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Ultragenyx Announces Positive 36-Week Data From Phase 1/2 Study of Recombinant Human Beta-Glucuronidase in Mucopolysaccharidosis 7

A Rapid and Sustained Dose-Dependent Reduction in Substrate Accumulation

NOVATO, Calif., Feb. 10, 2015 (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 36-week 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 in poster and oral presentations by Dr. Emil Kakkis, Chief Executive Officer of Ultragenyx, at the 11th Annual WORLD Symposium in Orlando, Florida.

"We are encouraged by the longer term safety and efficacy data that provide additional support for our ongoing Phase 3 study," said Sunil Agarwal, M.D., Chief Medical Officer of Ultragenyx. "We decided to proceed with the 4 mg/kg dose in the Phase 3 study based on the greater reduction in lysosomal storage at that dose."

The Phase 1/2 open-label clinical study assessed the safety, efficacy, and dose of rhGUS administered every other week via intravenous infusion in three patients. Results from the 12-week analysis evaluating 2 mg/kg of rhGUS every other week were presented in September 2014 and showed a decline in urinary glycosaminoglycans (GAG) excretion of approximately 40-50% from baseline. After the initial 12 weeks, the study entered a dose-exploration phase in which patients were treated with a lower and then higher dose of rhGUS. The 36-week results showed a greater change in urinary GAG excretion at the higher 4 mg/kg dose of rhGUS, with a mean urinary GAG reduction of approximately 60%.

Sustained decreases in liver size were observed in the two patients who had enlarged livers at baseline, and an improvement in pulmonary function was observed in the one patient who was able to perform the evaluations. Improvements were also observed in the MPS Health Assessment Questionnaire (HAQ) measure of functional capabilities and in the Physician Global Impression of Change (PGIC) scale of overall health status in this open-label study.

No serious adverse events or infusion-associated reactions were observed in the study. The most common adverse events were consistent with the symptoms of MPS 7 or related to intravenous administration of the investigational therapy, including respiratory disorders, infections, and arthralgia.

Patients from the Phase 1/2 study continue to be treated with rhGUS as part of a long-term extension study. Based on the findings from the Phase 1/2 study, in December 2014 Ultragenyx initiated a Phase 3 study of rhGUS in MPS 7 patients at a dose of 4 mg/kg every other week. Data from the Phase 3 study are expected in the first half of 2016.

Agreement has been reached with both the US Food and Drug Administration (FDA) and European Medicines Agency (EMA) on the Phase 3 study design. The FDA stated that their evaluation of the pivotal Phase 3 study will be based on the totality of the data on a patient-by-patient basis. FDA advised against the declaration of a primary clinical endpoint in order to allow for more flexibility in the overall efficacy evaluation, appreciating the heterogeneous and rare nature of this disease. The EMA has agreed that approval under exceptional circumstances could be possible based on the Phase 3 study with urinary GAG levels as a surrogate primary endpoint, provided the data are strongly supportive of a favorable benefit/risk ratio and that some evidence or trend in improvement in clinical endpoints is observed.

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).

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 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 and plans regarding continuation of the Phase 1/2 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 November 10, 2014, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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