BioMarin InvestorRoom

Kuvan and Naglazyme Data to Be Presented at the 11th Annual ICIEM Meeting

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BioMarin Pharmaceutical Inc. announced today that data from clinical studies of Kuvan (sapropterin dihydrochloride) and Naglazyme (galsulfase) will be presented at the 11th Annual Meeting of the International Congress of Inborn Errors of Metabolism (ICIEM) in San Diego, California, August 29 through September 2, 2009.

"We look forward to a number of abstracts, posters and platform presentations that will highlight a variety of topics on the treatment of PKU and MPS VI. In particular, results from the PKU studies will hopefully strengthen the safety and efficacy data for Kuvan and demonstrate both the broad use of the medicine across different patient populations as well as possible neurocognitive benefits," said Chief Medical Officer Hank Fuchs, M.D.

The following abstracts, posters and presentations will be featured:

Phenylketonuria (PKU)

#171: Sapropterin Dihydrochloride Response in 35 Patients with Hyperphenylalaninemia (Vernon, et al)

#179: Clinical Experience with KUVAN Therapy in Young Children with Phenylketonuria (PKU) (Mofidi, et al)

#185: An Outreach Program for Adults Living with PKU (Leviton and Burton)

#187: PKU Treatment with Tetrahydrobiopterin (Sapropterin) During Pregnancy (Cunningham, et al)

#192: Treatment of an Individual with Phenylketonuria and Neurological Impairment with Sapropterin (Adams)

#195: The KUVAN Adult Maternal Pediatric European Registry (KAMPER): A Long Term Observational Study of Patients with Hyperphenylalaninaemia Treated with KUVAN (Champigneulle, et al)

#202: Responsiveness of KUVAN in PKU Patients During the Expanded Access Program and Following FDA Approval (Bernstein, et al)

#205: Compliance in a Group of Adolescents and Adults with Phenylketonuria (Vilaseca, et al)

#208: Assessing Cognitive and Social-Emotional Functioning in Individuals with PKU: Tools for Use in the Metabolic Clinic by Psychologists and Non-Psychologists (Waisbren, et al)

#211: Revisiting Suboptimal Outcomes of Diet-Treated PKU Patients (Enns, et al)

#216: Neuropsychological Function in Individuals with Phenylketonuria Treated with KUVAN (Grange, et al)

#236: Factors that May Lead to Inappropriate Discontinuation of KUVAN After at Least 3 Months of Continued Therapy (Imperiale, et al)

#550: NBS Long-Term Follow-Up: Improving Outcome in PKU - Drug TX and Diet Liberalization (Adams, Adams)

#594: Sapropterin Dihydrochloride (6R-BH4) and Maternal Phenylketonuria: Two Case Studies (Moseley, et al)

#647: Assessment of the Effectiveness of Metabolic University: A Training Program for Registered Dietitians, Nurses, Genetic Counselors, and Physicians (Bernstein, et al)

Mucopolysaccharidosis VI (MPS VI)

#101: The First Report of the MPS VI Clinical Surveillance Program (CSP) (Hendriksz et al) Platform Presentation - Sunday, August 30, 16:00

#348: ERT in an MPS VI Patient who was previously treated with Bone Marrow Transplantation (Sohn et al)

#379: Enzyme Replacement Therapy in Eight Mucopolysaccharidosis Type VI Brazilian Children Under Age Three: Preliminary Data (Horovitz et al)

#400: Cholelithiasis in Two MPS VI Patients: Expanding the Phenotype of Maroteaux-Lamy Syndrome (Lourenco et al)

#402: Enzyme Replacement Therapy for MPS VI: Experience in Taiwan (Lin et al)

#405: Experience of Enzyme Replacement Therapy in Patients with Mucopolysaccharidosis Type VI (Micheletti et al)

#410: Mucopolysaccharidosis Type VI Diagnosis Using Dried Blood Spot Samples - A Brazilian Experience (Gimenes et al)

#413: Craniovertebral Abnormalities and Spinal Cord Compression in Type VI Mucopolysaccharidosis (Horovitz et al)

#423: Decompress Cervical Surgery in Patient with Advanced MPS VI (Solano et al)

#424: Sleep Evaluation in Patients with Mucopolysaccharidosis Type VI (Souza et al)

#426: Severe Cardiomyopathy is Reverted in Patient with Advanced MPS VI Under ERT (Solano et al)

#437: MPS VI - Report of a Very Mild Phenotype in a Brazilian Patient (Souza et al)

#441: High Prevalence of Mental Impairment in a Series of Six Patients with Mucopolysaccharidosis Type VI (Valayannopoulos et al)

