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Ultragenyx Announces Positive Interim Data From Phase 1/2 Study of Recombinant Human Beta-Glucuronidase in Mucopolysaccharidosis 7

Rapid and Sustained Reduction in Urinary Substrate Excretion Observed Over 12 Weeks

NOVATO, Calif., Sept. 3, 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 the presentation of positive interim data from the Phase 1/2 study of recombinant human beta-glucuronidase (rhGUS, UX003), an investigational therapy for the treatment of mucopolysaccharidosis 7 (MPS 7, Sly syndrome). The data are being presented at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium in Innsbruck, Austria.

The Phase 1/2 open-label clinical study is assessing the safety, efficacy, and dose of rhGUS administered every other week via intravenous infusion in three patients. A 12-week primary analysis phase evaluating 2 mg/kg of rhGUS every other week is being followed by dose-exploration and long-term extension.

"We are pleased with the 12-week results of the first clinical study to be conducted in MPS 7 and are grateful to the patients and investigators who are participating in the study," commented Emil D. Kakkis, Ph.D., M.D., Chief Executive Officer and President of Ultragenyx. "Based on the reduction of lysosomal storage shown in all patients in the Phase 1/2 study, we plan to move into Phase 3 testing."

Results from the primary analysis phase show evidence of clearance of lysosomal storage as indicated by the decline in urinary glycosaminoglycan (GAG) excretion and the reduction in liver size. The change in urinary GAG excretion was observed by two weeks after the first dose of rhGUS and declined by approximately 40-50% from baseline after 12 weeks of treatment. Decreases in liver size were observed in the two patients who had enlarged livers at baseline.

No serious adverse events were observed in the 12-week primary analysis phase and through up to 28 total weeks of treatment. The most common adverse events reported to date are infections and gastrointestinal disorders. No infusion-associated reactions were observed after a total of 38 infusions to date in these three subjects.

In addition to the Phase 1/2 study, one patient continues to be treated under an emergency Investigational New Drug application (eIND) sponsored by Dr. Joyce Fox and the Steven and Alexandra Cohen Children's Medical Center of New York. Through 24 weeks of treatment, a decline in urinary GAG excretion of 50-70% and a sustained reduction in the size of the enlarged liver and spleen have been observed. The data also show improved pulmonary function based on reduced carbon dioxide retention. No serious adverse events or infusion-associated reactions were observed through 12 infusions.

The Phase 1/2 study will continue through the dose-exploration phase and long-term extension. Based on the Phase 1/2 study results and the 24-week results of the patient treated under an eIND, the company intends to initiate a pivotal Phase 3 study by year-end 2014.

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 four approved enzyme replacement therapies for other MPS disorders: MPS 1 (Aldurazyme®, laronidase), MPS 2 (Elaprase®, idursulfase), MPS 4A (Vimizim™, elosulfase alfa), 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 clinical-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with a 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.

Forward-Looking Statements

Except for the historical information contained herein, the matters set forth in this press release, including statements regarding Ultragenyx's expectations regarding the timing of release of data, plans regarding continuation of the Phase ½ study and the timing of initiation of a pivotal Phase 3 study, are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, future results, performance, or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainties inherent in the clinical drug development process, including the regulatory approval process, the timing of our regulatory filings, and other matters that could affect the availability or commercial potential of our drug candidate. Ultragenyx undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of the Company in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on May 12, 2014, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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