

# BioMarin Announces 14 Presentations at 15th Annual WORLDSymposium™ 2019

## Presentations to Highlight Results from Studies for the Treatment of Three Forms of MPS and CLN2 Disease, a Form of Batten Disease

SAN RAFAEL, Calif., Feb. 4, 2019 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today that the company will present data in four platform and 10 poster presentations at the 15<sup>th</sup> Annual WORLDSymposium™ being held February 4-8, 2019 in Orlando, Florida.

"We look forward to the WORLDSymposium every year as it is an unprecedented forum to share and discuss pioneering research for the rare disease community," said Hank Fuchs, M.D., President of Worldwide Research and Development at BioMarin. "In addition to exploring new potential therapies in areas of great unmet medical need, we also continue to examine our treatments long after they have been approved by regulatory authorities. We look forward to sharing our findings."



## Listing of BioMarin Posters and Presentations at the 15<sup>th</sup> Annual WORLDSymposium™

### Platform Presentations

Presentations will be held in the Regency Ballroom U.

Title	Presenter
ICV-administered BMN 250 (NAGLU-IGF2) is well tolerated and reduces heparan sulfate accumulation in the CNS of subjects with Sanfilippo syndrome type B (MPS IIIB)  <b>Presentation:</b> February 7 at 1:00 – 1:15 PM	Maureen Cleary, Great Ormond Street Hospital
Insights into Sanfilippo syndrome provided by the ConnectMPS worldwide online registry  <b>Presentation:</b> February 7 at 2:15 – 2:30 PM	Edwin Chavez-Cintora, BioMarin Pharmaceutical
Persistent treatment effect of cerliponase alfa in children with CLN2 disease: A 3-year update from an ongoing multicenter extension study  <b>Presentation:</b> February 7 at 3:30 – 3:45 PM	Angela Schulz, University Medical Center Hamburg- Eppendorf
Evidence-based, expert-agreed recommendations for the management of patients with MPS IVA/VI: Recommendations to replace the specific missing enzyme  <b>Presentation:</b> February 7 at 4:15 – 4:30 PM	Christian J. Hendriksz, Steve Biko Academic Hospital, University of Pretoria

### Poster Presentations

MPS IVA & VI	
Title	Authors
Impact of two-year elosulfase alfa treatment on patient-	Adam J, Cleary M, Hendriksz CJ, Hiwot

<p>reported outcomes in patients with Morquio A: Results from an English Managed Access Agreement</p> <p><b>Presentation:</b> February 5 at 4:30 – 6:30 PM <b>Poster/Presentation:</b> #6</p>	<p>TG, Hughes D, Jones SA, Jovanovic A, Morrison A, Murphy E, Vijay S</p>
<p>Enzyme replacement therapy in patients with mucopolysaccharidosis type VI: Updated findings from the MPS VI Clinical Surveillance Program</p> <p><b>Presentation:</b> February 5 at 4:30 – 6:30 PM <b>Poster/Presentation:</b> #146</p>	<p>Harmatz P, Lampe C, Parini R, Sharma R, Leão-Teles E, Johnson J, Sivam D, Sisic Z</p>
<p>Analysis of the impact of cell-based neutralizing antibody status on long-term efficacy of elosulfase alfa</p> <p><b>Presentation:</b> February 5 at 4:30 – 6:30 PM <b>Poster/Presentation:</b> #LB-25</p>	<p>Hock B, Slasor P, Bray L, Lau K, Melton A, Jesaitis L, Qi Y, Solari P</p>

MPS	
Title	Authors
<p>Evidence-based, expert-agreed recommendations for the management of patients with MPS IVA/VI</p> <p><b>Presentation:</b> February 5 at 4:30 – 6:30 PM <b>Poster/Presentation:</b> #155</p>	<p>Hendriksz C, Giugliani R, Harmatz P, Scarpa M</p>
<p>Transition from pediatric to adult care in patients with mucopolysaccharidosis (MPS)</p> <p><b>Presentation:</b> February 5 at 4:30 – 6:30 PM <b>Poster/Presentation:</b> #207</p>	<p>Lampe C, McNelly B, Geavorkian A, Hendriksz C, Lobzhanidze T, Perez-Lopez J, Vashakmadze N, del Toro M</p>
<p>Critical care situations in adult patients with mucopolysaccharidosis (MPS)</p> <p><b>Presentation:</b> February 6 at 4:30 – 6:30 PM <b>Poster/Presentation:</b> #340</p>	<p>Stepien K, Geavorkian A, Hendriksz C, Lobzhanidze T, Perez-Lopez J, del Toro M, Vashakmadze N, Lampe C</p>
<p>LC-MS/MS-based glycosaminoglycan measurement: Expert considerations on methods and use in screening, diagnosis and management of</p>	<p>Fuller M, Auray-Blais C, Burke D, Burlina A, Chuang C-K, Cólón C, Crawford B, Fletcher J, Gelb M, Heales S, Izzo E, Kubaski F, la Marca G, Lawrence R, Matern D, Merry C, Miller N, Pasquali M, Wood T, Harmatz P</p>

patients with mucopolysaccharidoses	
<b>Presentation:</b> February 5 at 4:30 – 6:30 PM	
<b>Poster/Presentation:</b> #LB-18	

<b>MPS IIIB</b>	
<b>Title</b>	<b>Authors</b>
ICV-administered BMN 250 (NAGLU-IGF2) is well tolerated and reduces heparan sulfate accumulation in the CNS of subjects with Sanfilippo syndrome type B (MPS IIIB)	Cleary M, Muschol N, Couce ML, Harmatz P, Lee J, Lin S, Okur I, Ezgu F, Peters H, Solano Villarreal M, Shaywitz AJ, Cahan H, Grover A, Melton A, Smith L, Maricich SM, de Castro Lopez MJ
<b>Presentation:</b> February 5 at 4:30 – 6:30 PM <b>Poster/Presentation:</b> #67	
Understanding Sanfilippo signs, symptoms and physician testing patterns: Insights from the Simply Test for MPS™ enzyme-panel program (ST4MPS)	Gurnon S, Pan W, Chu D, Ly C, Wood T, Pollard L
<b>Presentation:</b> February 5 at 4:30 – 6:30 PM <b>Poster/Presentation:</b> #142	
Natural history data for young subjects with Sanfilippo syndrome type B (MPS IIIB)	Solano Villarreal M, Harmatz P, Cleary M, de Castro Lopez MJ, Lee J, Lin S, Okur I, Ezgu F, Muschol N, Peters H, Shaywitz AJ, Cahan H, Grover A, Maricich SM, Melton A, Smith L, Couce ML
<b>Presentation:</b> February 6 at 4:30 – 6:30 PM <b>Poster/Presentation:</b> #335	

### **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 seven commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit [www.biomin.com](http://www.biomin.com). Information on such website is not incorporated by reference into this press release.

### **Links to Full Prescribing Information on BioMarin Products**

[www.Brineura.com](http://www.Brineura.com)

[www.Naglazyme.com](http://www.Naglazyme.com)

[www.VIMIZIM.com](http://www.VIMIZIM.com)

Contact:

*Investors:*

*Media:*

*Traci McCarty*  
*BioMarin Pharmaceutical Inc.*

(415) 455-7558

*Debra Charlesworth*  
*BioMarin Pharmaceutical Inc.*

(415) 455-7451

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