#444: Treatment of Diarrhea in Patient with MPS - Cases Report (Frangipani et al)

#449: Phonological Assessment in Patient with MPS I, MPS II and MPS VI (Goncalves et al)

#464: Clinical Characterization and Follow up of 26 Colombian Patients with MPS VI (Solano et al)

#466: Prevalence of MPS VI Patients in Brazil (Martins et al)

#469: Maroteaux Lamy Syndrome: Enzyme Replacement Therapy Outcome in a Severe Form (Ospina et al)

#473: 20-Year Follow Up of Bone Marrow Transplantation in a MPS VI Patient with Substantial Residual Pathology (Andersson)

About PKU

PKU, a genetic disorder affecting approximately 50,000 diagnosed patients in the developed world, is caused by a deficiency of the enzyme phenylalanine hydroxylase. PAH is required for the metabolism of phenylalanine, an essential amino acid found in most protein-containing foods. If the active enzyme is not present in sufficient quantities, Phe accumulates to abnormally high levels in the blood and becomes toxic to the brain, resulting in a variety of complications including severe mental retardation and brain damage, mental illness, seizures, tremors, and limited cognitive ability. As a result of newborn screening efforts implemented in the 1960s and early 1970s, virtually all PKU patients under the age of 40 in developed countries have been diagnosed at birth. To learn more about PKU, please visit www.PKU.com. Information on this website is not incorporated by reference into this press release.

About Kuvan

Kuvan (sapropterin dihydrochloride) Tablets are indicated in the United States to reduce blood phenylalanine (Phe) levels in patients with hyperphenylalaninemia (HPA) due to tetrahydrobiopterin- (BH4-) responsive phenylketonuria (PKU). Kuvan is to be used in conjunction with a Phe-restricted diet. The active ingredient in Kuvan, sapropterin dihydrochloride, is the synthetic form of 6R-BH4 (tetrahydrobiopterin), a naturally occurring enzyme cofactor that works in conjunction with phenylalanine hydroxylase (PAH) to metabolize Phe.

Kuvan has received orphan drug designation from both the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMEA). Kuvan has received seven years of orphan exclusivity in the United States and ten years of market exclusivity in the E.U.

About MPS VI

MPS VI (also known as Maroteaux-Lamy syndrome) is a debilitating, life-threatening genetic disease caused by a deficiency of the enzyme N-acetylgalactosamine 4-sulfatase. This enzyme deficiency leads to the accumulation of certain complex carbohydrates, glycosaminoglycans (GAGs), in the lysosomes, giving rise to

progressive cellular, tissue and organ system dysfunction. The majority of individuals with MPS VI die from disease-related complications between childhood and early adulthood. Additional information can be found at www.mpsvi.com.

About Naglazyme

Naglazyme is the first and only enzyme replacement therapy indicated for the treatment of MPS VI. Naglazyme is indicated for patients with MPS VI. Naglazyme has been shown to improve walking and stair-climbing capacity.

The most common adverse events observed in clinical trials in Naglazyme-treated patients were headache, fever, arthralgia, vomiting, upper respiratory infections, abdominal pain, diarrhea, ear pain, cough, and otitis media. Severe reactions included angioneurotic edema, hypotension, dyspnea, bronchospasm, respiratory distress, apnea, and urticaria. The most common symptoms of infusion reactions included fever, chills/rigors, headache, rash, and mild to moderate urticaria. Nausea, vomiting, elevated blood pressure, retrosternal pain, abdominal pain, malaise, and joint pain were also reported. No patients discontinued for adverse events and all patients who completed the double-blind portion of the trial continued to receive weekly infusions of Naglazyme. Nearly all patients developed antibodies as a result of treatment, but the level of the immune response did not correlate with the severity of adverse events. Because antihistamine use may increase the risk of apneic episodes, evaluation of airway patency should be considered prior to the initiation of treatment. Consideration to delay Naglazyme infusion should be given when treating patients who present with an acute febrile or respiratory illness. Additional information can be found at www.naglazyme.com.

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises three approved products and multiple clinical and pre-clinical product candidates. Approved products include Naglazyme (galsulfase) for mucopolysaccharidosis VI (MPS VI), a product wholly developed and commercialized by BioMarin; Aldurazyme (laronidase) for mucopolysaccharidosis I (MPS I), a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; and Kuvan (sapropterin dihydrochloride) Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany. Other product candidates include PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in development for the treatment of PKU and GALNS (N-acetylgalactosamine 6-sulfatase), which is currently in Phase I/II clinical development for the treatment of MPS IVA. 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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