BioMarin Presents 15 Abstracts From Basic Research to Clinical Trials at Lysosomal Disease Network's 10th Annual WORLDSymposium(TM)

12 Abstracts on MPS diseases and 3 on Pompe Disease
10-Year Naglazyme® (galsulfase) Data Suggests Increased Survival of MPS VI Patients

SAN RAFAEL, Calif., Feb. 13, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today 15 data presentations at the Lysosomal Disease Network's 10th Annual WORLDSymposium™ from February 11-13 in San Diego, California.

"Since our founding 16 years ago, BioMarin has been deeply committed to patients suffering from lysosomal storage disorders and continues to build on our knowledge of MPS diseases, enzyme replacement therapies and technologies to deliver those therapies to a specific place in the cell," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "The depth and breadth of our research covers exciting new molecules that have the potential to change the course of these difficult to treat diseases. We are so grateful to the patients who participate in clinical trials, and they motivate us to bring the hope of new treatment options."

Paul Harmatz, M.D., Associate in Gastroenterology and Nutrition at the Children's Hospital and Research Center in Oakland, California will present data tracking Mucopolysaccharidosis Type VI (MPS VI or Maroteaux-Lamy syndrome) patients over a 10 year period who had been on treatment with Naglazyme® (galsulfase). The data suggests that patients treated with Naglazyme lived longer, had long-term improvement in endurance, and experienced improvement in pulmonary function and growth.

"The 10-year data on galsulfase provides evidence that reinforces the accepted thinking that early intervention with an enzyme replacement can make a big difference to patients," said Paul Harmatz, M.D. "As a treating physician, therapies that can change the long-term outcome of disease are the gold standard."

Presentations:

MPS VI (Maroteaux-Lamy syndrome)

Title	Authors
Galsulfase for Mucopolysaccharidosis Type VI: Analysis of Clinical Data Since 2000	Harmatz P, Hendriksz CJ, Giugliani R, Braunlin E, Quartel A
PRESENTATION	
Growth Charts for Individuals with Mucopolysaccharidosis VI (Maroteaux-Lamy Syndrome)	Quartel A, Graham S, Harmatz P, Lin P

MPS IVA (Morquio A syndrome)

Title	Authors
Burden of Disease Suffered by Patients with Morquio A Syndrome: Results from Patient-Reported Outcomes Survey	Hendriksz CJ, Lavery C, Coker M, Ucar S, Jain M, Bell L, Lampe C
The Heart and Cardiovascular System in Patients with Morquio A Syndrome	Kampmann C, Lampe C, Reinke J, Mengel E, Gökce S, Beck M, Kuroczynski W
Burden of Disease Suffered by Caregivers of Patients with Morquio A Syndrome: Results of a Self-Reported Outcomes Survey	Lampe C, Hendriksz CJ, Lavery C, Coker M, Ucar M, Bell L, Jain M
Prediction of the Molecular Consequences of Amino Acid Substitutions in the GALNS Gene using In Silico Tools	Kubaski F, Brusius-Facchin AC, Nemetz-Bochernitsan A, Giugliani R, Leistner-Segal S
PRESENTATION	
Keratan Sulfate (KS) Analysis in Morquio A Patients: LC-MS/MS Analysis of KS Disaccharides Demonstrates that Urine is a Better Source than Plasma to Monitor Dynamic Change in KS	Miller N, Taniguchi G, Decker C, Zhou H, Shediac R, Quartel A
Morquio A Locus-Specific Database: A Framework for a Curated Database	Ryles A, Du C, Oron T, Atwood R,

to Identify and Characterize Pathogenic Variants in the GALNS Gene	Mooney SD, Francis-Lyon P

MPS IIIB (Sanfilippo syndrome)

Title	Authors
Engineering of a Recombinant NAGLU Fusion Protein with Insulin-Like Growth Factor 2 Leads to Improved Cellular Uptake via a Glycosylation-Independent Lysosomal Targeting Pathway	Aoyagi-Scharber M, Christianson T, Wendt DJ, Pascale MNT, Yip BK, Holtzinger J, Chen Z, Woloszynek J, Cheung DS, Lo MJ, Dickson P, Fitzpatrick PA, LeBowitz JH
Intraventricular Enzyme Replacement Therapy with Glycosylation-Independent Lysosomal Targeted NAGLU in the Brain of Sanfilippo B Mice	Kan SH, Le S, Vincelette J, Bullens S, Brown J, Ohmi K, Lotshaw E, Aoyagi-Scharber M, Crawford B, Bunting S, Neufeld E, Dickson P
PRESENTATION	

MPS General

Title	Authors
Target-Population Screening for Lysosomal Storage Disorders - A Highly Efficient Tool for the Diagnosis of Patients	Cobos PN, Santer R, Zoltan L
The Development and Validation of Dried Blood Spot Enzymatic Assays for MPS Type IVA (Morquio) and Type VI (Maroteaux-Lamy) Syndromes	Ullal AJ, Millington DS, Wood TC, Bali D

Pompe disease

Title	Authors
Preliminary Clinical Efficacy and Safety of BMN 701, GILT-tagged Recombinant Human Acid Alpha Glucosidase (rhGAA), in Late Onset Pompe Disease: Results of an Extension Study	Byrne B, Barhon R, Barshop B, Bratkovic D, Desnuelle C, Geberhiwot T, Henderson R, Hughes D, Laforet P, Mengel E, Roberts M, Chinnapolamada G, Schweighardt B, Tompkins T, Lang W, LeBowitz J
PRESENTATION	
BMN 701 Mediated Receptor Redistribution is Responsible for Increased Uptake	LeBowitz J, Maga J, Schooler B, Chen G, Pungor E, Prince B, Liu G, Xia Y
A Comparison of Pharmacological Activity of Multiple Production Lots of BMN 701 by Glycogen Clearance in a Mouse Model of Pompe Disease	Peng J, Cahayag R, Crockett L, Fox M, O'Neill CA, LeBowitz J, Tsuruda L

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises four approved products and multiple clinical and pre-clinical product candidates. Approved products include 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) 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 VIMIZIM™ (N-acetylgalactosamine 6-sulfatase), formally referred to as GALNS, which successfully completed Phase 3 clinical development for the treatment of MPS IVA, PEG PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase 3 clinical development for the treatment of PKU, 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, BMN 111, a modified C-natriuretic peptide, which is currently in Phase 2 clinical development for the treatment of achondroplasia, BMN 701, a novel fusion of acid alpha glucosidase (GAA) with a peptide derived from insulin like growth factor 2, which is currently in Phase 1/2 clinical development for the treatment of Pompe disease, BMN 190, a recombinant human tripeptidyl peptidase-1 (rhTPP1) for the treatment of late-infantile neuronal ceroid lipofuscinosis (CLN2), 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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