BioMarin Announces 2 Oral and 8 Poster Presentations at Lysosomal Disease Network's 11th Annual WORLD Symposium(TM)

SAN RAFAEL, Calif., Feb. 9, 2015 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that the company would make two data presenations in addition to presenting eight posters at the Lysosomal Disease Network's 11th Annual WORLD Symposium™ from February 9-13 in Orlando, Florida.

"We are committed to conducting research to further support patients suffering from lysosomal storage disorders, both in diseases where we have products in development and approved products. We are dedicated to research that will lead to a better understanding of the long-term effects of treatment and its impact on patients' lives," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "For instance, there is a need to better understand the long-term use of our therapies and the experiences of adult patients."

Oral Presentations

Title	Authors
Impact of Elosulfase Alfa on Pain in Patients with Morquio A Syndrome: MOR-008 Study	Harmatz P, Treadwell M, Burton B, Mitchell J, Mischol N, Jones S, Pastores G, Lau H, Sparkes R, Sutton R, Shaywitz A, Haller C
Presentation: Thursday, Feb 12, 11:15 AM	
Improved Respiratory Function in a Mouse Model of Pompe Disease Treated with BMN 701	Peng J, Cahayag R, Crockett L, Cheung R, Zoog S, O'Neill C, Tsuruda L
Presentation: Wednesday, Feb 11, 2:00 PM	

Poster Presentations

MPS

Title	Authors
Urine Keratan Sulfate (uKS) Elevation in Lysosomal Storage Disorders (LSDs): Comparison of uKS Levels in Morquio/MPS IV Versus Non-Morquio LSDs	Auray-Blais C, Ellsworth K, Fietz M, Giugliani R, Harmatz P, Lavoie P, Millington D, Trinh M, van Vlies N, Wijburg F, Wood T, Zhang H, Miller N
The Morquio A Registry Study (MARS): Improving the Understanding of Morquio A Syndrome	Graham S, Waite A, Loba-Olopade O, Hunt J, Quartel A
Urine Keratan Sulfate (uKS) in Morquio A Patients Measured via LC-MS/MS Method: Improved KS Detection as Compared to Dye-Based Methods and Report of Age-Specific uKS Reference Ranges	Pasquali M, Auray-Blais C, Ellsworth K, Fietz M, Giugliani R, Harmatz P, Izzo E, la Marca G, Lavoie P, Millington D, Trinh M, van Vlies N, Wijburg F, Wood T, Zhang H, Miller N
Immunogenicity of Elosulfase Alfa, an Enzyme Replacement Therapy in Patients with Morquio A Syndrome: Results from MOR-004, a Phase 3 Trial	Schweighardt B, Tompkins T, Lau K, Jesaitis L, Qi Y, Musson D, Farmer P, Haller C, Shaywitz A, Yang K, O'Neill C
	Burton BK, Harmatz P, Mitchell J, Muschol N, Jones

Impact of Elosulfase Alfa on Exercise Capacity and Muscle Strength and Safety in Patients with Morquio A Syndrome	S, Pastores G, Lau H, Sparkes R, Sutton VR, Berger KI, Lewis G, Tarnopolsky M, Genter F, Haller C, Shaywitz A
Impact of Mucopolysaccharidosis on Daily Living, Employment, General Health and Parenthood of Adult Patients	Lavery C, Wedehase B, Graham S, Harmatz P, Hendriksz CJ
Risks of Long-term Port Use in the MPS Population	Hendriksz C, Harmatz P, Guigliani R, Roberts J, SG Arul

Pompe disease

Title	Authors
Pulmonary Function Predictors (VC, FVC, MIP, MEP) of Respiratory Insufficiency in Late-onset Pompe Disease	Quartel A, Young P, Conner E, Johnson EM, Berger KI

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises five approved products and multiple clinical and pre-clinical product candidates. Approved products include VIMIZIM® (elosulfase alfa) for MPS IVA, a product wholly developed and commercialized by BioMarin; Naglazyme® (galsulfase) for MPS VI, a product wholly developed and commercialized by BioMarin; Aldurazyme® (laronidase) for MPS I, a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; KUVAN® (sapropterin dihydrochloride) Powder for Oral Solution and Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany and Firdapse® (amifampridine), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS). Product candidates include drisapersen, an exon skipping oligonucleotide, which is currently undergoing regulatory submission for the treatment of Duchenne muscular dystrophy (exon 51), pegvaliase (PEGylated recombinant phenylalanine ammonia lyase, formerly referred to as BMN 165 or PEG PAL), which is currently in Phase 3 clinical development for the treatment of PKU, talazoparib (formerly referred to as BMN 673), a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase 3 clinical development for the treatment of germline BRCA breast cancer, reveglucosidase alfa (formerly referred to as BMN 701), a novel fusion protein of insulin-like growth factor 2 and acid alpha glucosidase (IGF2-GAA), which is currently in Phase 3 clinical development for the treatment of Pompe disease, BMN 111, a modified C-natriuretic peptide, which is currently in Phase 2 clinical development for the treatment of achondroplasia, BMN 044, BMN 045 and BMN 053, exon skipping oligonucleotides, which are currently in Phase 2 clinical development for the treatment of Duchenne muscular dystrophy (exons 44, 45 and 53), cerliponase alfa (formerly referred to as BMN 190), a recombinant human tripeptidyl peptidase-1 (rhTPP1) for the treatment of CLN2 disorder, a form of Batten disease, which is currently in Phase 1, BMN 270, an AAV-factor VIII vector, for the treatment of hemophilia A and BMN 250, a novel fusion of alpha-N-acetyglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of MPS IIIB.

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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