BioMarin Announces 3 Platform and 15 Poster Presentations at 13th International Congress of Inborn Errors of Metabolism 2017

SAN RAFAEL, Calif., Sept. 5, 2017 /PRNewswire/ -- BioMarin (Nasdaq:BMRN) announced that the company will present data in three platform presentations and fifteen poster presentations at the 13th International Congress of Inborn Errors of Metabolism (ICIEM) being held September 5-8, 2017 in Rio de Janeiro, Brazil. Presentations span a range of areas in rare genetic diseases, including neuronal ceroid lipofuscinosis type 2 (CLN2) disease, phenylketonuria (PKU) and mucopolysacchardosis (MPS).

Platform presentations include preliminary safety and pharmacodynamic response data from a Phase 1/2 study of ICV BMN 250, a novel enzyme replacement therapy for the treatment of Sanfilippo B (MPS IIIB), a long-term safety and efficacy analysis of cerliponase alfa to treat children with CLN2 disease, as well as updated five-year safety and efficacy data evaluating pegvaliase to treat adults with PKU.

Listing of Posters and Presentations Related to BioMarin Products and Programs at the 13th International Congress of Inborn Errors of Metabolism *(All times are local)*

Platform Presentations

Title	**Authors**
Preliminary safety and pharmacodynamics response data from a phase 1/2 study of ICV BMN 250, a novel enzyme replacement therapy for the treatment of Sanfilippo syndrome type B (MPS IIIB) **Parallel Session 1:** Wednesday, September 6 from 12:00-12:15	Steve Maricich, MD, PhD, BioMarin Pharmaceutical Inc. Novato, CA, USA
Long-term safety and efficacy of intracerebroventricular enzyme replacement therapy with cerliponase alfa in children with CLN2 disease: interim results from an ongoing multicenter extension study **Parallel Session 1:** Wednesday, September 6 from 12:00-12:15	Marina Trivisano, MD, Bambino Gesú Children's Hospital, IRCCS, Rome, Italy
Phase 2 long-term pegvaliase treatment for adults with phenylketonuria: updated year 5 safety and efficacy data from the PAL-003 extension **Parallel Session 7:** Wednesday, September 6 from 14:45-15:00	Jerry Vockley, MD, PhD, Children's Hospital of Pittsburgh and University of Pittsburgh, Pittsburgh, PA, USA

Poster Presentations

MPS	
Title	**Authors**
Design and rationale of ongoing observational and treatment studies for BMN 250, a novel enzyme replacement therapy for Sanfilippo syndrome type B (MPS IIIB) **Presentation:** Wednesday, September 6 from 17:30-20:00 **Poster/Presentation:** 597	Shaywitz A, Maricich S, Yu H, Kent S

Presenting signs and symptoms of MPS: results of an international physician survey **Presentation:** Wednesday, September 6 from 17:30-20:00 **Poster/Presentation:** 649	Clarke L, Ellaway C, Foster H, Giugliani R, Goizet C, Goring S, Hawley S, Jurecki E, Khan Z, Lampe C, Martin K, McMullen S, Mitchell J, Mubarack F, Muenzer J, Sivri S, Stewart F, Tylki-Szymanska A, White K, Wijburg F
Presenting signs and symptoms of MPS: results of systematic literature analysis **Presentation:** Wednesday, September 6 from 17:30-20:00 **Poster/Presentation:** 662	Mubarack F, Clarke L, Ellaway C, Foster H, Giugliani R, Goizet C, Goring S, Hawley S, Jurecki E, Khan Z, Lampe C, Martin K, McMullen S, Mitchell J, Muenzer J, Sivri S, Stewart FJ, Tylki-Szymanska A, White K, Wijburg F
Mucopolysaccharidosis type VI (MPS VI) and molecular analysis: a review of published classified variants in the ARSB gene **Presentation:** Wednesday, September 6 from 17:30-20:00 **Poster/Presentation:** 674	Tomanin R, Karageorgos L, AlSayed M, Bailey M, Izzo E, Miller N, Sakuraba H, Zanetti A, Hopwood JJ
Impact of elosulfase alfa treatment on patient-reported outcomes in Morquio A syndrome: results from the first year of an English managed access agreement **Presentation:** Wednesday, September 6 from 17:30-20:00 **Poster/Presentation:** 807	Lavery C, Jones SA, Hughes D, Murphy E, Jovanovic A, Hendriksz C, Cleary M, Hiwot T, Vijay S

