Assess Cognition in Normal and MPSIIIB Affected Dogs

Parson R, Ellinwood NN, Zylstra T, Greiner A, Johnson B, Millman S

Poster/Presentation: #234

Use of the T-maze to Assess Cognition in Normal and MPS IIIB Affected Dogs

Parsons R, Ellinwood NM, Zylstra T, Greiner A, Johnson B, Millman S

Poster/Presentation: #233

Validation of a Kinematic Assessment of Cerebellar Dysfunction in the Canine Model of Mucopolysaccharidosis Type III B (MPS IIIB)

Jeffrey N, Johnson J, Hess A, Safayi S, Ellinwood NM

Poster/Presentation: #146

Twenty-Six Week or Longer Intracerebroventricular (ICV) Infusion Study of BMN 250 Administered once every 2 weeks in a Canine Model of Mucopolysaccharidosis type IIIB (MPS IIIB)

Ellinwood NM, Johnson B, Jens J, Snella E, Ware W, Hostetter S, Ben-Shlomo G, Jeffery N, Safayi S, Millman S, Parsons R, Vuillemenot B, Butt M, Cooper J, Nestrasil I, Wendt D, Kennedy D, Grover A, Melton A, Cherukuri A, Wait JCM, Pinkstaff J

Poster/Presentation: #90

Design and Rationale of the Study Programs for BMN 250, a Novel ERT for Sanfilippo B Syndrome

Shaywitz A, Kent S, Oh M

Poster/Presentation: #280

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 Vimizim 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.

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.

Contact:

Investors:

Traci McCarty
BioMarin Pharmaceutical Inc.
(415) 455-7558

Media:

Debra Charlesworth
BioMarin Pharmaceutical Inc.
(415) 455-7451

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