

## **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**

<b>Title</b>	<b>Authors</b>
Intracerebroventricular cerliponase alfa (BMN 190) in children with CLN2 disease: Results from a Phase 1/2, open-label, dose-escalation study	A Schulz
<b>Presentation:</b> 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
<b>Presentation:</b> Thur., Sept. 8, 2016 13:15 — 14:45 pm	

### **Poster Presentations**

#### **CLN2 Disease**

<b>Title</b>	<b>Authors</b>
Expert opinion on the management of Intracerebroventricular (ICV) drug delivery	I Slavic, 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 Shediak, 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)**

<b>Title</b>	<b>Authors</b>
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 Hendriks, C Lavery, J Raiman, E Jurecki, Z Sisic, A Waite

### **Phenylketonuria (PKU)**

<b>Title</b>	<b>Authors</b>
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 µmol/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	H Levy, C Harding, N Longo, D Bilder, B Burton, R Zori, J Posner, JA Thomas, J Olbertz, O Rosen, Z Gu, M Merilainen,

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

The sixth interim analysis of the Kuvan® Adult  
Maternal Paediatric European Registry  
(KAMPER): interim results in PKU and BH4  
deficiency patients

HH Weng, D Dimmock

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

AC Muntau, FB Lagler, F Feillet, J Alm, A Burlina, A  
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](http://www.BMRN.com). Information on BioMarin's website is not incorporated by reference into this press release.

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