

BioMarin Announces 20 Poster Presentations at Society for the Study of Inborn Errors of Metabolism (SSIEM) Symposium 2018

Updated results from Phase 1/2 program for tralesenidase alfa (formerly BMN 250)

SAN RAFAEL, Calif., Sept. 5, 2018 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today that the company will present 20 poster presentations at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Symposium 2018, which will be held on September 4-7 in Athens, Greece.

Listing of Posters and Presentations at Society for the Study of Inborn Errors of Metabolism 2018 Symposium



Poster Presentations

CLN2 Disease	
Title	Authors
Monitoring neuropsychological function in CLN2 disease Poster: #345	Nickel M, Augustine E, Adams H, Baron I, Bjoraker K, Cohen-Pfeffer J, Delaney K, Elmerskog B, Newsom-Davis I, Rust S, Shapiro E, Tøssebro A, Schulz A
Experiences of CLN2 disease diagnosis in the UK Poster: #357	Burke D, Church H, Jackson M, Powers V, Tylee K
Intracerebroventricular cerliponase alfa in children with CLN2 disease: interim results from an ongoing multicenter, multinational extension study Poster: #367	Schulz A, de los Reyes E, Specchio N, Gissen P, Cahan H, Slasor P, Ajayi T, Jacoby D

Phenylketonuria (PKU)	
Title	Authors
Burden of illness in adult patients with phenylketonuria and associated comorbidities - a retrospective database study in Germany Poster: #095	Rutsch F, Muntau AC, Alvarez I, Lane P, Altevers J, Kohlscheen KM, Jacob C, Jain M, Schroeder C, Jha A, Treftz F
Efficacy & safety of sapropterin in PKU patients <4 yrs: 3-year extension of SPARK open-label, randomized phase 3b trial Poster: #097	Rutsch F, Burlina A, Eyskens F, Freisinger P, De Laet C, Leuzzi V, Sivri HS, Vijay S, Bal MO, Gramer G, Pazdírková R, Cleary M, Lotz-Havla AS, Mould DR, Lane P, Alvarez I, Muntau A
Prevalence of comorbidities among phenylketonuria patients - a retrospective study of US health insurance claims data	Burton B, Cederbaum S, Jurecki E, Lilienstein J, Alvarez I, Cohen-Pfeffer J, Irwin D, Levy H, Rohr

Poster: #098	
The burden of illness in adults with phenylketonuria (PKU): interim analysis of a cross-sectional study	Burton B, Longo N, Stuy M, Vockley J, van Backle J, Lane P, Alvarez I, Lilienstein J, Jurecki E
Poster: #099	
International best practice recommendations for the evaluation of responsiveness to sapropterin dihydrochloride in patients with phenylketonuria	Bhattacharya K, Adams D, Belanger-Quintana A, Bushueva T, Cerone R, Chien N, Chiesa A, Coskun T, De la Heras Montero J, Feillet F, Katz R, Lagler F, Muntau A, Piazzon F, Rohr F, Spronsen F, Vargas P, Wilcox G
Poster: #117	
Phase 3 PRISM clinical trials evaluating efficacy and safety of pegvaliase for treatment of adults with phenylketonuria	Thomas J, Levy H, Amato S, Vockley G, Zori R, Dimmock C, Harding C, Bilder D, Weng HH, Olbertz J, Merilainen M, Rosen O, Gupta S, Gu K, Larimore K, Northrup H
Poster: #122	
Long-term safety of induction, titration, and maintenance dosing of pegvaliase treatment in adults with phenylketonuria	Burton B, Harding C, Thomas J, Longo N, Posner J, Dimmock D, Zori R, Weng H, Olbertz J, Gershman E, Rosen O, Gupta S, Jones S, Gu K, Vockley J
Poster: #123	
Characterization of hypophenylalaninemia in pegvaliase treated adults with PKU	Harding C, Thomas J, Burton B, Zori R, Dimmock D, Vockley J, Weng HH, Olbertz J, Gershman A, Rosen O, Jones S, Li M, Longo N
Poster: #124	
An interim analysis of the KAMPER and PKUDOS registries: efficacy and safety of sapropterin before and during pregnancy	Feillet F, Ficicioglu C, Lagler FB, Longo N, Alm J, Muntau AC, Burlina A, Belanger-Quintana A, Trefz FK, Kittus R, Jurecki E, Alvarez I, Lilienstein J, Burton B
Poster: #125	
A meta-analysis of growth outcomes in phenylketonuria patients treated with phenylalanine-restricted diet + sapropterin	Muntau A, Feillet F, Burton B, McDonald A, Wessel A, Alvarez I, Lilienstein J, Lane P, Jurecki E, Longo N
Poster: #126	

Mucopolysaccharidosis (MPS)	
Title	Authors
Natural history data for young subjects with Sanfilippo syndrome type B (MPS IIIB)	Harmatz P, Cleary M, de Castro Lopez MJ, Lee J, Lin S, Okur I, Ezgu F, Muschol N, Peters H, Solano Villarreal MS, Shaywitz AJ, Cahan H, Grover A, Maricich SM, Melton A, Smith L, Couce ML
Poster: #315	
ICV-administered BMN 250 (NAGLU-IGF2) is well tolerated and reduces heparan sulfate	Lin S, Cleary M, Couce ML, de Castro Lopez MJ, Harmatz P, Lee J, Okur I, Ezgu F, Peters H, Villarreal MS, Shaywitz AJ, Cahan H,

accumulation in the CNS of subjects with Sanfilippo syndrome type B (MPS IIIB) Poster: #316	Grover A, Maricich SM, Melton A, Smith L, Muschol N
Critical care situations in patients with mucopolysaccharidosis (MPS) Poster: #321	Stepien K, Geavorkian A, Hendriksz C, Lobzhanidze T, Perez-Lopez J, del Toro M, Vashakmadze N, Lampe C
Transition from paediatric to adult care in patients with mucopolysaccharidosis (MPS) Poster: #324	Lampe C, McNelly B, Geavorkian A, Hendriksz C, Lobzhanidze T, Perez-Lopez J, Vashakmadze N, del Toro M
Mucopolysaccharidosis VI enzyme replacement therapy outcomes across the disease spectrum: findings from the MPS VI clinical surveillance program Poster: #362	Harmatz P, Lampe C, Teles EL, Parini R, Sharma R, Sivam D, Johnson J, Hawley S, Sasic Z
Enzyme replacement therapy in patients with mucopolysaccharidosis VI: updated findings from the MPS VI clinical surveillance program Poster: #363	Harmatz P, Lampe C, Leão-Teles E, Parini R, Sharma R, Sivam D, Johnson J, Sasic Z
Mucopolysaccharidosis VI enzyme replacement therapy initiated in adulthood: findings from the MPS VI clinical surveillance program Poster: #364	Lampe C, Harmatz, Leão-Teles E, Parini R, Sharma R, Sivam D, Johnson J, Hawley S, Sasic Z

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 seven 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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