

BioMarin Announces Two Oral and 16 Poster Presentations at Society for the Study of Inborn Errors of Metabolism 2016 Annual Meeting

Oral Presentations Include Updated Long-Term Data from Extension Study of Brineura™ (Cerliponase Alfa) in Children with CLN2 Disease, and Long-Term Safety and Efficacy Data of Pegvaliase in Adults with PKU

SAN RAFAEL, Calif., Sept. 08, 2016 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (NASDAQ:BMRN) announced today that two oral and 16 poster presentations related to the company's products and product candidates will be made at the Society for the Study of Inborn Errors of Metabolism (SSIEM) 2016 Annual Meeting, which will be held on September 6-9 in Rome, Italy. These 18 presentations at SSIEM cover a range of areas in rare genetic diseases, including Phenylketonuria (PKU), Mucopolysaccharidosis (MPS) and CLN2 disease, a form of Batten disease. The presentations focus on better understanding the burden of disease in PKU, intracerebroventricular (ICV) delivery and the multi-disciplinary management of CLN2 disease and the impact of treatment on adults with Morquio A syndrome, an MPS disease.

Oral Platforms at SSIEM

Updated long-term data from an extension study of Brineura™ (cerliponase alfa), a recombinant human tripeptidyl peptidase 1 (rhTPP1) to treat children with CLN2 disease, will be presented during an oral platform session. CLN2 disease is a rapidly progressing, fatal neurodegenerative disease with no approved treatments, where the majority of affected children lose their ability to walk and talk by approximately six years of age. Brineura is an enzyme replacement therapy designed to restore TPP1 enzyme activity and break down the storage materials that cause the disease. Brineura is currently under review by the U.S. Food and Drug Administration with a Prescription Drug User Fee Act goal date of April 27, 2017. BioMarin also has submitted a Marketing Authorization Application to the European Medicines Agency for Brineura, and it is undergoing validation at the Agency.

In addition, updated four year results of the long-term safety and efficacy of pegvaliase to treat adults living with phenylketonuria (PKU) will also be presented during an oral session. PKU is a rare genetic condition in which the body cannot metabolize the essential amino acid phenylalanine. Pegvaliase is an enzyme substitution therapy that is designed to substitute phenylalanine ammonia lyase for phenylalanine hydroxylase, the enzyme deficient in people living with PKU. BioMarin intends to submit a marketing application by the first quarter of 2017.

Additionally, data from 16 abstracts spanning CLN2 disease, mucopolysaccharidosis, PKU, and Pompe disease will be presented.

Listing of Posters and Presentations at Society for the Study of Inborn Errors of Metabolism 2016 Annual Meeting

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	A Schulz
Presentation: Thur., Sept. 8, 2016 10:30 am — 12:00 pm Evaluation of long-term safety and efficacy of pegvaliase treatment for adults with phenylketonuria: updated year 4 results	N Longo
Presentation: Thur., Sept. 8, 2016 13:15 — 14:45 pm	

Poster Presentations

CLN2 Disease

Title	Authors
Expert opinion on the management of Intracerebroventricular (ICV) drug delivery	I Slavc, JL Cohen-Pfeffer, S Gururangan, E Jurecki, J Krauser, T Lester, D Lim, M Maldaun, C Schwering, AJ Shaywitz, M Westphal

Expert opinion on the management of CLN2 disease

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

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

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

Mucopolysaccharidosis (MPS)

Title

Authors

Design and rationale of the study programs for BMN 250, a novel Enzyme Replacement Therapy (ERT) for Sanfilippo B syndrome

A Shaywitz, M Oh, S Kent

Dramatic mobility improvement on galsulfase ERT: a case report

J Zakharchuk

Clinical outcomes from a sub-analysis of adults with Morquio A in a long-term extension study of elosulfase alfa treatment

D Hughes, R Giugliani, N Guffon, AS Jones, KE Mengel, R Parini, R Matousek, E Jurecki, A Quartel

Morquio A Registry Study (MARS): design and baseline characteristics of enrolled patients

N Guffon, G Baujat, MB Bober, BK Burton, L Clarke, P Garcia, R Giugliani, CJ Hendriksz, C Lavery, J Raiman, E Jurecki, Z Sisic, A Waite

Phenylketonuria (PKU)

Title

Authors

National study to assess current practices of the management of phenylketonuria

F Rohr, ER Jurecki, S Cederbaum, J Kopesky, A Sanchez-Valle, K Viau, JL Cohen-Pfeffer

Neuropsychiatric concomitant medications and comorbidities in individuals with Phenylketonuria: findings from the PKUDOS Registry

S Waisbren, J Zambrano, M Grant, B Cheng, S Parker, D White, JL Cohen-Pfeffer

Case-control study of neuropsychological results in patients with mild hyperphenylalaninemia (MHP)

A Belanger-Qunitana, B Zamora

Neuropsychological and quality of life outcomes in untreated adults with mild hyperphenylalaninemia with phenylalanine levels between 360 and 600 $\mu\text{mol/L}$

A Feigenbaum, A Wilson, L Nagy, K Siriwardena, E Nasr, E Kerr

Phase 3 PRISM-1 and PRISM-2 clinical trial results: to evaluate the efficacy and safety of pegvaliase for the treatment of adults with phenylketonuria (PKU)

C Harding, S Amato, J Vockley, K Wierenga, H Li, D Bilder, B Burton, D Dimmock, N Longo, J Posner, JA Thomas, R Zori, O Rosen, F Greblikas, Z Gu, M Li, M Merilainen, HH Weng, H Levy

Phase 3 PRISM-2 long-term extension study evaluating efficacy and safety of pegvaliase for treatment of adults with phenylketonuria

H Levy, C Harding, N Longo, D Bilder, B Burton, R Zori, J Posner, JA Thomas, J Olbertz, O Rosen, Z Gu, M Merilainen, HH Weng, D Dimmock

The sixth interim analysis of the Kuvan® Adult Maternal Paediatric European Registry (KAMPER): pregnancies

F Feillet, FB Lagler, J Alm, AC Muntau, A Burlina, A Belanger-Qunitana, I Alvarez, A Champigneulle, FK Trefz

The sixth interim analysis of the Kuvan®

AC Muntau, FB Lagler, F Feillet, J Alm, A Burlina, A

Adult Maternal Paediatric European Registry (KAMPER): interim results in PKU and BH4 deficiency patients Belanger-Qunitana, I Alvarez, A Champigneulle, FK Trefz, FJ Van Spronsen

Pompe disease

Title

Long-term efficacy and safety of reveglucosidase alfa in subjects with late-onset Pompe Disease: 144-week follow-up of the POM-001/002 Studies

Authors

T Geberhiwot, B Byrne, BA Barshop, R Barohn, D Hughes, D Bratkovic, C Desnuelle, P Laforet, E Mengel, M Roberts, P Haroldsen, L Smith, K Yang, L Walsh

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare disorders. 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. Information on BioMarin's website is not incorporated by reference into this press release.

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