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Ultragenyx Announces Presentation of Data From a Single Patient Treated With Recombinant Human Beta-Glucuronidase at 10th Annual World Lysosomal Disease Network Symposium

NOVATO, Calif., Feb. 12, 2014 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (Nasdaq:RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today announced data presentations from a single patient treated with recombinant human beta-glucuronidase (rhGUS, UX003), an investigational therapy for the treatment of mucopolysaccharidosis 7 (MPS 7, Sly syndrome).

The following abstract is being presented in oral and poster presentations by William Sly, M.D., Chairman Emeritus of the Department of Biochemistry at Saint Louis University, at the 10th Annual World Lysosomal Disease Network Symposium in San Diego. The oral presentation will take place on February 13, 2014.

> **J.E. Fox M.D., L. Volpe M.D., J. Bullaro, E.D. Kakkis M.D., Ph.D., W.S. Sly, M.D.** Recombinant Human Beta-Glucuronidase Enzyme Replacement Therapy for Mucopolysaccharidosis Type 7: Report of the First Patient Treated

Dr. Sly is presenting the case study of a single 12 year old patient with advanced multi-system MPS 7 with respiratory insufficiency currently being treated with rhGUS. Preliminary data showed a reduction in lysosomal storage based on reduced excretion of urinary glycosaminoglycans and a reduction in the size of the enlarged liver and spleen. The patient showed an improvement of pulmonary function and no infusion-associated reactions during the first 14 weeks of treatment. The patient's caregivers also reported improved stamina and increased time spent in school.

The treatment is sponsored by Dr. Joyce Fox and Steven and Alexandra Cohen Children's Medical Center of New York under an emergency IND (eIND) granted by the Food and Drug Administration. rhGUS is provided by Ultragenyx to the hospital under this eIND.

About MPS 7

Mucopolysaccharidosis type 7 (MPS 7, Sly syndrome), originally described in 1973 by William Sly, M.D., is a rare genetic, metabolic disorder and is one of 11 different MPS disorders. MPS 7 is caused by the deficiency of beta-glucuronidase, an enzyme required for the breakdown of the glycosaminoglycans (GAGs) dermatan sulfate and heparan sulfate. These complex GAG carbohydrates are a critical component of many tissues. The inability to properly break down GAGs leads to a progressive accumulation in many tissues and results in a multi-system disease.

While its clinical manifestations are similar to MPS 1 and MPS 2, MPS 7 is one of the rarest among the MPS disorders. MPS 7 has a wide spectrum of clinical manifestations and can present as early as at birth. There are no approved therapies for MPS 7 today. The use of enzyme replacement therapy as a potential treatment is based on 20 years of research work in murine models of the disease. Enzyme replacement as a strategy is well established in the MPS field as there are currently three approved enzyme replacement therapies for other MPS disorders: MPS 1 (Aldurazyme®, laronidase), MPS 2 (Elaprase®, idursulfase), and MPS 6 (Naglazyme®, galsulfase).

Ultragenyx initiated a Phase 1/2 study in the UK to evaluate the safety, tolerability, efficacy, and dose of intravenous administration of rhGUS in December 2013.

About Ultragenyx

Ultragenyx is a development-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with an initial focus on serious, debilitating metabolic genetic diseases. Founded in 2010, the company has rapidly built a diverse portfolio of product candidates with the potential to address diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no approved therapies.

The company is led by a management team experienced in the development and commercialization of rare disease

therapeutics. Ultragenyx's strategy is predicated upon time and cost-efficient drug development, with the goal of delivering safe and effective therapies to patients with the utmost urgency.

For more information on Ultragenyx, please visit the company's website at www.ultragenyx.com.

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