CLN2	
Title	**Authors**
Long-term safety and efficacy of intracerebroventricular enzyme replacement therapy with cerliponase alfa in children with CLN2 disease: interim results from an ongoing multicenter extension study **Presentation:** Wednesday, September 6 from 17:30-20:00 **Poster/Presentation:** 787	Trivisano M, Schulz A, Specchio N, Gissen P, de los Reyes E, Cahan H, Slasor P, Ajayi T, Jacoby D

PKU	
Title	**Authors**
Phase 3 PRISM-2 long-term extension study evaluating efficacy and safety of pegvaliase for treatment of adults with phenylketonuria **Presentation:** Wednesday, September 6 from 17:30-20:00 **Poster/Presentation:** 285	Vockley J, Levy H, Amato S, Zori R, Thomas J, Burton B, Harding C, Longo N, Posner J, Bilder D, Olbertz J, Gu Z, Lau K, Lin M, Larimore K, Dimmock D
Phase 2 long-term pegvaliase treatment for adults with phenylketonuria: updated year 5 safety and efficacy data from the PAL-003 extension **Presentation:** Wednesday, September 6 from 17:30-20:00 **Poster/Presentation:** 288	Zori R, Levy H, Longo N, Lau K, Rosen O, Li M, Larimore K, Decker C, Weng H, Vockley J
Seventh interim analysis of the Kuvan® Adult Maternal Paediatric European Registry (KAMPER): interim results in BH4 deficiency patients **Presentation:** Wednesday, September 6 from 17:30-20:00 **Poster/Presentation:** 295	Van Spronsen FJ, Burlina A, Alm J, Belanger-Quintana A, Feillet F, Lagler FB, Muntau AC, Trefz FK, Jurecki ER, Kittus R, Alvarez I
Seventh interim analysis of the Kuvan® Adult Maternal Paediatric European Registry (KAMPER): interim results in phenylketonuria patients **Presentation:** Wednesday, September 6 from 17:30-20:00 **Poster/Presentation:**296	Van Spronsen FJ, Muntau AC, Lagler FB, Feillet F, Alm J, Burlina A, Belanger-Quintana A, Alvarez I, Lilienstein J, Jurecki E, Trefz FK
Interim analysis of the phenylketonuria (PKU) patients enrolled in the PKUDOS registry **Presentation:** Wednesday, September 6 from 17:30-20:00 **Poster/Presentation:**297	Lilienstein J, Burton B, Grant M, Ficicioglu C, Kopesky J, Nguyen-Driver MD, Moore C, Jurecki E, Longo N
An interim analysis of the Kuvan®Adult Maternal Paediatric European Registry (KAMPER) and phenylketonuria developmental outcomes and safety (PKUDOS) registries: pregnancies **Presentation:** Wednesday, September 6 from 17:30-20:00	Feillet F, Ficicioglu C, Lagler FB, Longo N, Alm J, Muntau AC, Burlina A, Belnager-Quintana A, Trefz FK, Kittus R, Jurecki E, Alvarez I, Lilienstein J, Burton B

Poster/Presentation: 298	
Estimation of resource use and quality of life in phenylketonuria (PKU) patients in Ireland **Presentation:** Wednesday, September 6 from 17:30-20:00 **Poster/Presentation:** 262	Pastores G, Bracken J, Hughes J, Rogers Y, Stenson C, Clark A, Lloyd A, Alvarez I, Jain M, Kha A, Monavari A
Short-term biological variance of PHE in patients with phenylketonuria **Presentation:** Wednesday, September 6 from 17:30-20:00 **Poster/Presentation:** 250	Potter M, Pendar A, Langley E, Geraghty M
Amino acid level correlations between tandem mass spectrometry and ultra-performance liquid chromatography and the clinical relevance for phenylketonuria management **Presentation:** Wednesday, September 6 from 17:30-20:00 **Poster/Presentation:** 251	Potter M, Pendar A, Langley E, Geraghty M

Links to Full Prescribing Information on BioMarin Products

www.Brineura.com

www.kuvan.com

www.Naglazyme.com

www.VIMIZIM.com

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 six 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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